PATIENT ACCESS TO ALTERNATIVE TREATMENTS: BEYOND THE FDA

HEARINGS

BEFORE THE

COMMITTEE ON GOVERNMENT REFORM AND OVERSIGHT HOUSE OF REPRESENTATIVES

ONE HUNDRED FIFTH CONGRESS

SECOND SESSION

FEBRUARY 4 AND 12, 1998

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PATIENT ACCESS TO ALTERNATIVE TREATMENTS: BEYOND THE FDA

WEDNESDAY, FEBRUARY 4, 1998

HOUSE OF REPRESENTATIVES,
COMMITTEE ON GOVERNMENT REFORM AND OVERSIGHT,
Washington, DC.

The committee met, pursuant to notice, at 10:13 a.m., in room 2154, Rayburn House Office Building, Hon. Dan Burton (chairman of the committee) presiding.

Present: Representatives Burton, Morella, McHugh, Horn, Mica, Davis of Virginia, Souder, Pappas, Snowbarger, Miller, Waxman, Sanders, Maloney, Norton, Kucinich, Davis of Illinois, and Tierney.

Staff present: Kevin Binger, staff director; Judith McCoy, chief clerk; William Moschella, deputy counsel and parliamentarian; Laurie Taylor and Carolyn Hicks, professional staff members; Teresa Austin, assistant clerk/calendar clerk; Will Dwyer, director of communications; Ashley Williams, deputy director of communications; Robin Butler, office manager; Phil Schiliro, minority staff director; Phil Barnett, minority chief counsel; Cherri Branson, minority counsel; Karen Lightfoot, minority professional staff member; Ellen Rayner, minority chief clerk, and Jean Gosa, minority staff assistant.

Mr. BURTON [presiding]. Good morning. A quorum being present, the Committee on Government Reform and Oversight will begin its duties.

Today, we will begin a series of hearings to examine issues and problems related to alternative medical treatment for millions of desperately ill Americans. We will also look at deep-seated flaws in the process of the Food and Drug Administration that governs access to some of these treatments. These issues are often controversial. My purpose in holding these hearings is to lay the issues on the table and deal with them in the most reasonable and balanced way.

We owe it to the millions of patients, their families, and loved ones who are not satisfied with conventional treatments. Health is the first of all liberties, and each person is the proper guardian of his or her own health. Yet, in our system of health care, personal choice in treatments is all too often not an option. The FDA often gets in the way of our choices of alternative medicines and treatments.

Medicine is a matter of weighing the benefits of a treatment against any possible harm that may result. At times the Federal health care agencies seem to put much more energy and effort into avoiding harm than they do in weighing the benefits—this, despite

the fact that conventional medicine sanctioned by the FDA offers some highly toxic, extremely expensive, and at times only marginally successful treatments for most deadly illnesses.

For example, more than 25 years have passed since President Richard Nixon first declared war on cancer. He predicted a cure within 5 years. So far, cancer has won. We have no cure and made only little progress in developing cures. Meanwhile, we know that half a million people will die from cancer this year alone. There are growing numbers among them who will depart from conventional treatments because they are too toxic, too expensive, and less effective.

The same can be said of other serious diseases, such as multiple sclerosis, hepatitis C, arthritis, asthma, and many others. The meager advances by conventional medicine in the treatment of these diseases has made alternative and complementary therapies overwhelmingly popular. Indeed, more than 45 percent of Americans will use some alternative therapy this year alone, and they will pay for it out of their own pockets. Who can blame them for searching against hope for a cure?

More and more doctors are having tremendous success in using alternative treatments together with conventional treatments. Others are succeeding with time-proven, natural preparations in ways that are less toxic, less damaging, and often less expensive than the typical conventional therapy.

Despite the growing popularity and success of alternative treatments, some of our Government institutions are fighting that trend. The FDA dictates what treatments doctors can use in treating serious illnesses, but most of those are toxic and often dangerous to already-weakened patients. Meanwhile, our Government agencies have spent untold billions of dollars trying to find elusive cures.

In addition, the FDA has harbored a culture of intimidation and sometimes harassment against those who are looking for alternative cures. Today, we will hear from a researcher who is at impasse with the FDA after spending his career searching for a cure for cancer. He found himself so overwhelmed by FDA paperwork requirements that, as an individual researcher, he simply could not comply. Today his research is on hold, with no hope to resume.

It is sometimes done under the guise of defending good science and weeding out fraud, but frequently it undermines the practice of good medicine and the potential for greater advances and possible cures. Many doctors are truly healing patients through innovative, safe, and effective measures. At the same time, doctors who use alternative treatments do so at great peril to their reputations and their right to practice medicine. It is because of the tremendous courage of many of these doctors and their patients that some progress has been made with respect to alternative treatments in this country.

They are not alone in history. It seems that all great discoveries were met for a period of time with skepticism and ridicule. For instance, Louis Pasteur was ridiculed for his germ theory of disease and he was ostracized from the medical community for some time. Dr. Ignaz Semmelweis spoke to his colleagues about the importance of preventing the passing of infection to women in childbirth

by washing their hands after an autopsy. They laughed at him when he talked about that. He was similarly ridiculed and died an early death without any recognition. Finally, there was Jonas Salk, a young doctor with the only hope against polio. He produced a vaccine that was initially forbidden by the medical establishment.

If anything, history teaches us that in the long term those who are ridiculed for their discoveries are often eventually proven right. If we don't learn from history, we're doomed to repeat it, and that makes progress difficult. What's clearly needed is a shift in thinking from Government knows better to the people know better. At least, there needs to be more of a balance.

The FDA process for access to new treatments is a good example of this need. That is why today we will examine options available to seriously ill patients for promising new treatments, and also the barriers to getting access. Access to a treatment in the development process that is not approved by the FDA generally requires participation in a clinical trial, but many patients do not qualify under the strict guidelines of a trial. The FDA then makes a life-or-death decision as to whether a patient can have the treatment under a special exception. If the answer is no, their access is shut off with no appeal.

And I have personally experienced that in my family, and I know many of the people in the audience today have as well. When that kind of a decision is made, it's very, very, very hard to deal with. You hurt inside because you want to help your loved one, and because of regulations, you can't do a darned thing about it. So you just sit there and try to figure out the best way to cope while you watch them lose their hair and maybe sometimes even their will to live.

Under these conditions, patients must apply through an FDA regulatory process to try to gain access to their desired treatment. This can be lengthy, trying, and frustrating, especially for someone who is terminally ill. And for those who do not have the stamina, the family support, sometimes the legal fees, or even congressional help, it can be a dead end, and they just die.

We know from the FDA's own records that in 1996, about 500 cancer patients were given access to an experimental drug through the FDA, compared to a half a million who died last year. We will hear compelling testimony from some of those patients about how the FDA process is broken. If that is true, then the Congress is obliged to find a way to fix it.

We know that the FDA process cannot accommodate a half a million people. So, in essence, we as a Government are deciding who gets treated and how they are treated, and everyone else is on their own. We cannot tolerate that in an open society where choice and the right to a healthier life is the first liberty.

These hearings will also explore ways to help those hundreds of thousands who get left out of the FDA-sponsored experimental treatments, and therefore, are left out in the cold. We will hear from patients' families who have lost hope and are facing death, who are up against a massive bureaucracy, which seems to have little understanding of their pain, suffering, and desperation.

I believe that if a patient is terminally ill, he should have access to any experimental treatment on the market. After all, it's their

life, and if they're adjudged terminally ill, who better than they should be able to make the decisions on how they get treated? Certainly not the Government. He or she should not have to wade through red tape. He or she should not have to fight against a bureaucracy. He or she should not have to spend thousands of dollars. If someone is fighting for their life, the Government ought to be helping them find new alternatives, not throwing up roadblocks. A truly compassionate society will help find solutions to greater access to new and promising treatments. Good health and medicine require it.

Let me just say in closing my opening statement, obviously, the FDA is needed and they do a lot of good, but they do throw up roadblocks in many cases against people, in front of people, who are terminally ill, and they then cutoff all hope, and that hope should never be curtailed by any individual or any government. That's why we're holding these hearings.

Mr. Waxman.

Mr. WAXMAN. Thank you very much, Mr. Chairman.

Today, we're going to hear from a number of witnesses who are fighting cancer. I have the greatest sympathy for them and their families. I appreciate their willingness to come forward and tell their stories, and I hope they will succeed in fighting and in defeating this disease.

What cancer patients need is to have access to experimental drugs, to have those experimental drugs properly tested, and to learn whether or not they work. No one wants to waste money on treatments that don't work. No one wants to forego treatments that might be more effective. And if something does work, it should be made available to as many patients as possible.

The issue of access to unapproved drugs has far-reaching implications both for those who are ill today and those who become ill in the future. That is why this issue was taken so seriously and studied so exhaustively by another committee of Congress, the Commerce Committee, which has primary jurisdiction over the Food and Drug Administration.

Over the course of 3 years, the Commerce Committee held dozens of hearings in both Health and Environment and the Oversight Subcommittees, and heard from patients, providers, and researchers on the subject of FDA reform. The results of that careful review and thorough analysis was carefully crafted, bipartisan legislation to reform the FDA, legislation that became law just 2 months ago. As a member of the Commerce Committee, I participated extensively in that process.

That FDA reform legislation contained a number of provisions that will directly benefit patients who seek to use experimental therapies. The bill broadened access to experimental drugs to people with serious illness and life-threatening diseases. The bill contained a fast-track process for drugs with the potential to help patients who have diseases for which there are now few treatments, and it created a clinical data base, so that patients will have greater access to comprehensive information about experimental therapies for serious and life-threatening diseases.

Many of you who are here today testified before the Commerce Committee and participated in that process, and I want to assure

you that your message was heard. Congress did act. Congress passed, and the President signed into law, FDA reform. Although I'm not sure of the purpose of this hearing, given the fact that new legislation has been enacted, and there is no oversight goal because the law doesn't take effect until later this month, I want to welcome the witnesses. I will be here for as much of this hearing as possible, although I must apologize to those witnesses that testify when I'm not here, because I do have a conflict in my schedule.

I thank you for holding this hearing, Mr. Chairman, and will look forward to getting the full testimony and record that will help us evaluate this issue further.

Mr. BURTON. Thank you, Mr. Waxman.

Our first panel today will be a former colleague of ours, Mr. Berkley Bedell. Mr. Bedell, would you come forward and take your seat?

Oh, I'm sorry. Let me swear in the witness, and then I'll yield to you.

[Witness sworn.]

Mr. BURTON. Please be seated.

I understand we have some Members who would like to make some opening statements. So, Mrs. Morella, would you like to be recognized?

Mrs. MORELLA. Thank you, Mr. Chairman. Just a brief statement to thank you for holding these hearings on patient access to alternative treatments. I know, and as we've heard, that you're personally committed to this issue, and I appreciate your dedication to helping all desperately ill Americans expand their treatment options, regardless of their individual resources.

I look forward to hearing from today's panel of patients and medical and professional experts. This testimony is very important. The patients before us sought alternative medicine for life-threatening illnesses.

Congressman Bedell, I want to thank you for sharing your experiences overcoming Lyme disease and prostate cancer using an alternative treatment. It's great to see you. I look forward to your testimony.

I also look forward to hearing from our medical and professional experts. Your experiences will shed light on the barriers to alternative medicine. I'm interested in hearing recommendations on how the FDA can both maintain its critical mission of consumer protection and safety and help to foster innovative new treatments of chronic and terminal illnesses.

I'm also interested in hearing from the witnesses about NIH's Office of Alternative Medicine, its mission, and its contributions.

There's no doubt alternative medicine serves an important function, and it makes sense to look at how to expand treatment options. In doing so, however, it is critical that we ensure that patients receive accurate information, not only about safety, but also about efficacy.

I thank you, Mr. Chairman.

Mr. BURTON. Thank you, Mrs. Morella. Mr. Sanders.

Mr. SANDERS. Thank you, Mr. Chairman. I'll be brief.

I'm a co-sponsor of the legislation, and I think what I would say that is in my own State of Vermont, about 2 years ago, we held

a hearing on alternative medicine. You know, in Vermont it gets cold and it gets snowy, and it was a cold, snowy day in January, and we held the meeting in Randolph, VT, and to my surprise, we had over 500 folks come out. Dr. Wayne Jonas, who is the head of the Office of Alternative Medicine, was a guest speaker; Dr. Herbert Bensen from the Harvard University Institute of Mind/Body was also there.

I know that in the State of Vermont there is a strong effort, shared by some of our hospitals, increasingly shared by some of the medical establishment, to take a look at alternative approaches to disease, to try to also understand how we can develop a safe environment in terms of nutrition, in terms of exercise, in terms of the air that we breath, the food that we eat, so that people don't get sick in the first place.

So I applaud you, Mr. Chairman, for holding this hearing, and I look forward to hearing the testimony of our witnesses. Thank you.

Mr. BURTON. Thank you, Mr. Sanders.

Mr. Davis, did you have any opening statements?

Mr. DAVIS OF VIRGINIA. Briefly, first of all, I look forward to hearing Congressman Bedell. What's today's conventional wisdom sometimes becomes obsolete as we learn more about it. The question for us is always to try to find the right balance. So I'm very interested in reviewing the testimony today.

Congressman, I never had an opportunity to meet you, but I actually did a settlement for your son, Tom, years ago, as a young attorney out in McLean in the early seventies, and followed your bouts with Lyme disease and prostate cancer, and look forward to having you and the other witnesses here today on a topic that is just critical.

Mr. Chairman, I appreciate your holding these hearings. Thank you.

Mr. BURTON. Thank you, Mr. Davis.

I'm sorry, Mr. Horn, you're down there at the end; I didn't see you for a second.

Mr. HORN. Thank you, Mr. Chairman. I'm delighted you are holding these hearings. My wife has had breast cancer; I've had prostate cancer. We're both in good shape, despite that. I think we ought to take a very careful look at every possibility we can think of. Obviously, it has to meet certain scientific tests down the line, but I have long felt—and I participated as a witness in the Barton hearings of the Commerce Committee—I have long felt that the FDA was not moving as rapidly as it should in a whole range of areas, not simply alternatives, but even in the basic pharmaceutical areas, where they seem to get things online a little faster in Europe than we do in the United States. I think we all realize the FDA is there to provide for the public safety. On the other hand, if you can organize yourself properly, they ought to be there to move things along as rapidly as possible, rather than as slowly as possible. So I'm looking for a little guidance in that from our various witnesses.

Thank you, Mr. Chairman.

Mr. BURTON. Thank you, Mr. Horn. Mr. Snowbarger.

Mr. SNOWBARGER. Pass.

Mr. BURTON. He passes, OK.

[The prepared statements of Hon. Constance A. Morella, Hon. Christopher Cox, and Hon. Edolphus Towns follows:]

The Honorable Constance A. Morella
Committee on Government Reform and Oversight
Patient Access to Alternative Treatments: Beyond the FDA
February 4, 1998

Mr. Chairman, I want to thank you for holding hearings on patient access to alternative treatments. I know you are personally committed to this issue, and I appreciate your dedication to helping all desperately ill Americans expand their treatment options, regardless of their individual resources.

I look forward to hearing from today's panel of patients and medical and professional experts. This testimony is very important; the patients before us sought alternative medicine for life-threatening illnesses. Congressman Bedell, thank you for sharing your experiences overcoming Lyme Disease and prostate cancer using an alternative treatment. It is great to see you, and I look forward to your testimony.

I also look forward to hearing from our medical and professional experts. Your experiences will shed light on the barriers to alternative medicine. I am interested in hearing recommendations on how the FDA can both maintain its critical mission of consumer protection and safety and help foster innovative new treatments of chronic and terminal illness. I am also interested in hearing from the witnesses about the NIH's Office of Alternative Medicine, its mission, and its contributions.

Alternative medicine serves an important function, and it makes sense to look at how to expand treatment options. In doing so, however, it is critical that we ensure that patients receive accurate information not only about safety, but also about efficacy.

FEBRUARY 4, 1998

OPENING STATEMENT OF REP. CHRISTOPHER COX

VICE-CHAIRMAN, COMMITTEE ON

GOVERNMENT REFORM AND OVERSIGHT

HEARING ON ACCESS TO MEDICAL TREATMENT

Mr. Chairman, thank you for holding this hearing today.

The question before us today is: what obligations does the Food and Drug Administration have to severely-ill patients who have beneficial, unapproved treatment interrupted as a result of U.S. food and drug law investigations or prosecutions? A broader question is whether U.S. food and drug law should be changed to permit patients greater freedom of choice in medical treatment.

As evidenced by today's witnesses, an increasing number of severely-ill patients are turning to alternative forms of medical treatment, after finding more traditional medicine to be unsuccessful. In fact, the New England Journal of Medicine has reported that more than one out of every three Americans have at some time relied on an alternative form of medical treatment.

Unfortunately, the Food and Drug Administration is making it difficult, if not impossible, for many patients to gain access to desired forms of alternative medical treatment. Some of this is due to the fact that the FDA operates under a law written decades ago and for the specific purpose of providing for the regulation of more traditional forms of medicine.

Toward this end, I'd be interested in hearing from our witnesses today their thoughts on the merits of H.R. 746, the Access to Medical Treatment Act, bipartisan legislation of which I am an original sponsor.

As introduced, the Access to Medical Treatment Act would ensure that individuals are free to choose to be treated by any legally authorized health care practitioner with any method of medical treatment--provided that there is no evidence that the treatment causes harm, and that the patient is fully informed about any possible side effects.

For those of us for whom a complete overhaul of federal food and drug laws remains a top priority, today's hearing should provide a much-needed dose of reality for lawmakers who believe that our food and drug laws are truly serving the best interests of severely-ill patients.

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STATEMENT OF REP. EDOLPHUS TOWNS

BEFORE THE COMMITTEE ON GOVERNMENT
REFORM AND OVERSIGHT

FEBRUARY 4, 1998

Mr. Chairman, I am a bit amazed about today's hearing topic of access to medical treatment "beyond the FDA".

Yesterday, Democratic members and some Republican staff were briefed by FDA. Some of the information in the briefing related to drugs currently under FDA review, so I am limited in what I can say. However, it is clear to me that FDA has good answers to many of the questions that may arise today. It is unfortunate that our request to let FDA testify today was rejected.

However, Mr. Chairman, I am here to day because it appears that this hearing is intended to encourage the use of experimental therapies on gravely ill people. Apparently, this committee is having a memory lapse or enjoys self-contradiction.

In the first session of the 105th congress, this committee voted unanimously to adopt a report examining the illnesses experienced by Persian Gulf War veterans. That report, entitled "VA, DOD Continue to Resist Strong Evidence Linking Toxic Causes to Chronic Health Effects" was based on eleven hearings held by the subcommittee. During those hearings, the Committee heard testimony and reviewed thousands of documents provided by private citizens and the federal Departments. We reached one major unequivocal conclusion: the use of unapproved or investigational drugs should not have been allowed in a setting that prevented oversight, evaluation and monitoring. In unanimously passing that report, this full Committee shared that conclusion. Mr. Chairman, I believe that you voted in support of that report. Yet here we are today encouraging the use of unapproved, investigational therapies in settings that are without the appropriate oversight, evaluation and monitoring.

Now some will say that I am unsympathetic and have no compassion for the sick. Nothing could be further from the truth. Let's be clear. The FDA generally approves the applications of any patient who wants to use an investigational drug where the doctor agrees to conduct the proper evaluation. I know that a sick patient may be desperate and willing to try anything. I know that it is not fashionable, but I believe that the government has a duty to

safeguard the best interests of its citizens, in sickness and in health. There are some things that we simply cannot allow the free market to decide and the safety of potentially dangerous drugs is one of them.

Additionally, Mr. Chairman, as a member of this committee and the Commerce Committee, I know that the Commerce Committee had several hearings about this topic. As a result of those hearings, the 105th Congress passed legislation which thoroughly addressed the issue of patient access to investigational drugs. The President signed that legislation into law in November.

Therefore, Mr. Chairman, unfortunately, this hearing does not add to the discussion of health care in America. Today we walk ground previously trod by others and leave no meaningful impression.

#######

STATEMENT OF BERKLEY BEDELL, FORMER MEMBER OF CONGRESS

Mr. BURTON. Mr. Bedell, do you have an opening statement?

Mr. BEDELL. I sure do. [Laughter.]

Mr. BURTON. Good. You always have, and you've always been very eloquent, Mr. Bedell. [Laughter.]

Mr. BEDELL. First of all, thank you very much for holding these hearings and getting some things out in the open that I think are very urgent.

Mr. Chairman and members of this committee, I come before you as one who has served with some of you and one who has experienced the challenges and opportunities you live with. This testimony is about one of those opportunities which you face at this time.

I also come before you as a patient who can thank alternative treatments for disease for the fact that I am alive and well. I left Congress because I came down with Lyme disease. My Lyme disease was cured by a special whey from milk at a cost of about $500, after conventional treatments consisting of an estimated $25,000 were not effective. I also came down with prostate cancer, and again, it appeared that a $600 alternative treatment was successful after it appeared that my surgery and radiation, at an estimated cost of $10,000, had not cured my cancer.

It breaks my heart to have to tell the Lyme disease patients who contact me because their current treatments are not curing them that the treatment that cured me is not available to them because of Government regulations. This is a whey from cow's milk.

The problem arises from a Supreme Court decision in *United States* v. *Rutherford,* in which lower courts ruled that a person does have a legal right to use the medical treatment of their choice. On appeal, the Supreme Court ruled that there was no such legal right because Congress had not authorized it. The court literally sent the issue back to Congress, but so far Congress has failed to act to assure people of this right. How sad.

So now no one can use a treatment in the United States unless the FDA decides that, in their opinion, it is "safe and effective." And it costs millions and millions of dollars to go through the FDA approval process. This freezes out anyone except giant corporations, and makes it utterly impossible for low-cost, non-patentable medicines to get into the system.

H.R. 746, the Access to Medical Treatment Act, is your opportunity to solve this problem. Let me explain the bill.

The bill provides that any person shall have the right to be treated by whatever treatment that person desires, so long as: The treatment is provided by a properly licensed practitioner, under the limits of their practitioner's license, who has examined the patient. There is no evidence that the treatment would be of danger to the patient. The patient has been informed of the contents of the treatment, and any possible side effects, including a written statement that says, "This treatment has not been certified safe and effective by the Federal Government, anyone who uses it does so at their own risk." There have been no advertising claims made regarding the efficacy of the treatment, and the patient has signed a state-

ment that they have been informed of all of the above, and still wish to be so treated.

This bill is tightly drawn. It will not change the FDA, nor its approval process. Because of peer pressure, pharmaceutical advertising, malpractice insurance problems, and insurance policies, the vast majority of doctors will not change the way they practice medicine in the short run. Firms who wish to advertise and promote a medicine will still have to go through the FDA approval process.

But it will break the current monopoly, and make it possible for people to try some of these alternative treatments such as the ones I used.

I know that partisan politics is a factor in Congress today, but this is a nonpartisan bill. Mr. Chairman, you're a Republican and I'm a Democrat. We probably would have voted very few times together, but this is an issue that we would both agree to, and I firmly want everyone to know this is a people's bill; it's not a partisan bill. It was introduced by a Democrat; Mr. Chairman, a Republican, you're a co-sponsor. In the Senate, it was introduced by Senator Daschle, the minority leader; Senator Lott, the majority leader, is a co-sponsor. There's large numbers of both Republicans and Democrats as co-sponsors.

I attach a list of the organizations that support this legislation, and also information on a poll by a nationally recognized polling firm of cardiologists and oncologists, where the majority of both said that they felt they should be permitted to use unapproved drugs and devices as long as they carried a warning about their unapproved status. The Access to Medical Treatment Act includes this, and it also includes several other protections.

There are those who say, "We have to protect the people." What a crazy argument. Anyone can go into a store and buy rat poison off the shelf that might kill them, but persons suffering from Lyme disease are prohibited from obtaining the whey from cow's milk that might cure them.

The issue here is pure and simple. The issue is whether informed citizens should have the right to make their own health care decisions or whether a Federal Governmental agency should make that decision for them. You do not need a poll to know how people feel about that. People are crying out across the land to get the Government off their back and let them make their own decision.

We let people make their own decisions as to how to be helped to end their lives, but we will not let them choose the method to help them save their lives. My God, and we brag that this is the land of the free.

Life is full of blessings and heartaches. My being alive and healthy is a tremendous blessing for me. My heart aches for those who are not as fortunate as I, and are suffering and dying because our Government—that is you folks—says they cannot be free to choose their own type of medical treatment.

But the greatest tragedy of all is that so far you good people in the Congress have not yet seen fit to pass this legislation and correct this tragedy. I pray that this will change.

[The prepared statement of Mr. Bedell follows:]

Testimony of former Congressman Berkley Bedell 2-4-98 Page 1.

Mr. Chairman, and members of this Committee, I come before you as one who has served with some of you, and one who has experienced the challenges and opportunities you live with. This testimony is about one of those opportunities which you face at this time.

I also come before you as a patient who can thank alternative treatments for disease for the fact that I am alive and well. I left Congress because I came down with Lyme disease. My Lyme disease was cured by a special whey from milk at a cost of about $500 after conventional treatments costing an estimated $25,000 were not effective. I also came down with prostate cancer, and again it appeared that a $600 alternative treatment was successful after it appeared that my surgery and radiation at an estimated cost of $10,000 had not cured my cancer.

It breaks my heart to have to tell the Lyme disease patients who contact me because their current treatments are not curing them, that the treatment that cured me is not available to them because of government regulations. This is a whey from cow's milk!!!

The problem arises from a Supreme Court decision in United States vs. Rutherford, in which lower courts ruled that a person does have a legal right to use the medical treatment of their choice. On appeal the Supreme Court ruled that there was no such legal right because Congress had not authorized it. The Court literally sent the issue back to Congress, but so far Congress has failed to act to assure people of this right. How sad!!!

So now no one can use a treatment in the United States unless the FDA decides that in their opinion it is "safe and effective." And it costs millions and millions of dollars to go through the FDA approval process. This freezes out anyone except giant corporations, and makes it utterly impossible for any low cost non-patentable medicines to get into the system.

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The bill provides that any person shall have the right to be treated by whatever treatment that person desires, so long as:

* The treatment is provided by a properly licensed practitioner, under the limits of the practitioner's license, who has examined the patient.

* There is no evidence that the treatment would be of danger to the patient.

* The patient has been informed of the contents of the treatment, and any possible side effects, including a written statement that says, "This treatment has not been certified safe and effective by the Federal Government, anyone who uses it does so at their own risk."

* There have been no advertising claims made regarding the efficacy of the treatment.

* And the patient has signed a statement that they have been informed of all of the above,

and still wish to be so treated.

This bill is tightly drawn. It will not change the FDA, nor its approval process. Because of peer pressure, pharmaceutical advertising, malpractice insurance problems, and insurance policies, the vast majority of doctors will not change the way they practice medicine in the short run. Firms who wish to advertise and promote a medicine will still have to go through the FDA approval process.

But it will break the current monopoly and make it possible for people to try some of the alternative treatments such as the ones I used.

I know that partisan politics is a factor in Congress today, but this is a not a partisan bill. It is a PEOPLE'S BILL. It was introduced by a Democrat, and your chairman a Republican is a co-sponsor. In the Senate, it was introduced by Senator Daschle, and Senator Lott is a co-sponsor. In both houses there are large numbers of co-sponsors from each party.

I attach a list of organizations that support this legislation, and also information on a poll by a nationally recognized polling firm of cardiologists and oncologists. The majority of both said that they felt they should be permitted to use unapproved drugs and devices as long as they carried a warning about their unapproved status. The Access to Medical Treatment Act includes this and it also includes several other protections.

There are those who say, "We have to protect the people." What a crazy argument. Anyone can obtain rat poison off the shelf that might kill them. But persons suffering from Lyme disease are prohibited from obtaining the whey from cow's milk that might cure them.

The issue here is pure and simple. The issue is whether informed citizens should have the right to make their own health care decisions, or whether a federal governmental agency should make that decision for them. You do not need a poll to know how people feel about that. People are crying out across the land to get the government off their back and let them make their own decisions.

We let people make their own decisions as to how to be helped to end their lives, but we will not let them choose the method of help to save their lives. My God!!! And we brag that this is the land of the free.

Life is full of blessings and heartaches. My being alive and healthy is a tremendous blessing for me. My heart aches for those who are not as fortunate as I, and are suffering and dying because our government--that is you--says they cannot be free to choose their own type of medical treatment.

But the greatest tragedy of all is that so far you good people in Congress have not yet seen fit to pass this legislation and correct this tragedy. I pray that this will change.

Concept Paper
Access to Medical Treatment Act H.R. 746

The Problem:
The United States has the best medicine in the world, and the Food and Drug Administration plays an essential role in evaluating the safety and efficacy of medical treatments. However, the current health care delivery system serves to discourage the development and utilization of alternative medical treatments that may have untold potential.

The time and expense currently required to gain FDA approval of a treatment works to limit participation in this system to large pharmaceutical companies. It makes it difficult to take advantage of the potentially innovative contributions of individual practitioners, scientists, smaller companies and others who do not have the financial resources to complete the FDA approval process. It also serves to prevent low-cost treatments from gaining access to the market.

Therefore, it makes sense to consider opening up the system to alternative treatments that may help patients *and* are not proven harmful under certain carefully circumscribed conditions.

The Proposal: This legislation would allow an individual to be treated by any licensed health care practitioner with any method of medical treatment the individual desires, so long as:

1) there is no basis to conclude that the treatment would be dangerous to the individual; and
2) the patient is fully informed of its side effects.

The bill also strictly regulates the circumstances under which claims can be made with respect to the efficacy of a treatment.

• FDA's role would not be changed.
The Access to Medical Treatment Act would not dismantle or appreciably change the current operations of the FDA or the conventional medical community. The FDA would still have responsibility for certifying treatments as safe and effective. This legislation merely attempts to open up the system to the utilization of certain new alternative treatments. The claims restriction in the bill is designed to remove incentive for major marketing efforts of non-FDA approved treatments, and should address the legitimate concern that this legislation could inadvertently become a "bypass" for the FDA approval process.

• Consumer protections are an essential element of the bill.
The bill contains several important protections to address the issue of consumer safety. In addition to the claims restriction, these protections include a tight definition of who qualifies as a health care practitioner, strict informed consent requirements, and a stipulation that treatments administered under this legislation may not pose a danger to the patient.

• This is a freedom of choice issue.
Freedom of choice is one of the bedrock principles upon which our nation rests. Permitting administration of alternative medical treatments, provided that individuals are not misled or misinformed, extends freedom of choice to the realm of medicine. This legislation stems from the conviction that an individual suffering from a life-threatening or otherwise serious disease for which conventional medicine offers limited hope should not be denied access to a non-conventional treatment if there is reason to believe that it might be beneficial.

Section-by-Section Analysis:

Section 1. Short title

Section 2. Definitions

Section 3. An individual shall have the right to be treated with any method of medical treatment that he or she desires, so long as the following conditions are met:

- the practitioner has personally examined the patient
- the administration of the treatment does not violate licensing laws
- there is no reasonable basis to conclude that the treatment poses an unreasonable and significant risk of danger to the patient
- the patient has been informed in writing of the nature of the treatment, including side effects and any other information necessary to meet FDA informed consent requirements
- the patient has been informed in writing of the fact that the treatment has not been declared safe and effective by the federal government, and has signed a written statement indicating that he or she has been made aware of this information
- any label on the treatment is not false or misleading
- no advertising claims have been made with respect to the efficacy of the treatment, except for accurate and truthful reporting by a practitioner of the results of his or her administration of a treatment

Section 4. Practitioners must report the nature and results of any treatment found to be dangerous to the Secretary of Health and Human Services. The Secretary of Health and Human Services must properly disseminate this information.

Section 5. Practitioners must report the nature and results of any treatment found to have a positive effect (significantly greater than the positive effect expected from a conventional treatment) on life-threatening medical conditions to the Office of Alternative Medicine.

Section 6. A treatment may be produced and introduced or delivered into interstate commerce for use in accordance with this Act, as long as there have been no advertising claims made by the manufacturer, distributor, or seller.

Section 7. A practitioner, manufacturer, distributor, or other seller may not violate any provision of the Controlled Substances Act in the provision of treatments in accordance with this Act.

Section 8. A health care practitioner who knowingly violates any provisions of this Act shall not be covered by the protections of this Act and shall be subject to all other applicable laws and regulations.

Poll of 160 randomly selected hospital-based oncologists, and 216 randomly selected cardiologists and cardiac surgeons by a nationally recognized polling firm.

8. What would your position be on a proposal to change FDA law so that unapproved drugs and devices could be made available to physicians as long as they carried a warning about their unapproved status? Would you strongly favor, somewhat favor, somewhat oppose, or strongly oppose such a proposal?

	Cardiologists	Oncologists
TOTAL FAVOR	53%	61%
Strongly favor	21%	24%
Somewhat favor	31%	37%
TOTAL OPPOSE	44%	37%
Somewhat oppose	24%	24%
Strongly oppose	20%	13%
Don't know/refused	3%	2%

10. And finally, how many years have you been in practice?

	Cardiologists	Oncologists
5 years or less	7%	14%
5-8 years	7%	14%
8-12 years	14%	14%
12-15 years	17%	11%
More than 15 years	56%	47%

ABOUT THE AUTHOR

Michael I. Krauss is a professor of law at George Mason University School of Law in Arlington, Virginia. He is a graduate of Carleton University, and received his degrees in law from the Université de Sherbrooke and Yale University. He has published numerous law review articles on issues of tort law, market processes and comparative law. His writings have also appeared in publications ranging from the *Wall Street Journal* to *Reason* magazine and *Policy Review*. In 1994 he received George Mason University's first "Teacher of the Year" award for excellence in teaching.

A slightly different version of this monograph will shortly be published in the *George Mason University Law Review*.

ACCESS TO MEDICAL TREATMENT ACT H.R. 746
AS OF 1-17-98

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BILL LIPINSKI	(IL)	MIKE	5-5701	

Individuals and Organizations Endorsing the Access to Medical Treatment Act S 578/HR746

Frank Lawlis
Prudene Broadwell
Mr. Durk Pearson and Ms. Sandy Shaw
Academy for Guided Imagery
Academy of Ambulatory Foot Surgery
Academy of Psychosomatic Medicine
Allergy and Asthma Network,
Mothers of Asthmatics
American Academy of Anti-Aging Medicine
American Academy of Biological Dentistry
American Academy of Environmental Medicine
American Academy of Environmental Medicine
American Academy of Head, Neck,& Facial Pain
American Academy of Medical Infrared Imaging
American Academy of Metabolic Medicine
American Academy of Orthomolecular Medicine
American Academy of Pain Management
American Association of Oriental Medicine
American Association of Naturopathic Physicians
American Association of Physician Specialists
American Autoimmune Related Diseases Association
American Back Company
St. Joseph's Professional Center
American Board of Chelation Therapy
American Board of Medical Psychotherapists & Psychodiagnosticians
American Board of Post Anesthesia Nurses
American Botanical Council
American Chiropractic Association
American Chiropractic Association
American College for Advancement in Medicine
American College for Advancement in Medicine
American College of Addictionality and Compulsive Disorders
American College of Sports Medicine
American EPD Society
American Herbalist Guild
American Holistic Centers
American Holistic Health Association
American Holistic Medical Association
American Holistic Nurses Association
American Liver Foundation
American Massage Therapy Association
American Occupational Therapy Association
American Orthotic and Prosthetic Association
Americans for Freedom of Choice in Health Care
Aromatherapy Seminars
Arklahoma Healing Arts Alliance
Artemisia
Association for Network Chiropractic Spinal Analysis
Arthritis Trust of America
Association of Applied Psychophysiology & Biofeedback
Asthma and Allergy Foundation of America
Atkins Center for Complementary Medicine
Ayurvedic Institute
Back Pain Association of America
Bastyr University
Biofeedback Certification Institute of America
Board for Certification in Pedorthics
Board for Orthotist Certification
Body of Knowledge Hellerwork
Bonnie Prudden Pain Erasure
Burditt & Radzius
Burzynski Patient Organization

Cancer Awareness Coalition
Cancer Control Society
Deepak Chopra
Center for Mind Body Medicine
Neil Kahanovitz, M.D.
Clinical Directors Network
Committee for Freedom of Choice in Medicine
Consumer Health & Safety Information and Support Network
Council for Responsible Nutrition
Crohn's & Colitis Foundation of America
Cystic Fibrosis Foundation
Electro Therapy Association
Epilepsy Foundation of America
Golden Group
Great Lakes Association of Clinical Medicine
Health Freedom Task Force
Herb Research Foundation
Hischel Society
Hudson Institute
Institute for Natural Medicine
Institute of Pain Management
Integral Health Professional Network
International Academy of Compounding Pharmacists
International & American Association of Clinical Nutritionists
International Association of Cancer Victors & Friends
International Chiropractors Association
International College of Applied Kinesiology
International Council for Health Freedom
International Foundation for Alternative Research in AIDS
International Oxidative Medical Association
International Rolf Institute
MarCal Associates
Maryland Nutritionists Association
Myofascial Pain Therapy
National AIDS Nutrient Bank
National Acupuncture and Oriental Medicine Alliance
National Acupuncture Detoxification Association
National Association for Music Therapy, Inc.
National Center for Homeopathy
National Certification Board for Therapeutic Massage & Body Work
National Coalition of Hispanic and Human Services Organizations
National Coalition for Cancer Survivorship
National College of Naturopathic Medicine
National Commission for the Certification of Acupuncturists
National Council for Improved Health
National Council for Therapeutic Recreation Certification, Inc.
National Health Federation
National Multiple Sclerosis Society
National Nutritional Foods Association
National Psoriasis Foundation
No. American Society of Teachers of the Alexander Technique
Orthomolecular Medical Society
Pacific Association for Holistic Aromatherapy
Physicians Association for Anthroposophy
Physicians' Committee for Responsible Nutrition
Polarity Wellness Center
Pure Food Campaign
Raftis Associates
Rosenthal Center for Alternative and Complementary Medicine
Sacro Occipital Research Society International
San Diego Hospice
C. Norman Shealy MD PhD DSc
Shealy Institute
Society for Behavioral Medicine
Society of Pain Practice Management
Sorsi
Southwest College of Naturopathic Medicine & Health Sciences
Swankin & Turner
Synergy Physical Therapy

Thought Technology
Traditional Acupuncture Associates
Traditional Acupuncture Institute
Trager Institute
University of the Pacific School of Pharmacy
Utah Natural Products Association
Vegetarian Awareness Network
Vegetarian Resource Group
Wholistic Health Center
World Research Foundation
The Wholistic Referral Network

December 12, 1997

Mr. BURTON. Thank you, Mr. Bedell. We appreciate your comments, and it is a bipartisan issue, a nonpartisan issue.

Let me say, before I ask you a couple of questions, we'll try to stick to the 5-minute rule as much as possible. So the witnesses, if you could confine your statements to 5 minutes, we'd appreciate it.

I read in Life magazine, after my wife developed breast cancer and the prognosis was not good—they said to her she had a 50 percent chance to survive 5 years, and I might add, she's doing very well because of an experimental program she's in. But I read in Life magazine about a doctor who had an experimental program in Highland Park, IL, that had helped a lot of women extend their lives when they were adjudged terminally ill from breast cancer. So I called to see about getting my wife in that program, and I was successful in doing that. There were about 75 women in it, and they were limited to how many they could get in the program because of FDA regulations.

After a while, I contacted the FDA about expanding the program, and they started looking into the expansion of it, and then they said, oh, well, they haven't met all the criterion they should. So they shut the program down. I was trying to get it expanded because it was helping my wife and 70-some other women, and they decided to shut it down.

Well, fortunately, because we had long talks and discussed this issue, they did find a way to reopen the program, and it's kind of on a temporary basis. But when they closed that program down, I have to tell you, a lot of those women called me because they knew my wife was in the program, and they were just so distraught. You know, you have to have a good mental attitude in order to have good health. If you lose hope or start to lose hope, you start going downhill many times. And because of that, many of these women, I believe, started to suffer physically, because they were closing down the program that gave them the only hope for survival. That is just a tragic situation.

So, like you, Congressman, I have experienced this in a personal way. I guess my colleague, Mr. Horn, has experienced it. When people's lives are at stake, as I said in my opening statement, they most certainly should have every opportunity to survive, and if that means after they've been adjudged terminally ill or almost terminally ill that they want to try alternative therapies or treatments or medicines or herbal treatments, or whatever it happens to be, they ought to be able to do it. After all, as you've said, it's their life.

Now you're familiar with the provisions in the FDA reform package that attempt to increase patient access, are you not?

Mr. BEDELL. Yes, I am.

Mr. BURTON. Do you think they make meaningful change for desperately ill patients?

Mr. BEDELL. I'd like for Congressman Waxman to hear because—Henry, my opinion is that you did take a step forward, but you need to know my opinion, that you really did not address the real issue. And I think the real issue is whether properly informed people should have the right to make their own decision or whether the FDA should do it for them. The changes you've made ask the

FDA to be a little bit more open in what they do, but it still leaves it completely up to them to tell me whether or not I—what I can do.

I agree completely with the first part of your statement. Let me tell you, I think we both want to do the same thing. The problem we have is, the way the law reads today, that there's no way for most of these treatments to ever be tried to even find it out. I know the FDA will tell you that there is, but I'm here to tell you that I think it's very clear that for most of the treatments I know about there's just no way in the world for them to be tried, and if they hold promise, as the treatment that your wife has, then my argument is not just that that should be—that it should help her, but that may lead us into further steps where it can help a whole lot of people rather than the 75 or so.

It's perfectly proper for people to disagree, and, Henry, you and I may disagree on some things; that's understandable. But I would hope we would be able to try to get to the bottom of what the real problem is, because, at least in my opinion, that legislation that was passed does not, although I support what was done, does not address the real problem.

Mr. BURTON. I'll yield to Mr. Waxman in just a moment for his 5 minutes, and then he can——

Mr. BEDELL. I'm sorry to take your time.

Mr. BURTON. That's all right. You can get away with calling him "Henry"; I can't. [Laughter.]

Why is there such difficulty in gaining access to many of these unconventional treatments such as herbal remedies, and what do you perceive as the main obstacles?

Mr. BEDELL. Well, I think there's a clear obstacle, and the obstacle is that you cannot use these treatments unless FDA gives you permission to, and in order to get permission, you have to get what's called an IND. I've got here what the FDA says that you have to do in order to get an IND. One of the main things is they have to decide that there's not any other alternative treatment except the one that you're proposing to use. There are very few ailments that there isn't some alternative treatment. It may not be—it may be far from completely effective, but certainly it's hard to find some that don't have some of that.

The other requirements they've got to get an IND—you're going to have some Burzynski treatment patients here. Dr. Burzynski sent me a copy of a picture of what he submitted at one time to try to get an IND, and the stack was that high [indicating] of things that he had sent in to get an IND, and he didn't get it, after doing all that.

Mr. BURTON. Let me, before my time is up——

Mr. BEDELL. Sorry, I talk too much; I'm a Congressman.

Mr. BURTON. That's all right.

Mr. BEDELL. You can expect that—I was.

Mr. BURTON. That's an institutional problem around here. [Laughter.]

Let me just say one more thing, and that is, I want to tell Henry—I hope Henry will listen to this story—when I was a State legislator, we had a real fight over the Laetrile bill and the Chimopapene bill and others which you may not be familiar with,

but these are solutions to problems for many patients that were suffering from cancer and back problems, and so forth. One of the people—and I won't mention his name or his position in government—but one of the people in government in Indiana was diametrically opposed to what we were trying to do, and he fought us every step of the way. Later, his wife became terminally ill with cancer, and when it became a personal problem in his family, he started using methods and treatments that were not approved by the FDA in order to try to save her life. So the thing that became so apparent to me—and I do not criticize him for this, because I would have done the same thing, because it was his wife's life and he loved her dearly, but the fact is, when it was a problem that was out there that people had to deal with, he listened to the people and the AMA and others, and said, hey, listen, we can't do that; that's not a good procedure; it's not proven, and so on and so forth. However, when it became a personal thing with his wife, immediately he started searching for any possible answer or remedy. As a result, he turned to things that were not "legal" in order to try to save his wife's life.

So, that's one of the things I think we ought to consider as Members of Congress in this decisionmaking process. Think of it not only as a generic problem, but think of it as your own personal problem. If your wife or your child or your mother becomes ill with something and there's no treatment on the market that's going to solve her problem, but there's an experimental treatment that's out there or an herbal treatment that you want to use, and it's not been approved by the FDA, what would you do?

Mr. Waxman.

Mr. WAXMAN. Thank you very much.

I'm going to call you "Mr. Bedell" even though we're old friends because I think that's appropriate—I'm old-fashioned in terms of the protocol, but we are old friends, and we have some disagreements, but we have a lot more that we agree upon on this issue, let alone other issues.

Mr. BEDELL. I think we do.

Mr. WAXMAN. There's no question in my mind that when you're sick or you have a loved one who's sick, you want to find anything that's going to work. It's not going to make a lot of difference to you whether it's been approved by FDA or not approved by FDA and in an experimental phase, because it's no solace to say that, had you lived long enough, you would have had access to something that later was approved by FDA as safe and effective.

But, on the other hand, when you're desperately looking for something to help you, especially if it's a disease like cancer or some other terminal illness, when you're desperate, you can start grabbing onto things that are worthless, being promoted by people that are charlatans.

And the chairman mentioned Laetrile. I remember those hearings on Laetrile. We used to have meetings where the rooms were packed, where people couldn't move, demanding that Laetrile be made available. They wanted it. Anecdotal evidence was that it worked. But, finally, we said, let's get a clear study of Laetrile by the National Cancer Institute. They did their study, and they came back and said, "this is worthless." You don't hear much about

Laetrile anymore. But not only is it worthless, it's harmful when people are going to use something that doesn't work and forego a therapy that may work.

Now we don't have the answers. That's what's driving us all crazy. We don't have the answers. We don't feel like conventional scientists have all the answers either. So we want to encourage research. We want to encourage experimentation. I believe we need an FDA to make sure that when we have drugs, that they go through at least a review of the safety and efficacy. I believe efficacy ought to be required.

So the question you have is: Well, what about those drugs that show some promise? I think if a drug shows promise, we shouldn't make people have to wait until it's approved. First of all, some drugs may never be approved because it's so expensive. Some drugs may never be approved because the manufacturer doesn't see a profit to make. So, therefore, you may never get to that point. The whole idea of the FDA modernization law that was passed was to give people more access to drugs during the experimental phase.

Mr. Bedell, you took cow's milk and it helped you. Wouldn't you have wanted that to be tested? I mean, you don't want just your one incident to be the way to determine whether it's effective. People can have other reasons why they're cured. If it works, we want to know if it works, and the best way to know if something works is to test it, and to do it in a systematic, scientific manner.

So don't you agree that you want these things tested?

Mr. BEDELL. Absolutely. Can I answer?

Mr. WAXMAN. Sure.

Mr. BEDELL. First of all, thank you for your openness and that we can have this conversation.

First of all, I want to make it clear—you said "promote medicine." The Access to Medical Treatment Act, in my opinion, would not permit the promotion of any medicine. Let me read you a little bit from that act.

Mr. WAXMAN. Well, but answer my question. Don't you want these things tested?

Mr. BEDELL. Yes, and the question is, how are they going to be tested? And my argument would be, under the current system, that, in effect, most of them cannot be tested, but under the act they could be tested without making it possible for them to be promoted.

Mr. WAXMAN. Well, you disagree, then, with the way that drugs are now tested to see if they're effective, not the fact if there is no FDA—you just don't think the process by which scientists try to determine whether drugs are effective is the correct way to determine it?

Mr. BEDELL. No, I think that's fine, but I don't think it's the only way, and I wouldn't think you'd think it's the only way, Henry. If you had 50 people treated by my cow's milk by a practitioner, and all 50 of them recovered from their Lyme disease, I would think that's a pretty good indication that that must be a pretty good treatment, even though you had not gotten a group of scientists to sit over here and say, well, this doesn't prove a damned thing. I think that people want to know what works and what doesn't work.

I don't think people are the same as the scientists that want to know all these things who put all the roadblocks in.

But I want to make it very, very clear that for anybody to market or promote a medicine, the Access to Medical Treatment Act still requires them to go through this same FDA approval process. I want that very, very clearly understood.

Mr. WAXMAN. The middle ground—more than some middle ground—what I think we accomplished in the legislation is that, while those experiments are being done, however they're being done, people will have the ability to get access to these drugs. We're not waiting until the end result. I know you're shaking your head. You might want it looser.

Mr. BEDELL. No, no.

Mr. WAXMAN. But I think that there's a role for FDA. We have FDA still involved, but during that time we have the ability of people to get access to drugs. If there's no FDA involved, and it's simply the patient's choice for whatever they want, you may believe that's the way it should be. I find that troubling because I know that human nature is such that people will take advantage of it.

Another part of that——

Mr. BEDELL. Well, they can't under this bill. This bill would not permit them to take advantage. You can't promote anything under this bill. But you said, "these drugs," and that's where, if we have a disagreement, where it comes. These drugs are the drugs that FDA decides should be looked at——

Mr. WAXMAN. It's not just the promotion. I want somebody to watch to be sure they are doing testing and research in a way that's going to be valid, to come to some conclusion. I've heard a lot of stories about people who have used drugs for which there's anecdotal statements that they've worked, but they don't systematically test it. They do use one drug one day, change it another day, and so you never know what is really working and what's consistent. I'm not a scientist, but I do believe there is a rationality to it, not simply anecdote.

Mr. BEDELL. But I'm taking too much time, but we need to clearly understand that under that bill the ones that can be looked at and tested are the ones that FDA decides can be looked at and tested, and if FDA decides that you cannot look at the whey from cow's milk that cured my Lyme disease, under your bill there's no way to go through that unless the person has the money and the wherewithal to satisfy the FDA and spend the money necessary——

Mr. WAXMAN. I disagree with you as to what the bill will do, and——

Mr. BEDELL. Well, then we'd better have a talk because——

Mr. WAXMAN. We ought to have further clarification, but it's my understanding that bill will allow people to have access to drugs, not FDA saying they can't have; if it's during an experimental phase, people will be able to become part of the clinical test——

Mr. BEDELL. The issue is, which drugs can they have access to? That's the issue.

Mr. BURTON. Thank you, Mr. Waxman, Mr. Bedell.

Mrs. Morella.

Mrs. MORELLA. Thank you, Mr. Chairman, and thank you, Mr. Bedell, for your personal experience testimony.

I'm curious about a number of things. First of all, I'm familiar with some alternative therapies—the vitamin supplements, nutritional supplements—but could you expand a little bit on what alternative therapies exist?

Mr. BEDELL. For what?

Mrs. MORELLA. Just in general.

Mr. BEDELL. Well, do you want me to tell you about my Lyme disease?

Mrs. MORELLA. Well, no, just generically, what would you include. What would you encompass in your definition of alternative therapies?

Mr. BEDELL. Well, I think I'd like to tell you about my Lyme disease. That's about as nontoxic as you can get, when you talk about whey from cow's milk. What happened with me with my Lyme disease was, as I told you, I went through three series where heavy doses of antibiotics were dripped into my veins daily—once 3 weeks, once 4 weeks, once 6 weeks. There's a place in Iowa that makes veterinary medicine, and they make it by injecting into the udder of a cow killed germs of a particular type while the cow is pregnant, before the cow has a calf. When the cow has the calf, they then take the first milk—it's called colostrum—it's really quite different from regular milk, and they take the whey from that as their medicine. Their theory is that if the cow had really been infected, the unborn calf would have contracted the disease from the mother cow before it was born, and Mother Nature would have in the colostrum what was necessary to cure the calf then from that disease. And this really—I got some of the killed spirochetes, got them to be run through the cow by that place, and this is really what cured my Lyme disease.

Mrs. MORELLA. How does one find out about alternative therapies? Is there a measurement of the efficacy?

Mr. BEDELL. That's one of the problems that we have, and I want it clearly understood that the Access to Medical Treatment Act does not address that very well. The only way people would find out about the things that were tried under this Access to Medical Treatment Act would be from word of mouth from the practitioner or the patients who had been successfully treated, and if they were not successfully treated, I argue that there aren't very many people who go around promoting something that didn't appear to work very well for them.

Mrs. MORELLA. Successful treatment comes from their own personal experience——

Mr. BEDELL. That's right, and it has for——

Mrs. MORELLA [continuing]. And they say, "I believe that this has helped me."

Mr. BEDELL. Sure.

Mrs. MORELLA. We may want to look into how we measure it.

I represent NIH, as you know, and you were involved, weren't you, in the establishment of the Office of Alternative Medicine?

Mr. BEDELL. Yes, I was. Yes.

Mrs. MORELLA. I'm wondering——

Mr. BEDELL. Senator Harkin did it, but I was also involved in talking——

Mrs. MORELLA. Yes. I'm curious about how has that Office of Alternative Medicine helped to bolster the use of alternative medicine? Tell us a little bit——

Mr. BEDELL. Primarily, over the fact that it was established so that people know that there are some people in Government that have some concern over alternative medicine, and so on.

Mrs. MORELLA. Is there a role for it in this legislation?

Mr. BEDELL. Beg your pardon?

Mrs. MORELLA. Is there a further role to enhance and expand that office?

Mr. BEDELL. Only that for treatments that practitioners find to be effective, they should report it to HHS, which we assume we'd end up with it.

Mrs. MORELLA. Do we need, in addition to facilitation at the FDA, more general scientific research?

Mr. BEDELL. Well, all through history, science has been the thing that's held back innovation from going forward. You know, it's just a fact. And I'm not against science. Science is tremendously important, what they've done in terms of looking at how our bodies operate, and all that sort of thing. But most scientists are wedded to what they feel they know, and what they know is what we've known in the past, and most scientists are not particularly interested in anything new or innovative. The chairman has already mentioned that, and what's happened historically. Certainly, I'm not anti-scientist, but if we think that people don't know anything about what they're doing, I think we're equally wrong.

Mr. BURTON. The time has expired.

Mrs. MORELLA. Indeed.

Mr. BURTON. Can I tell a story?

Mrs. MORELLA. Yes, indeed, Mr. Chairman.

Mr. BURTON. I went to Africa. I was the senior Republican on the African Subcommittee. You'll find this very interesting. I developed a stomach problem—this is after I was in Congress—and I couldn't eat anything and keep it down. So I heard a story about a guy named Barry Marshall down at the University of Virginia, and I went down to see Barry Marshall because I had tried everything, and he gave me a series of antibiotics with bismuth and some other things, and said this had been very effective. I took it, and within 1 week I had no more stomach problems.

Now Barry Marshall went to Belgium at an international gastro meeting with all of the stomach experts around the world, and he told of his theory that there was a bacteria that was living in the stomach that was causing 95 percent of the problems people experienced. It wasn't because of nerves that people were getting ulcers. It was because of this bacteria. It's called the helicobacter; H-pylori bacteria they called it. He told them about it, and they literally laughed him off the stage like they did Louis Pasteur.

Now Barry Marshall, who's a good friend of mine now—he cured my stomach problem—Dr. Barry Marshall went home, and he drank the bacteria and he got deathly ill, and then he took the treatment himself and he cured himself. He used this as the way

to prove to the scientific community that his treatment worked. He literally endangered his own life.

Now he's been nominated for the Nobel prize because now everybody accepts his thesis that the bacteria that he talked about does and can live in the stomach, where before they didn't believe it could. If he had not done that, his treatment would not be approved today, and people wouldn't be being cured of stomach problems. People who had cancer of the stomach have been cured by his treatment.

Now the only reason I bring that up is, there was a treatment that was ruled ineffective, was not approved, and he forced the issue and won, and he probably one day will get the Nobel prize for it. This is in our timeframe.

Thank you very much for yielding.

Mrs. MORELLA. There are probably many other instances like that, and I think we should continue to look to alternative medicine solutions, but I'm reminded, in linguistics and logic, the post hoc ergo propter hoc fallacy, that because something occurs after something, we attribute it to what occurred prior to it. So I think we must be cautious about that.

Thank you, Mr. Chairman.

Mr. BURTON. I apologize for taking so much of your time.

Mr. Sanders.

Mr. SANDERS. Thank you.

This is an enormously complicated and emotional issue. Nobody has the magic answer. I agree certainly with Henry Waxman, who, by the way, is one of the heroes of this country in taking on the tobacco interest, who for years and years told us there was no problem there, and so forth and so on, and we're indebted to Mr. Waxman for that effort.

Mr. BURTON. Can we applaud for that?

Mr. SANDERS. Right. Yes, we should. [Laughter.]

And Mr. Waxman is absolutely right also, in that we all know there are totally unscrupulous people out there who prey off human misery and panic and will make a buck trying to sell useless products when people are hopeless and at their wit's end.

Having said that, there's another side to the story. Let me just mention a few things that come to my mind about how often formal or establishment science, which also does enormous work—we all know that. I just came from the doctor's office yesterday, and got my shots and all that stuff; we're indebted to that. Let's just go through a few things.

Nutrition, today there are very few people who deny the importance of good nutrition in terms of preserving health or bad nutrition and disease; 20 or 30 years ago, it was little ladies in California, they used to call about these little old ladies in California who were the people who were talking about that. Now it is common knowledge. Cigarettes, 40 years ago, we had doctors on television advertising the brands of cigarettes that they smoked. Emotions and human health, 30 or 40 years ago, they were separated; here's physical health; here's emotional health. Now there's a general understanding that there's a strong correlation between how we feel about ourselves, our attitudes in life, our emotional health, and physical illness. Breast feeding, 40 years ago, women were casti-

gated for doing this terribly unnatural thing of breast feeding their babies, and now study after study—that was the medical establishment—a terrible thing to spoil your child and breast feed. Now, obviously, everyone thinks it is the right thing to do. Exercise, 30 years ago, bed rest was the cure to everything, and now hospital after hospital have gymnasiums in their hospitals because doctors understand that getting the body moving again is important.

Heart treatment, just one example, Dr. Dean Ornish—and I should point out that the growth of alternative medicine is such that many insurance companies now are beginning—beginning, and that's a whole other issue—to start funding alternative programs. Dean Ornish developed an alternative treatment for heart disease. It is now being funded by a number of—premiums are being paid—not premiums, but it is being funded now by a number of insurance companies. His stress is on a low-fat diet, exercise, meditation.

The whole issue of meditation, laughter, as a kooky idea, is now not thought to be so crazy. Acupuncture, it took a New York Times journalist to get sick in China—remember, what was the name of the guy, the editorial writer, James Reston had a problem in China, it was 20 or 30 years ago. He was operated on with acupuncture, and suddenly acupuncture became in vogue in this country, despite the fact that it had been used thousands of years in China. Chiropractic medicine is now being accepted in limited ways in hospitals; massage therapy; herbal remedies.

Now what's the point? The point is that the U.S. Government and their doctors, and the AMA, do not necessarily know all of the answers to all of the problems. And while there is no question that sometimes people will think they're being cured by a remedy, and they may be wrong, there may be other factors, it is also important to point out that the U.S. Government is not right all of the time also.

Right at this moment, I have been deeply involved on the issue of Gulf War illness, and I must say that, after 6 years, if you go to the VA today and you go to the DOD today, they don't know the cause of the problem. They have no effective treatment. Yet, they are not looking out to others for developing alternative treatments.

Four years ago, in this room, the late Mike Synar and I were discussing an issue about carpets and how certain types of carpets were making people ill because carpets are heavily laced with chemicals, and there are hundreds of doctors who were treating people who had been made ill by carpets. Yet, we could not get the EPA to talk to one of those physicians, not one. I mean, it was a horrible, horrible experience. That raises the issue of indoor air quality, multiple chemical sensitivity, and all of that stuff. Has the Government done a good job on that? No, it's not.

So I think the balance that we want is that we want to go after quacks, and the quacks are out there, but, on the other hand, everything being equal, we want to respect the right of people to approach practitioners and get the treatment that they feel works for them, as long as they understand the limitations. I think you mentioned what was in the law, and I think that that's important.

So I would say that we should open up the process. I support the legislation.

Mr. WAXMAN. Will the gentleman yield to me?

Mr. SANDERS. Yes, I'd be happy to.

Mr. WAXMAN. I don't disagree with what you're saying. We ought to respect new theories, new experiments, new ideas, because sometimes we reach conclusions just because they're new, they're not accurate. But Barry Marshall, for example, was mentioned, had a hypothesis. He was laughed at when he had no data. When he developed the data, when he showed that his hypothesis worked, then he got the drug approved in a very quick timeframe. He, if we had him here, would probably defend the idea that you have to use some kind of scientific scrutiny, and rationality to determine whether it works, and he was the best example of somebody who did that.

I worry that we do dismiss ideas, and we all know how so many things were dismissed that now are accepted, common wisdom, but we ought not to be so quick to dismiss it. There's something about human nature that's a plus and a minus, and often it's minus because human nature is such where people aren't open to new ideas, but we ought to be open to them, and then pursue them, not ignore them, but not just accept them without——

Mr. SANDERS. I agree, Henry, but the truth of the matter is, if you had a Federal Government today or an apparatus or a bureaucracy that was vigorous—I mean, for example, we fought very hard to get the Office of Alternative Health, and do you know what? That office, which is being besieged with telephone calls, is not getting the respect from the NIH that it wants right now, and we have to fight that battle as well.

So I—well, OK, I've said enough. Thank you, Mr. Chairman.

Mr. BURTON. Well, thank you, Mr. Sanders.

Let me, before I yield to Mr. Horn, just say that I think you helped make the point, though; Mr. Marshall was having a very difficult time in getting everything done until he actually made himself a guinea pig. Then, once he proved it, then they started—the wheels started moving. It seems to me they should have expedited that in a better way, rather than waiting until he actually put his life at risk before he checked that out—before they checked it out.

Mr. Horn.

Mr. HORN. Thank you, Mr. Chairman. I have one question, and then I'll yield the rest of my time to you.

Let me ask you, to what degree is the idea that if a person is terminally ill, and declared so by appropriate doctors, that they should receive a particular medication or alternative medicine, even if the FDA has not approved it? We certainly see that problem in AIDS, where a lot of people are worried that I can't have access to that because the FDA hasn't approved it, and yet they're clearly in a terminally ill situation.

Mr. BEDELL. Sure.

Mr. HORN. Is there any language on that?

Mr. BEDELL. I'm not real knowledgeable about AIDS, but because of the political pressure, there have been some loosening for AIDS patients. But if I was a cancer patient and was sent home by conventional medicine to die, there's nothing more they can do for me, I can go to Germany or I can go to the Bahamas or I can go to Mex-

ico, or somewhere else, but I cannot have access to any of these treatments here in the United States because the FDA has said that they cannot be administered here in the United States.

Mr. HORN. Well, I'm raising the question: If you're declared terminally ill, should they have it, regardless of what the FDA now says?

Mr. BEDELL. Oh, well, sure. I can take you to any number of those clinics and they will show you—I'm not saying everybody—but they will show you patients—you can check it—that have been terminally ill that have been cured by those people. Now the question is not, to me, whether it's 1 in 10 or 1 in 1,000, or what it is. My argument would be that if I am terminally ill and sent home to die, and there's some place I can go that there's some reasonable chance that it might somehow save my life, it's very wrong for the Government to tell me I can't do it here in my own country unless I've got enough money to go somewhere else.

That is part of the argument here. The argument here is whether people should have the right to make those decisions if they're properly informed, and not misled, themselves, or whether a Government bureaucrat should tell them they've got to go home and die. To me, I just can't imagine that. Maybe I'm too—maybe I'm soft-hearted; I don't know, but death is not really a small item in our society.

Mr. HORN. Well, I thank you, and I yield back my time to the chairman, if he'd like to use it. I have to get to a meeting.

Mr. BEDELL. Mr. Chairman——

Mr. BURTON. Thank you.

Mr. BEDELL [continuing]. Can I quickly say something that's in this bill?

Mr. BURTON. Sure. The gentleman has yielded me his time, and I'll let you——

Mr. BEDELL. Let me read you—Henry, I want you to be sure to hear this. In the bill it says that, subsection (c), "there have been no advertising claims made with respect to the efficacy of the medical treatment by the practitioner, manufacturer, or distributor," and that is defined in the bill, advertising claims. The term "'advertising claims' means any representations made or suggested by statement, word, design, device, sound, or any combination thereof, with respect to a medical treatment." I would argue that his says that you can't market anything, which I think is our big concern, you can't market anything without going through the FDA process, even if this legislation were passed.

Mr. BURTON. Who seeks time? Mr. Kucinich. The gentleman is recognized for 5 minutes.

Mr. KUCINICH. Thank you very much, Mr. Chairman.

I think we're at a point in American history where people are starting to look at alternative solutions to many things, and medicine is one area where the structured medicine, which has served millions of Americans for many, many years, is—there are currently people looking at ways to try to expand that structure, certainly respecting the practical alternatives which allopathic medicine provides for people, but at the same time availing ourselves of expanding knowledge of the possibilities of alternative treatments.

The testimony of the former Congressman, as well as discussions I have had with literally hundreds of my constituents on this issue over the past few years, makes it obvious that across this country there is an awareness that alternative methods of treatment are part of a vast, if you want to call it, "underground" approach to treating serious illnesses.

I think that as we go into this issue, we come to many different aspects which challenge our whole assumptions about health care right from the beginning, and that I think is healthy. The idea that perhaps someone else can even be responsible for health is an issue that's being challenged, I think will be challenged in the next millennium. People are starting to take more responsibility for their health. We seek the assistance of medical practitioners to help facilitate our wellness, but it begins with our own knowledge and understanding. So people are looking at diet, as it creates conditions of health. People are looking at the possibilities of how their everyday life practice creates what we know as health.

While I'm from a community which has one of the finest medical facilities anywhere in the world, Cleveland, OH, and I'm a strong supporter of that great medical complex in Cleveland, OH, I also know that we're at the eve of a new dawn of understanding in health care, and we have to expand the possibilities. We have to permit for the emergence of alternative ways of curing people.

Hundreds of years ago, the methods that are now used by allopathic practitioners would have seemed to be impossible. The changes in technology have improved the exercise, facilitated the practice of allopathic medicine. But, again, as we introduce new ways of thinking about health, it's inevitable that alternative methods need to be looked at.

And we also really don't know the way in which the relationship between psyche and soma interact. That's still a vast, uncharted area in health care: how people can come to believe that something works for them, and maybe doesn't work for another person.

So this kind of a hearing, Mr. Chairman, is extremely important because it gives us a chance to open up a window and look through this great expanse of knowledge and possibilities, and to hear from people who have experienced wellness as a direct of alternative methods when everything else failed. We have to respect that testimony, and we have to take it for being what it is; and that is, it's representative of a great number of people who have come to this table with—and will come here—with stories of how they benefited. So I think in Congress, what we need to do is to listen carefully and find ways that we can enable more and more of our people to achieve wellness using the broadest range of possibilities.

Thank you very much, Mr. Chairman.

Mr. BURTON. The gentleman yields back the balance of his time.

Mr. KUCINICH. I yield back.

Mr. BURTON. Mr. Davis, you're next. I've been informed we have two votes possibly on the floor, and I would like to finish with Mr. Bedell before we head for the vote if it's possible.

Mr. DAVIS OF ILLINOIS. Well, thank you very much, Mr. Chairman. As a matter of fact, I only have one or two questions.

Let me thank you so much for your testimony. I really enjoyed it. I appreciate hearing it.

Let me ask you, do you think that there is any strong possibility that we might have difficulty under the act regulating or determining who practitioners really are?

Mr. BEDELL. No. The practitioners are, I think, clearly defined, and the important thing is that this legislation says, under the limits of their license. So that this legislation says that, if you were a chiropractor, this would not permit you to give injections, for example. So that the issue of who a practitioner is doesn't expand in any way who a practitioner is, and it clearly says that you cannot do anything more than you're already authorized to do under the law.

Does that answer your question or——

Mr. DAVIS OF ILLINOIS. It does. The followup thought that I have: Should the act be passed—one of the criticisms of the Food and Drug Administration has been that it sometimes takes an awfully long time to make the determination about the use—someone mentioned the Chimopapene treatment earlier, which is something that I had some years ago. As a matter of fact, I traveled to Canada at the time to receive it because it could not be provided by my physician, and that's been about 20 years, and of course I'm pleased to note that I've been walking ever since and doing quite well as a result of it.

But do you think it might help facilitate or speed up the practice of the Food and Drug Administration in terms of the time that it takes to determine whether or not a treatment or a drug could be safely used?

Mr. BEDELL. No, I do not, and this legislation does not address that issue. In fact, I have to tell you, in my opinion, if the Food and Drug Administration certifies that something is safe and effective, they ought to be darned sure that it is safe and effective, and this legislation in no way would adversely change how they operate in terms of certifying things as safe and effective. I would not personally believe that they should. I believe people are entitled to know—have clear, accurate information, and if the Government tells them something is safe and effective, I think they ought to have every confidence that that is, indeed, true. I do not criticize the FDA for the fact that you told them to make sure it's safe and effective; they'd better really do it.

Mr. DAVIS OF ILLINOIS. And you have no fear that it may generate a proliferation of practitioners who have ideas and great theories, but no real opportunity for them to have been determined useful or not, that individuals may not run off and use them anyway?

Mr. BEDELL. I didn't understand your question.

Mr. DAVIS OF ILLINOIS. Well, I'm saying, all of us get ideas that—specially people who are intellectual and people who have been trained and people who know things, and those have not always been tested, or tested effectively or not——

Mr. BEDELL. That's right.

Mr. DAVIS OF ILLINOIS [continuing]. And will use them. I'm saying, do you think that it may generate more people using them than——

Mr. BEDELL. Well, in the legislation, if it's shown to be dangerous, they have to report it immediately, any treatment. So that

there are—I do not doubt for a minute, whether we have the legislation or not, there are going to be some people treated with a treatment that doesn't work. I would argue that we've got a lot of those right today in our system. But this legislation says that if I have an idea and I'm a practitioner and I treat a person with that, first of all, there cannot be any evidence that it's endangered the patient, and if I find that it is, I have to report it immediately to the Government, so that information can be dispensed. And if I continue to administer it, I'm in violation of the act, and I have no protection under the act anymore.

Mr. DAVIS OF ILLINOIS. I thank you very much, and I have no further questions, Mr. Chairman.

Mr. BURTON. Mr. Tierney, no questions?

I want to thank you, Congressman Bedell, for being with us.

We will stand in recess until these two votes, and then we'll go to the next panelists.

Mr. BEDELL. I apologize for taking so much time, Mr. Chairman, to both you and the other witnesses.

To the gentleman from California, I appreciate the fact that you're here, and I agree with you; I think we agree on a lot more than what we disagree on, and I want you to know it was a pleasure serving with you.

Mr. BURTON. Thank you, Mr. Bedell.

The committee stands in recess. Hopefully, we'll be back in about 15 minutes.

[Recess.]

Mr. BURTON. The committee will reconvene. Will everybody please take their seats, and could we get the doors shut, please?

I have a couple of unanimous consent requests. I don't think they're controversial. I'll bring these up at this time.

I ask unanimous consent that the record remain open to receive answers to additional questions the committee may propound to the witnesses. Without objection, so ordered.

I ask unanimous consent that all written statements submitted by Members and the witnesses be included in the appropriate part of the record. Without objection, so ordered.

Our second panel is Mr. Jack Kunnari, Becky Nippert, Genevieve Sherman, Mary Jo Siegel, and Ann Fonfa. I hope I pronounced that correctly. Would you please come forward and take your seats at the table. I think you have name tags there.

Ms. Fonfa sits there, Ms. Siegel, Ms. Sherman, Ms. Nippert, then Mr. Kunnari.

We normally swear everybody in. I know that you're not going to give false statements, but it's just standard procedure.

Is everyone here?

Ms. FONFA. Mr. Chairman, I need to affirm.

Mr. BURTON. Beg your pardon?

Ms. FONFA. I would need to affirm; I don't swear.

Mr. BURTON. OK, you may affirm.

Would you raise your right hands, please?

[Witnesses sworn.]

Mr. BURTON. Be seated.

All right, I guess we could have switched the name tags around.

I think maybe it would probably be proper just to go down the line and ask you if you have opening statements, and we'd like for you, if you can, to limit your statements to 5 minutes, and we'll submit any extra that you have for the record.

So, Ms. Fonfa, may I have your opening statement?

STATEMENT OF ANN FONFA

Ms. FONFA. Yes, thank you.

When a person's diagnosed with cancer, her immediate desire is to be given the treatment that will cure it. We want to rely on our doctors for the answer. I know, because I was diagnosed with breast cancer in January 1993 at the age of 45. I found a lump during my monthly breast self-exam, just 2 months after a clinical exam by my doctor.

We don't have any answers for cancer. If we were doing well with conventional treatments, cancer mortality rates would surely have fallen dramatically, and 50 percent of all women diagnosed with breast cancer would not be dead in 15 years.

I recently attended the 15th Annual Symposium of the Chemotherapy Foundation. Many of the speakers mentioned the moderate gains now being achieved through the use of chemotherapy. Unfortunately, survival time does not seem to be impacted by any new developments in drug use, and if survival has not improved, then surely we must look in other directions.

In the past 2 years, the American Cancer Society and NCI would place a nonconventional treatment on the unproven methods list, and that was the kiss of death. No research funds would be received once an idea was trashed this way. So instead of examining new parameters, they were written off almost immediately. Now this may be great for keeping the system running neatly, but it sure has been lousy for a person with cancer.

The fact that natural treatments usually are not owned or promoted by any company has probably limited development. NCI should take charge and design appropriate clinical trials to move their investigation forward rapidly.

Interestingly, treatments that were classified as unproven have lately been re-examined and removed from the list; for example, hyperthermia and Coley's toxins. It's my belief that the impetus to explore alternatives comes from the consumer movement.

The Chemo Prevention Branch of NCI now has a mandate to explore many natural substances used in those modalities. As a patient and advocate, I often wonder how to approach a conventional physician with my nontoxic protocols. Last year, after extensive research and discussions with several scientists, and my physician in Mexico, I began using high-dose vitamin A and vitamin E in liquid form. I started this on March 1, 1996. By the 22nd, I observed a decrease in the tumor, and over the next week it continued to reduce in size. When I went to my oncologist to show him, he said, "I don't remember what the tumor used to look like." And I could understand that, but what was so enraging was that he exited the room almost immediately thereafter. He never touched the lump. He didn't even measure it. He barely looked at it. Surely a concerned, interested, open-minded clinician would want to rejoice along with his patient at such a result, especially since the Chemo

Prevention Branch has been looking into the use of vitamin A, known as retinoids, and researchers are currently using it to treat some cancers.

It's 5 years since I began researching alternative and complementary cancer therapies. Did I find a single magic bullet? No, but then I no longer believe in that concept. I think each patient may find something that's right for them. I wish there were tests devised to tell us who might benefit from which treatment, including the conventional ones. In fact, I deplore the idea that we cannot distinguish the patients for whom chemotherapy is effective from those who are simply harming their bodies with no gain. This is an area our tax dollars should pay to explore. After all, most cancer patients are given the conventional treatment, and many still die.

Many people with cancer call me for information and advice. I tell them I'm not a doctor, and I don't have any answers, but what I do know about are possibilities, and there are many. Only if we know what is available is the concept of informed choice fully functioning.

Materials should be in every surgeon's and oncologist's office, so that patients have immediate access to choices in treatment. Yes, in some cases when a patient is deemed terminal the doctor will not object if the family comes up with something to try that's out of the norm, but rarely will they know enough about the possibilities to offer advice. It's almost as if they were wearing blinders. No matter how many patients die of their disease, the physician has no personal responsibility to explore the options.

An additional torment is the insurance question. We may seek and try several options. They're almost always less expensive than conventional treatments, but receive not a penny in coverage. I personally spent $27,000 on a 5-week trip to a clinic in Mexico. My insurance company would not even cover the blood test I received, nor did they pay to have a catheter inserted, so that I could use certain treatment. When I returned to New York, the surgery to remove the catheter was fully covered.

Comparing the costs, I noticed that a 1-hour-and-fifteen-minute surgery in New York cost me $7,000. Four hours of surgery would have cost me as much as the 5-week stay, which I credit with helping me regain control of my health. Of course the surgery would have been fully covered.

Another aspect that's rarely addressed is the fact that many oncologists prescribe drugs in what's called an off-label use. This means they follow hunches and not accepted protocol. If they're willing to do this with chemotherapy drugs, why not expand the horizons to allow the use of nontoxic treatments? Although few medical schools offer course work on nutritional issues, complementary, or natural medicine, continuing education courses are now available. Of course there's not much money to be made from natural substances.

And why haven't we heard about treatments that are used in other countries? Germany uses homeopathy and herbs, as does France. In China, cancer treatment is normally combined with herbs, and standard protocol, while Japan has pioneered the use of medicinal mushrooms. American doctors need to expand their vision. People with cancer in their families are looking for doctors

who will respond to questions about alternative and complementary treatment. We will no longer accept uninformed responses. Our lives are at stake, and we need access to all medical options.

Thank you.

[The prepared statement of Ms. Fonfa follows:]

Testimony of Ann E. Fonfa February 4, 1998

When a person is diagnosed with cancer her immediate desire is to be given the treatment that will cure it. We want to rely on our doctors for the answer.
I know because I was diagnosed with breast cancer in January of 1993 at the age of 45. I found a lump during my monthly breast self-exam, just two months after a clinical exam by my doctor.

We don't have any answers for cancer. If we were doing well with conventional treatments, cancer mortality rates would surely have fallen dramatically. And 50% of all women diagnosed with breast cancer would not be dead in 15 years.
I recently attended the XVth annual symposium of the Chemotherapy Foundation. Many of the speakers mentioned the moderate gains now being achieved through the use of chemotherapy. Unfortunately survival time does not seem to be impacted by any new developments in drug usuage. And if survival has not improved, then surely we must look in other directions.

In past years, the American Cancer Society would place a non-conventional treatment on the Unproven Methods list, and that was the kiss of death. No research funds would be received once an idea was trashed this way. So instead of examining new parameters, they were written off almost immediately. Now this may be great for keeping the system running neatly but it sure has been lousy for a person with cancer. The fact that natural treatments usually are not owned or promoted by any company has probably limited development. NCI should take charge and design appropriate clinical trials to move their investigation forward rapidly.

Interestingly, treatments that were classified as unproven have lately been re-examined and removed from the list, i.e. hyperthermia. It is my belief that the impetus to explore alternatives comes from the consumer movement. The Chemoprevention branch of NCI now has a mandate is to explore many natural substances used in these modalities.

As a patient and advocate, I often wonder how to approach a conventional physician with my non-toxic protocols. Last year, after extensive research and discussions with several scientisits(Dr. Zachrau, Dr. Issels) and my physician in Mexico, I began using high dose Vitamin A and Vitamin E in liquid form. I started this on March 1st 1996. By the 22nd I observed a decrease in the tumor. Over the next week, it continued to reduce in size. When I went to my oncologist to show him, he said "I don't remember what the tumor used to look like". I could understand that but what was so enraging was that he exited the room almost immediately thereafter. He never touched the lump, he didn't even measure it, he barely looked at it. Surely a concerned, interested open-minded clinician would want to rejoice along with his patient at such a result. Especially since the Chemoprevention branch has been looking into the use of Vitamin A known as retinoids, and researchers are currently using it to treat cancers.

It is five years since I began researching alternative/complementary cancer therapies. Did I find a single magic bullet? No, but then I no longer believe in that concept. I think each patient may find something that is right for them. I wish there were tests devised to tell us who might benefit from which treatment, including the conventional ones. In fact, I deplore the idea that we cannot distinguish the patients for whom chemotherapy

is effective from those who are simply harming their bodies with no gain. This is an area our tax dollars should pay to explore. After all most cancer patients are given the conventional treatments and many still die.

Many people with cancer call me for information and advice. I tell them I am not a doctor and I don't have any answers. But what I do know about are possibilities. And there are many. Only if we know what is available is the concept of informed choice fully functioning. Materials should be in every surgeon and oncologists office so that patients have immediate access to choices in treatment.

Yes, in some cases when a patient is deemed terminal, the doctor will not object if the family comes up with something to try that is out of the norm. But rarely will they know enough about the possibilities to offer advice. It is almost as if they are wearing blinders. No matter how many patients die of their disease, the physician has no personal responsibility to explore the options.

An additional torment is the insurance question. We may seek and try several options. They are almost always less expensive than conventional treatments but receive not a penny in coverage. I personally spent $27,000 on a five week trip to a clinic in Mexico. My insurance company would not even cover the blood tests I received, nor did they pay to have a catheter inserted so that I could have certain treatments. When I returned to New York, the surgery to remove the catheter was fully covered. Comparing the costs, I noticed that a one hour and 15 minute surgery in New York cost me $7000. Four hours of surgery would have cost as much as the five week stay which I credit with helping me regain control of my health. Of course the surgery would have been fully covered.
Another aspect that is rarely addressed is that fact that many oncologists prescribe drugs in what is called an "off-label" use. This means they follow hunches and not accepted protocol. If they are willing to do this with chemotherapy drugs, why not expand the horizons to allow use of non-toxic treatments? Although few medical schools offer coursework on nutritional issues, complementary or natural medicine, continuing education courses are now available. Of course, there is not much money to be made from natural substances.

Why haven't we heard about treatments that are used in other countries? Germany uses herbs and homeopathy as does France. In China, cancer treatments normally combine herbs with the standard protocol, while Japan has pioneered the use of medicinal mushrooms. American doctors need to expanded their vision.
People with cancer and their families are looking for doctors who will respond to questions about alternative and complementary treatments. We will no longer accept uninformed responses. Our lives are at stake and we need access to all medical options.

Ann E. Fonfa, 28 West 38th Street #12E New York, NY 10018
Ann Fonfa@aol.com

Mr. BURTON. Thank you, Ms. Fonfa. That was very interesting, and I would like—do we have copies of all their statements? I read your statement regarding vitamin A and vitamin E, and will ask you some questions later on during the hearing.

Ms. Siegel.

STATEMENT OF MARY JO SIEGEL

Ms. SIEGEL. I would like everybody to know that I feel very, very fortunate to be here today. Seven years ago, I was stricken with non-Hodgkins lymphoma, a cancer for which no conventional cure exists. My husband, Steve, and I were devastated by this prognosis, but determined to find a cure. We consulted top lymphoma specialists across the country, who confirmed that the disease was terminal.

The only hope, they said, was an experimental procedure called an autologous bone marrow transplant. I would receive extremely high-dose chemotherapy and as much radiation as people who were within 1 mile of ground zero at Hiroshima. I would become sterile, lose my hair, experience severe nausea and vomiting, and be kept in complete isolation for 6 weeks. There would be damage to my internal organs and a greater than 50 percent chance that I would develop leukemia. We decided to keep looking.

We soon discovered the work of Dr. Stanislaw Burzynski, who was treating advanced cancer patients with a gentile, nontoxic therapy. As I began Dr. Burzynski's treatment, malignant tumors were growing throughout my body, my bone marrow was infiltrated, and there was a large and growing tumor on the side of my neck. After only 3 weeks on this medicine, that tumor disappeared. Subsequent scans performed at UCLA showed continual reduction in tumor size.

During this treatment, my quality of life was excellent. I was an active, involved mother, raising three teenagers. Within 12 months, that same lymphoma expert at UCLA, who originally diagnosed me, pronounced me in remission. I went off the treatment.

Two years later, a followup scan revealed a return of the disease. Immediately, Dr. Burzynski prescribed a regimen of antineoplaston capsules. Within 5 months, I was once again in remission, and have remained cancer-free to this day.

The tragedy is that the FDA has been keeping what author Tom Elias calls "the century's most promising cancer treatment" from becoming widely available to cancer patients. The agency has spent millions to harass, discredit, and even imprison Dr. Burzynski.

In November 1995, the FDA indicted Dr. Burzynski on 75 criminal counts, most having to do with alleged technical violations of the Interstate Commerce Act, and none having to do with his practice of medicine or the effectiveness of his drug. In 20 years of practice, not one patient had ever filed a complaint. If Dr. Burzynski had been convicted, he could have been sentenced to 290 years in a Federal prison.

Are antineoplastons effective? Apparently, the FDA believes they are, because it fought tenaciously to keep the question of efficacy out of the trial. FDA also fought to keep the full truth from the jury by preventing Dr. Burzynski's patients from testifying. Thank-

fully, Dr. Burzynski was acquitted on all counts. FDA was unable to find even a single patient to testify against him.

The FDA lost the courtroom battle. Yet, it continues to wage war against Dr. Burzynski and his patients. The agency interferes in his practice by telling him who he can and cannot treat. With many types of cancer, the FDA requires patients to have failed not one, but two rounds of chemotherapy before they can be treated with antineoplastons.

FDA forbids the use of steroids in the treatment of Dr. Burzynski's lymphoma patients, when they are needed to temporarily shrink tumors and relieve pain. Because I was on treatment prior to the FDA involvement, Dr. Burzynski was able to prescribe Medrol to relieve the pain in my neck caused by that tumor. Now, however, the FDA's twisted logic dictates that good data collection outweighs humane medical treatment.

Did Congress give the FDA the right to play God? Was it your intent to give FDA the kind of power it exercises over life-and-death decisions with no accountability? FDA-approved remedies have failed to work for the majority of Dr. Burzynski's patients. Their only choice is antineoplastons or death. Shouldn't it be the doctor and patient making these important medical decisions rather than an FDA official?

It's time for a new approach to treating cancer, meaning expanded access to new, experimental, and innovative drugs. Until we have a cure, all of it, conventional and alternative, is experimental.

Even researchers in Japan are busily advancing research on antineoplastons with encouraging results. Doctors and scientists around the world eagerly await the approval of antineoplastons. So why is the FDA so determined to impede the progress of a drug with such promising results?

As a constituent of Mr. Waxman, I know he supports both the FDA and a woman's right to abortion, but how can he really condone a Government policy that grants a mother the right to choose death for fetus while denying a dying cancer patient one last hope for life? It's time for Congress in its oversight role to ensure that FDA carries out Clinton's March 29, 1996, promise to expedite the approval process for innovative, new cancer drugs like antineoplastons. The terminally ill deserve the chance to win their personal war on cancer, and it's up to this body to ensure they have the weaponry with which to fight.

Thank you.

[The prepared statement of Ms. Siegel follows:]

MARY JO SIEGEL

TESTIMONY BEFORE THE HOUSE GOVERNMENT REFORM
AND OVERSIGHT COMMITTEE

FEBRUARY 4, 1998

Seven years ago, I was stricken with a fatal cancer, non-Hodgkins lymphoma, for which no conventional cure yet exists. This disease is treatable for periods of time with chemotherapy and/or radiation, but the outcome is always death.

My husband Steve and I were devastated by my prognosis, but determined to find a cure. Our research took us to top lymphoma specialists at esteemed medical institutions like UCLA, USC, Stanford and the Dana Farber Cancer Institute in Boston. All the experts confirmed our worst fear. With existing therapies, my disease was incurable.

At Dana Farber, a ray of hope emerged with the recommendation that I undergo an autologous bone marrow transplant. This highly controversial procedure would require that I receive extremely high-dose chemotherapy and as much radiation as people who were within one mile of **"ground zero"** at Hiroshima. I would lose my hair, experience severe nausea and vomiting, and the threat of bacterial and viral infection would keep me in **complete isolation** for 6 weeks. My quality of life, post treatment, would be drastically diminished. From the chemotherapy I would become sterile. There would be damage to my heart, lungs, liver, kidneys, and bladder. Collateral radiation damage would affect my eyes, salivary glands and thyroid, with a greater than 50% chance that I would develop leukemia if I were lucky enough to survive just 10 years. I was frightened and suspicious because only a handful of patients had survived this procedure with good long-term results. One such person was the late Senator Paul Tsongas, who eventually died of complications caused by the procedure.

Fortunately, we discovered the work of Stanislaw Burzynski MD, Ph.D., who was treating advanced cancer patients with a gentle, non-toxic therapy he had discovered. As I began Dr. Burzynski's antineoplaston treatment, my lymphoma had progressed to stage 4 (there is no stage 5). Malignant tumors were growing throughout my body. My bone marrow was infiltrated and there was a large and growing tumor on the side of my neck.

After only 3 weeks on this medicine, that tumor disappeared! Subsequent scans performed at UCLA showed continual reduction in tumor size.

During antineoplaston treatment, my quality of life was excellent, virtually free of side effects. I was an active and involved mother, an absolute necessity when you are raising 3 teenagers. More importantly, the drug stopped my supposedly terminal cancer. Within 12 months I was pronounced in remission, not by Dr. Burzynski, but by the same lymphoma expert at UCLA who had originally diagnosed me and told me I faced certain death from this disease.

I went off treatment and remained in remission for 2 years, when a follow-up scan revealed a possible return of the disease. Immediately, Dr. Burzynski prescribed a regimen of antineoplaston capsules. Within 5 months I was once again in remission, and have remained cancer free to this day.

That's the end of the good news. The tragedy is that our government, namely the FDA, has been keeping what author Tom Elias calls "the century's most promising cancer treatment" from becoming widely available to cancer patients. The agency has spent untold millions of taxpayer dollars in a systematic attempt to harass, discredit, stonewall and even imprison Dr. Burzynski.

As incredible as it sounds, in November 1995, FDA indicted Dr. Burzynski on **75 criminal** counts, most having to do with alleged technical violations of the Interstate Commerce Act and none having to do with his practice of medicine or the effectiveness of his drug. Dr. Burzynski had been legally treating patients under Texas State law for some 20 years and not one patient in all that time had ever filed a complaint. If Dr. Burzynski had been convicted on all 75 counts, he could have been sentenced to **290 years in a federal prison**.

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Are antineoplastons effective? Ask the FDA. Apparently it believed the answer is

yes, because it fought tenaciously to keep the question of antineoplastons' effectiveness out of the trial. Dr. Burzynski tried to make it a part of the trial. Apparently, both the FDA and Dr. Burzynski believed he could prove the drug works. FDA also fought to keep the full truth from the jury by preventing Dr. Burzynski's patients from testifying, while Burzynski asked the judge to allow the patients to tell their stories.

In the end, **Dr. Burzynski was acquitted on all counts**. But I ask you in Congress, and particularly my representative, Mr. Waxman, how you can allow the FDA to squander taxpayer money in an idiotic prosecution, the success of

which would mean the deaths of hundreds of cancer patients? FDA was unable to find even a single patient to testify against Dr. Burzynski!

Peter Barton Hutt, a former FDA Chief Counsel, has said "if you beat the FDA in court, you have an angry FDA that is willing to slit your throat". Indeed, while it lost the courtroom battle against Dr. Burzynski, **it continues to wage war against him and his patients.** The agency interferes in his practice by telling him whom he can and cannot treat. With many types of cancer, the FDA requires patients to have failed not one, but **two** rounds of chemotherapy before they can be treated with antineoplastons. In many cases the chemo has so ravaged their immune systems, they literally have nothing left to fight with and they die.

FDA forbids the use of steroids in the treatment of Dr. Burzynski's lymphoma patients, even when they are needed to temporarily shrink tumors and relieve pain, as in my own case. Because I was on treatment prior to the FDA taking over his practice of medicine, Dr. Burzynski was able to inject me with "Medrol" to relieve pain and tightness in my neck caused by the tumor. Now, however, the FDA is not concerned with patient comfort. Their twisted logic dictates that good data collection outweighs humane medical treatment.

The FDA demands that Dr. Burzynski's lymphoma patients stop treatment if they have not achieved 50% tumor reduction within 6 months. The absurdity of this typically arbitrary FDA requirement became clear when one Burzynski patient—Frances Langham—was to be forced off treatment when she achieved a **44%** reduction after 6 months! She is lucky to be from Arkansas and politically connected. She received a "special dispensation" allowing her to continue treatment. But the FDA removed her from the clinical trial, meaning that even if

cured in the future, FDA will count her as a "treatment failure" in determining how effective Antineoplastons are! These treatment "restrictions" are only applied to Dr. Burzynski's clinical trials, whereas lymphoma patients involved in Idec Pharmaceutical's C2B8 and Elan Pharmaceutical's phenylacetate trials do not have to meet these same treatment criteria. Is it possible that FDA has a bias against Dr. Burzynski and his patients have to suffer as a result?

Who gave the FDA the right to play God? Was it the intent of Congress to give FDA the kind of power it exercises over life and death with no accountability? By denying terminally ill cancer patients access to antineoplastons, this agency literally decides "who shall live and who shall die".

I have had to watch as children and adults suffer and die as a result of FDA intransigence. Patients plead to be allowed into antineoplaston clinical trials, but FDA says "no you don't qualify." **Shouldn't it be the doctor, in concert with the patient, making these important medical treatment decisions, rather than an FDA official who doesn't even know the case?** Clearly the FDA is denying these patients their **freedom of medical choice**. Because conventional, FDA-approved remedies have failed to work for the majority of Dr. Burzynski's patients, often **their only choice is antineoplastons or death!**

It's been 26 years since President Nixon declared the "War on Cancer." Public expenditures now exceed 30 billion dollars and private research and development funds must total at least 10 times that amount, yet the death rate continues its relentless climb. It's time for a new approach to treating cancer. The only way this will become reality is by allowing cancer patients expanded access to new, experimental and innovative treatments. **Until we have a cure, all of it, conventional and alternative is experimental!**

Dr. Nicholas Patronas, chief of Neuro-Radiology at NCI, testified under oath that **antineoplastons are the most effective treatment for brain tumors he has ever seen.** Top oncologists have lauded Dr. Burzynski's work, including those at the University of Washington and Georgetown University. Doctors and scientists around the world eagerly await the approval of antineoplastons. Dr. Michael Friedman, the current commissioner of the FDA once wrote that **"Antineoplastons deserve a closer look...the human brain tumor responses are real."** So why is FDA so determined to impede the progress of a drug with such promising results?

Congressmen, we implore you to restore the right to choose our own health care. You have the power to give us back our freedom. Mr. Waxman, as your constituent I know you staunchly support both the FDA and a woman's right to an abortion. ***But can you really condone a government policy which grants a mother the right to choose death for her fetus, while denying a dying cancer patient one last hope for life?***

In his March 29, 1996 press conference, President Clinton announced new initiatives to expedite the approval process for innovative new cancer drugs like antineoplastons. Since then FDA has bluntly stated that the President's initiative has changed **nothing.** It's time for congressional oversight to insure that mandate is carried out. **The terminally ill deserve the chance to win their personal war on cancer** and it's up to Congress to insure they have the weaponry with which to fight. Thank you.

Mr. BURTON. I'm glad that you're doing so well, and we really appreciate your testimony, and I will make sure Mr. Waxman gets a copy of it.

Ms. SIEGEL. Thank you.

Mr. BURTON. Ms. Sherman. Would you pull the microphone as close as you can? Because those microphones don't pick it up as well as they should.

STATEMENT OF GENEVIEVE SHERMAN

Ms. SHERMAN. All right. This is directed to Mr. Chairman and members of the committee.

Mr. BURTON. Excuse me just 1 second. Ms. Siegel, who's that nice-looking gentleman behind you? [Laughter.]

Ms. SIEGEL. That's my husband.

Mr. BURTON. I had a feeling. [Laughter.]

OK. Please continue.

Ms. SHERMAN. My name is Genevieve Sherman. I live in Haddonfield, NJ, and I am very grateful to have been given the opportunity to be present today—grateful not only for the opportunity to speak to you about alternative medicine, but grateful to be alive. You see, I am a cancer survivor—not a survivor by chance, but a survivor by choice. My choice was in direct defiance of mainstream medicine's recommendation for breast cancer treatment. My choice was alternative treatment, and it is that choice, and my right as a U.S. citizen to receive it, that brings me here today.

Please allow me to give you a brief summary of the circumstances which lead me to this choice. In January 1991, I was diagnosed with stage four breast cancer with lymph node involvement. My surgeon removed the right breast and lymph nodes and referred me to an oncologist at Jefferson Hospital for followup treatment. He stated that inasmuch as nine lymph nodes had been affected, and given the aggressive nature of my cancer, my survival depended solely on the outcome of chemotherapy. I stated I would have to think about the treatment. Neither the doctor nor medical science could assure me success. My friends' experiences and my own knowledge of the chemotherapy was a dark prospect at a time when not only living or surviving counted, but the quality of life was essential for me. Chemotherapy is not a sure bet, and its ravishing effects on the body and the body's immune system can be devastating.

My daughters had read about a renown physician in New York who was a strong advocate of alternative and complementary medicine. I then went to see him. We discussed the alternative treatments that he felt would be effective in my case. He stressed that I would be an active participant in my cure. That was the key word, "active." He assured me that I would not experience the debilitating effects of chemotherapy—no hair loss, no nausea, no stomatitis, no weight loss, no chronic diarrhea or crushing fatigue. It was at that point that I made the most important decision of my life. My oncologist was strongly opposed and more or less had washed his hands of me when learning of my decision.

I would now be traveling to New York from New Jersey three times a week initially to be monitored and receive various alternative treatments consisting of supplements to strengthen my im-

mune system; IVs with cancer-fighting alternative treatments, nutritional counseling, and various other herbal and plant preparations. My bloodwork was monitored on a regular basis. My diet was addressed as well as my emotional state. It was a total and complete approach to fighting and beating cancer. At the clinic the mood was always high. People from all over the country had placed their faith in alternative medicine when mainstream medicine had failed them or turned them away to, quote, "finish their personal business." People of all ages, backgrounds, and life experiences getting better with no ill effects—yes, there were all challenges and at times a setback or two, but overall there was success for all different types of illnesses, not just cancer.

Oh, and I want to strongly point out that during my 7½ years of treatment, going on 8, I never experienced any reaction to any of the treatments, no side effects ever. I've never had any constraint on any of my fun things, and certainly led a very active life.

My cancer markers began to drop to within normal range, and my liver and bone scans remained negative. My spirits soared, and I had hope for the first time since being diagnosed. I was able to be treated for cancer and still remain in a vital, active personal relationship with all my friends and my family.

It has been almost 8 years since I first began treatment, and I remain cancer-free. Of course, I continue treatment with my physician in New York, but my visits are few and far between. The treatment protocol is constantly monitored and adjusted to address my current needs. My choice had paid off. I am alive and enjoying my family, friends, and all that life for an almost 78-year-old woman has to offer—thanks to the brave and pioneering few in medicine who choose to offer a safe and healthier approach to life and health.

Now, I must relate the only down sides to alternative medicine, which is extremely bothersome to those who have chosen this route. Recently, I was advised that any cancer treatment medicine for the IVs I had been receiving through a local physician, who consults with my primary physician in New York, Dr. Robert Atkins, could no longer be sent through the mail. And, incidentally, I have to go up to New York to get the medicines, take them home, and then take them to the other physician, so that I can be treated intravenously. He, this one physician, is afraid to have any cancer treatment type of medicine on his shelves for fear the FDA or someone will come in and put him out of business. I truly feel it is my choice and my right to receive them.

I am also deeply concerned that none of these therapies are covered by Medicare or any other insurance. We put out about $25,000 to $30,000 on just the vitamins. My husband, age 77, has to continue to work in order that I may continue my life-saving treatments. We have depleted all of our life savings in order that I may have the right to choose the course of my cancer treatment.

My concern is for those who do not have the resources to provide them the option of choosing. These therapies allow the human being to remain a productive person while treating. Chemo and radiation do not. Each week I sit in my doctor's office with as many as 26 or more patients who are recipients of chelation or other alternative therapies. Naturally, we are constantly discussing the

merits of these treatments. However, we all share a sense of despair that these treatments are not more easily accessible to those in great need of them or that at some time or another they may be unavailable to us or our loved ones altogether. Either the cost will prohibit it or it will be made unavailable to us because of FDA regulations.

These issues must be addressed and remedied as soon as possible. Every American man, woman, and child has the right to choose and receive the course of medical treatment they feel best suits their lives. It should not be the Government or the insurance companies' right to deem what choices a person will have. Currently, the success rate for such traditional treatments as chemotherapy and radiation is not exactly high. Yet, these treatments are covered by insurance. Alternative therapy quite often allows the patient to remain working without the need for assistance. In my opinion, this seems a considerable cost-effective reason for covering alternative treatments—only one of the many reasons. That is why it is imperative that more must be done to make alternative medicine accessible to the American public.

And I do thank you for giving me this opportunity to speak with you today and share my thoughts.

[The prepared statement of Ms. Sherman follows:]

January 26. 1998

Congress of the United States
House of Representatives
Committee on Government Reform and Oversight
2157 Rayburn House Office Building
Washington, D.C. 20515-6143

Dear Mr. Chairman and Members of the Committee;

1 My name is Genevieve Sherman. I live in Haddonfield, New Jersey and I am very grateful to have been given the opportunity to be present today. Grateful not only for the opportunity to speak to you about alternative medicine but grateful to be alive. You see, I am a cancer survivor. Not a survivor by chance, but a survivor by choice, My choice was in direct defiance of mainstream medicine's recommendation for breast cancer treatment. My choice was alternative treatment and it is that choice and my right as a U.S. citizen to receive it, that brings me here today.

Please allow me to give you a brief summary of the circumstances which lead me to this choice. In January of 1991, I was diagnosed with stage four breast cancer with lymph node involvement. My surgeon|removed the right breast and lymph nodes and referred me to an oncologist at Jefferson University for follow-up treatment. He stated that inasmuch as nine lymph nodes had been affected and given the aggressive nature of my cancer my survival depended solely on the outcome of chemotherapy. I stated I would have to think about the treatment. Neither the doctor nor medica1 science could assure me success, My friend's experienced and my own knowledge of the chemotherapy was a dark prospect at a time when not only living or surviving counted but the quality of life was essential for me. Chemotherapy is not a sure bet and it's ravaging effects on the body and the body's immune system can be devastating.

My daughters had read about a renowned physician in New York who was a strong advocate of alternative and complementary medicine. I then went to see him. We discussed the alternative treatments that he felt would be effective in my case. He stressed that I would by an active participant in my cure. That was the key word... active. He assured me that I would not experience the debilitating effects of chemotherapy. No hair loss, no nausea, no stomatitis, no weight loss, no chronic diarrhea or crushing fatigue. It was at that point that I made the most important decision of my life. My oncologist was strongly opposed and more or less had washed his hands of me when learning of my decision.

2

I would now be traveling to NewYork from New Jersey three times a week initially to be monitored and receive various alternative treatments consisting of supplements to strengthen my immune system; I.V.'s with cancer fighting alternative treatments, nutritional counseling and various other herbal and plant preparations. My bloodwork was monitored on a regular basis. My diet was addressed as well as my emotional state. It was a total and complete approach to fighting and beating cancer. At the clinic the mood was always high. People from all over the counrty had placed their faith in alternative medicine when mainstream medicine had failed them or turned them away to quote "finish their personal business" People of all ages, backgrounds and life experiences getting better with no ill effects. Yes, there were challenges and at times a set back or two, but overall there was success for all different types of illnesses; not just cancer.

My cancer markers began to drop to within normal range and my liver and bone scans remained negative. My spirit soared and I had hope for the first time since being diagnosed. I was able to be treated for cancer and still remain a vital, active person! It has been almost eight years since I first began treatment and I remain cancer free. Of course I continue treatment with my physician in New York but my visits are few and far between. The treatment protocol is constantly monitored and adjusted to address my current needs. My choice had paid off...I am alive and enjoying family, friends and a l l that life for a 77 year old woman has to offer. Thanks to the brave and pioneering few in medicine who choose to offer a safe and healthier approach to life and health.

Now I must relate the only downsides to alternative medicine, which is,extremely bothersome to those who have choosen this route. Recently, I was advised that any cancer treatment medicine for the I.V.'s I had been receiving through a local physician, who consults with my primary physician in New York, Dr. Robert Atkins, could no longer be sent through the mail. These treatments are crucial to the health of chose depending on them. It is already difficult enough to receive some of the more unconventional treatments because of restrictions placed upon them. I truly feel it is my choice my right to receive them.

I am also deeply concerned that none of these therapies are covered by Medicare or any other insurance. My husband (age 77) has to continue to work in order that I may continue my life-saving treatments. We have depleted all of our life savings in order that may have the right to choose the course of my cancer treatment. My concern is for those who do not have the resources to provide them the option of choosing. These therapies allow the human being to remain a productive person while treating. Chemo and radiation to not. Each week I sit in my doctor's office with as many as 26 or more patients who are recipients of chelation or other alternative therapies. Naturally, we are constantly discussing the merits of these treatments. However, we all share a sense of despair that these treatments are not more easily accessible to those in great need of them or that at some time or another they may be unavailable to us or our loved ones altogether. Either the cost will prohibit it or it will be made unavailable to us because of FDA regulations. These issues must be addressed and remedies soon as possible. Every American man, woman and child has the right to choose and receive the course of medical treatment they feel best suites their lives. It should not be the government or the insurance companies right to deem

what choices a person will have. Currently the success rate for such traditional treatments as chemotherapy and radiation is not exactly high, yet these treatments are covered. Alternative. therapy quite often allows the patient to remain working without the need for assistance. In my opinion, this seems a considerable cost-effective reason for covering alternative treatments. Only one of the many reasons. That is why it is imperative that more must be done to make alternative medicine accessible to the American public. I thank you for giving me this opportunity to speak to you today.

Genevieve J. Sherman
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Genevieve J. Sherman

Mr. BURTON. Thank you, Ms. Sherman. You don't look like you're 77. [Laughter.]

Ms. SHERMAN. Yes, I'll be 78 April 1st. [Laughter.]

Mr. BURTON. Happy birthday.

Ms. SHERMAN. And loving it. Pardon?

Mr. BURTON. I said, happy birthday.

Ms. SHERMAN. Thank you very much.

Mr. BURTON. Ms. Nippert.

STATEMENT OF BECKY NIPPERT

Ms. NIPPERT. Yes, I'm Becky Nippert. I'm 44 years old. I'm from Memphis, TN. I'm a single parent of three children. I became an RN in 1975 and a nurse and anesthetist in 1982. In 1989, I was diagnosed with breast cancer. I had a mastectomy surgery and 6 months of chemotherapy, and I got very sick and did lose my hair. That same year I had reconstruction surgery.

In 1994, I got a recurrence in my bones. At first it was three bones, and then they gave me a less than 1 percent chance of survival. My tumor markers were elevated, and I went for chemo, first of three treatments preparing me for the bone marrow transplant. My 1 week in the hospital cost $33,000. The chemo wasn't working because in 7 weeks my tumors went from 3 to 13 bone tumors, and my tumor marker was elevated. So since it wasn't working, I looked into alternative therapy, and I heard about a promising one down in Freeport, Bahamas. It's not the one that everyone knows down there. It was another one that was just being given down there for a while.

I went down there, and over the next few months I came home and I'd go back down there and get some more and bring it back home, and I was treated by a doctor here. Over the next few months, my tumors started disappearing on the bone scans and my tumor markers went down.

In September 1995, I called the company to order new medicine. I was told that the FDA had raided the company and there would be no more distribution of this therapy in this country anymore. Confused, scared, and angry, I made several calls to the FDA. I wrote President Clinton a letter. I wrote the FDA several times. I wrote the Commissioner of the FDA several times and appealed to him on every kind of level, even sending pictures of my children, and never heard from him. I wrote some Senators. I got no answers and no help. And to this day, 2½ years later, there has been no explanation. I didn't know if it was the company. I didn't know if it was the FDA. All I know is that no one was there for the patients.

I did get a small supply of the medicine from a nearby hospital for a while. My doctor would not treat me. So being a nurse, I had to start the IVs on myself. Sometimes I stuck myself up to nine times in one night. It was pretty much of a blood bath, but this is what I had to resort to.

In February 1996, I came into this building, to a subcommittee of the Commerce Committee. There were four of us that testified on this particular therapy. Two came to lend the moral support that were also patients. Three of those patients are dead today. One is on his death bed right now. The two of us that are still alive

were on the medicine the longest time. We were all given the death sentence by conventional medicine, and we were doing well when our medicine was pulled. The only thing these patients were protected from was hope and life.

Being a cancer veteran, I'm tired of watching patients die from conventional treatment. Being a registered nurse and a nurse anesthetist, I tried the conventional therapy first. Most recently, I've been on a treatment from Germany, and I'm very thankful to God to report that, since last summer, I've had clear scans and am cancer-free.

I've been working full time for over a year after being on disability for 22 months. I'm alive today in spite of feeling restricted and abandoned by the Government in this "free" United States, while others have not been so fortunate. I'm alive today because of prayer and alternative medicine.

One thing that really bothered me really bad was that none of the doctors that treated me—and I knew them well—ever asked what cured me. When patients are not helped by standard treatment, many are glad to be guinea pigs on treatments that show promise. I know I was. We think, what do we have to lose? And maybe we have something to gain. We know better than anyone else that we might die. We sign all the responsibilities back on ourselves.

I was wondering, could we do clinical trials on humans in this situation? We could advance medicine and find out what works on different types of cancer in the process. I'm tired of mediocrity; I'm tired of maintaining the status quo; I'm tired of falling behind, and definitely not being on the cutting edge. We put anyone down that has an individual idea and we don't give them a chance. We don't branch out into areas we don't fully understand, and we stay in our comfort zones, at the expense of people's lives.

Conventional medicine has been in control for the last 50 to 100 years. More people are getting cancer now than ever before, and more people are dying from cancer now than ever before. I say, stop the insanity. "Insanity" defined is doing the same thing over and over, expecting different results.

Please stop giving the research grants in the same areas. The trials are expensive and lengthy, and only the last phase of the trials are on humans, and they're all in the same dismal, conventional bias. It's not working.

The problem summarized is: It's hard for patients to find out about alternative therapies in this country. The doctors don't know about them, and even if they did, they can't really treat the patients here because they could get in trouble or get their license removed, which many have.

The patients, many of them or most of them, are too weak to travel to other countries, and they lack followup when they get back home. Also, as I've said, the grants are given in the same areas. There's no branching out.

And last, but not least, what one of the others has covered: There's no insurance coverage. Everything is out of pocket.

I was one, by God's grace, who slipped through the cracks. I believe God spared me to speak for those who can't speak for themselves. Please don't turn your back to the problem and neglect so

great a need. Don't wait until it happens to your husband, your wife, your child, or your prostate or breast. I plead with you, help patients get other therapies that may save their lives.

And I do appreciate and thank you for giving me the opportunity to testify.

[The prepared statement of Ms. Nippert follows:]

TESTIMONY OF BECKY NIPPERT

My name is Becky Nippert. I am forty-four years old and live in Memphis (Germantown), Tennessee. In '75 I became a registered nurse, and in '82 I became a nurse anesthetist. In '89 when I was 35 years old, I was diagnosed with breast cancer. At this time I was married with three small children, age: 5, 3 and 5 months. I had a mastectomy, placement of a Hickman catheter to do six months of intense chemotherapy and two breast reconstructive surgeries that year. I was very sick from treatment and lost my hair. In '93 the cancer returned in the same area. The surgery I had removed much of the chest muscle to prepare me for the 37 radiation treatments I received while working full-time and raising my three children as a single parent. My marriage ended in divorce four months before the recurrence. While I took radiation, I was put on the drug Tamoxifen, which was short-lived because of the severe side effects. Six months after finishing radiation, I had another reconstructive surgery.

Seven weeks later, in August '94, I went for my cancer checkup and reported to my doctor rib pain, general loss of a sense of well-being and fatigue. He checked the tumor marker Ca 15-3, which is a blood test that, when elevated, indicates cancer activity in the blood. Normal is 3 to 27. My previous results had never been elevated. The test result was 49. An enlarged lymph node had come up in my neck and my doctor ordered a bone scan which showed tumor invasion in my sternum and two ribs. I got a rib biopsy on the rib which was fractured by cancer activity and it confirmed metastatic breast cancer to the bones. He recommended the only thing left, the stem cell rescue or bone-marrow transplant. I had heard of some success with this for lymphomas or leukemias, but in all the patients I talked to with breast cancer who had this therapy, none had a good result from it.

All reported recurrences rather quickly after that or their families reported they had passed. This is an accepted treatment covered by insurance. A second opinion was to get my ovaries out, get my affairs in order and get the bone-marrow transplant. My original tumor was estrogen receptor positive, which means tumor growth is aggravated and enhanced by the presence of estrogen. I had ovary surgery, thereby cutting out most of the estrogen production in my body. I did get my affairs in order and this relieved me of some stress. While I was thinking about the bone-marrow transplant and weighing what to do, I started the first three standard chemo treatments to prepare me for it. The hospital where I went put me on the bone-marrow transplant unit. The person in the room next to me was on kidney dialysis for shutdown of her kidneys secondary to the bone-marrow transplant. The patient in the next room was sent to the intensive care unit for septre shock. The 32-year old female patient next to her was rocking back and forth on her bed babbling to herself. When I asked what was wrong with her, I was told she had been isolated 52 days and was in an isolation psychosis, but she would probably eventually be all right. After this chemo, my tumor markers went up to 59, and in the seven weeks since my bone recurrence, my tumor load had increased from 3 to 13 bone tumors. My bill for one week in the hospital for chemotherapy was $33,000.00. At this point I heard about an experimental treatment that had positive results from trials in Monterrey, Mexico. Trials had also started in Freeport, Bahamas. Since the oncology experts had given me a less than one percent for survival, and I didn't see how the bone-marrow transplant could help, but only harm me further, I decided to try the treatment. I went to Freeport and got on this therapy, which was to be given by IV three times a week after I go back home.

After being on the therapy two weeks, my tumor markers went down to 49 and in the next few months they went to 41 to 29 to21 and then down in the teens and to this day have not been elevated. My tumors started to disappear on the bone scans. I aborted the pursuit of the bone-marrow transplant. My quality of life was good and most of my bone pain subsided. In September '95, when I was trying to order new treatment from the company, I was told after repeated attempts to reach them that the company had been raided by the F.D.A. and there would be no more distribution of this treatment in this country again. No explanations were given. I had been cut off from the therapy that was making me well. I wrote President Clinton and received a form letter from the White House. I wrote the Commissioner of the F.D.A., David Kessler, several times and never personally heard from him, but got form letters from the F.D.A. The last one I received said that they were sorry that they could be of no further assistance to me. I wrote my Senators and Congressmen. To this day, two and a half years later, I still don't know what happened. I was able to get some extra treatment from a nearby hospital which ad used it prior to the F.D.A shutdown.

My local doctor didn't feel like he could treat me anymore. I asked the doctor in Freeport, Bahamas, to let me have some of the medicine. I knew there were about 2000 doses of the treatment in a freezer in that medical clinic down there. I told her I would keep quiet and would move myself and children down there. She said no. I found out that the F.D.A. had gone down to question her and investigate. This is the same agency I called and could never get an answer that they even knew anything about it. I was able to get a small supply and a freezer to keep in my home. Three times a week I struggled to get IVS started on myself to administer the medicine. Many nights it was a blood bath, sticking myself up to nine times. Thank God I was a nurse with the ability to do this. When the medicine was gone, I still had three bone tumors.

In February '96, I came to this building and appeared before a subcommittee at a hearing of the Commerce Committee to gain access to the treatment that had been shutdown. Four of us patients on this specific therapy testified and two other patients came to lend moral support. We pleaded to get our treatment back. We all were recovering and doing well on it. Nothing happened to help us and today three of those patients are dead and one on his death bed. Only two of us are still alive, and we were the ones that were able to stay on the medicine longer than the others. We were all given the death sentence by conventional medicine. We tried an unconventional new therapy. We were getting our lives back and doing well when the medicine was pulled with no explanation or help from anyone. This negligence killed them. In my field it is called malpractice.

There was no one we could find to help us as patients. I heard on the second panel that today there is a lawyer who represents patients to the F.D.A. Why, in the run-around and phone tag with all these important people, were we not told about him? Is this something new or something that was not available two years ago? No one was there for us. One person on that committee said they wanted to do no harm. Let me tell you what goes on down in the trenches. I am a cancer veteran. I have been there and done that. My friend, Rosemary, got some metastasis in her lung from breast cancer. She got the stem cell rescue at Christmas and died New Year's eve of liver failure, not from the cancer, but the treatment. She is dead at 42-years old. Merrilee Malcom, my friend from Atlanta, wanted alternative therapy and begged for it after being refractory to standard therapy. She died at age 32. She was written off and not helped after chemo. They told her she failed therapy. She did not fail chemo. Chemo failed her. Please wake up. Many more people have died from cancer than

in the holocaust, and we are even having to debate this? We, as a country, have failed miserably in this area.

I have been a nurse and a nurse anesthetist my whole adult life. I tried everything conventional first. It didn't work and when I found something that did, my government cut it off with no provisions for the patients that were benefitting from it. Many patients that were doing well on this treatment have died. The only thing they were protected from was hope and life. Since then I have been on a treatment from Germany. I have been totally cancer free with clear scans since last summer. I am alive today because of prayer and alternative treatments. At this point give us the treatments we need. Remember we were written off and given the death sentence. Is this inhumane to do research on humans? I was glad to be a guinea pig. Speaking as a guinea pig and cancer patient, I say no. We have nothing to lose and maybe something to gain. We can advance medicine in the process.

We don't have a powerful lobby like the pharmaceutical companies. After we are diagnosed, cut on, treated with poison and then radiated, most of us are worn out. We have then become victims of the cancer industry and most can't speak for themselves anymore because they have been beaten down in every area. Conventional therapy has had total control from 50 to 100 years. We have more cancer and more people dying from cancer than ever before. We are not exploring the unknown or allowing people with different opinions a chance. We only go for mediocrity and maintaining the status quo. We are falling behind and are definitely not on the cutting edge. We put down anyone with an individual idea or something new and original. We stay in our comfort zones at the expense of people's lives. We don't branch out into areas we don't fully understand. How can we when the requirement to approved treatment is $240,000,000 and ten years of trials, the last of which is on humans.

Overseas where the medical giants are, they are allowed to use different procedures without these almost totally impossible requirements. Many people here are too weak to travel to other countries or can't afford it. I, and many others, would like therapies with decreased side effects. Give us the choice. Medicine can be advanced so that we can find out what works well on different types of cancer. Why not give us our treatment of choice? Remember we are talking mainly of people here who are at the end-stage. What do we and what do you have to lose? The patients, more than anyone else, know they may die. They have already been written off. Open up to more than one bias and give other types of therapies a chance. It has to come from you. The doctors just can't start doing it unless it is approved by you.

I have a friend in Seattle, Washington, who is a radiation oncologist, and lost his medical license for using alternative therapies in his practice. He gave his patients choices and they did well. When he wanted to appeal to the Supreme Court, he was told he couldn't do that because it was against the ruling of the American Medical Association, the American Cancer Society and the National Health Institute. They are basically immune and have no accountability. This is where it ended. The heads of these and the H.M.O.s, who now control medicine, make seven-figure salaries. They will keep giving grants in the same areas, and we aren't doing anything but advancing. Please open your eyes. I am alive today in spite of being abandoned and restricted by the government while others have not been so fortunate. I was on my own fighting to stay alive with no help at all from this free United

States, only obstacles. This is the ultimate injustice.

Please spare me the cliche "that you don't want the patients to be harmed." I am today cancer-free, and have been working full-time doing anesthesia again for over a year after having been on disability for 22 months. I believe God spared me to speak for those who can't speak for themselves. By my story, you can see the near impossibility to get anything other than conventional therapy. Please don't turn your back to the problem and neglect so great a need. Don't wait until it happens to your wife, husband or child, or your prostate or breast. Don't wait until your back is against the wall or your neck on the line. I plead with you: Help patients get other therapies that may save their lives.

Mr. BURTON. Thank you, Ms. Nippert.

I want to apologize to our panelists for not having more Members here. I appreciate my colleague for being here. We ought to have every Member here. We've had a number of hearings on more sensational subjects, and everybody's been in attendance. And I don't know of any hearing that's as important as the one we're having today, because it does affect lives across this country. So I'm a little disappointed we don't have more Members here, but I can assure you that we'll make sure they all get copies of this, and I'll try to make sure that, as an advocate for all of you, that we get the message out to all the other Members of Congress, because I have a personal stake in it as well.

Mr. Kunnari.

STATEMENT OF JACK KUNNARI, ACCOMPANIED BY DUSTIN KUNNARI, JACK KUNNARI'S 6 YEAR OLD SON

Mr. JACK KUNNARI. Hello. My name is Jack Kunnari. This is my son, Dustin, and the lovely lady behind me is my wife, Maryann.

It is a privilege to testify here today, but it also troubles me that we need congressional hearings and new legislation to give us back our constitutional rights. The Declaration of Independence states that, "we are endowed by our Creator with certain inalienable rights." Among these are "life, liberty, and the pursuit of happiness." This should also mean the pursuit of health. As I learned when my son, Dustin, became sick, this is a right we have lost.

Dustin was diagnosed exactly 4 years ago, at the age of 2½, with a deadly medulloblastoma brain tumor the size of a golf ball. This is the type of tumor that can grow real rapidly and cede into the spine. We were told that he had perhaps a year to live.

The neurosurgeon could only remove 75 percent of it. Radiation was out of the question because of his age; we were told it would leave him a vegetable.

We were then referred to the University of Minnesota for a clinical trial. A computer would randomly put Dustin on one of two highly toxic chemotherapy drugs. According to the informed consent, the side effects of these drugs included bone pain, hearing loss, irreversible damage to kidney and bladder, devastation of his immune system, learning disabilities, sterility, and leukemia. In addition, the doctors could not name a single child who had done well following any of these treatments.

We were told by one intern that, when we asked if there were some case histories of patients with similar type tumors as Dustin's and his age, if there were any case histories that they could refer to and give us an idea of how effective their treatment was—one intern came back and said, "I can think of one child that did well for a while." That was the answer we got.

When we told the doctors we would not subject him to such a cruel treatment with so little hope, they told us they could get a court order to treat Dustin as they wished. I told them, "You do what you have to do, but you will not treat my son with this treatment."

In April 1994, we visited Dr. Burzynski's clinic in Houston. The difference between it and the hospital was like night and day. Dr. Burzynski's patients were full of life and hope. Unlike the cancer

ward at the university, you could talk to patients who were getting better. Dr. Burzynski and his staff were very honest, courteous, and professional. Dr. Burzynski made no promises, but said he had good results with brain tumors, and agreed to treat Dustin for 6 weeks to see if antineoplastons would have any effect.

After 6 weeks, the MRI showed Dustin's tumor was completely gone. There was no trace whatsoever. In fact, I put the scan up on my living room window, before he started the antineoplastons and after, and we didn't have a doctor's report yet, but I called my wife in the room, and I said, I can see that there's no tumor; that was so clear. That was 6 weeks of treatment.

A year later, a second tumor about 1 inch by 1 inch in size appeared. Dr. Burzynski increased Dustin's dose of antineoplastons, and this tumor was dissolved in 5 months. Dustin continues in remission today and is off intravenous treatment. The antineoplastons caused no side effects whatsoever, and throughout the treatment Dustin was a healthy, happy, active child—no different from any other children, except for the backpack he always wore with his pump and antineoplastons.

From the time we went to Dr. Burzynski, the biggest threat to Dustin's life was not his cancer; it was the FDA. In February 1996, with cold disregard for the life of my son and Dr. Burzynski's other patients, the FDA used legal maneuvers to stop Dr. Burzynski's terminal cancer patients from receiving antineoplastons. If not for quick and compassionate action by Joe Barton and his Investigations and Oversight Subcommittee, FDA would have succeeded, and Dustin likely would not be here today.

Why did the FDA take this action? It was not because it suspected the drug doesn't work. In fact, the current head of the FDA, Dr. Michael Friedman, has written that Dr. Burzynski's "human brain tumor responses are real." It was not because his patients had a better treatment option available; Dustin had none. Incredibly, it was because these patients did not live in Texas, and had to cross State lines to be treated and to take the drug back home with them. FDA claimed this was a crime, regardless of whether or not the drug was saving people's lives.

As a result of its actions, Dr. Burzynski can now only treat patients with FDA's approval. The FDA has final say over medical decisions concerning Dr. Burzynski's patients, not Dr. Burzynski. Some of its rules and decisions are arbitrary and against patients' interests. It terrifies me to realize that under current rules Dustin would have to stop treatment when the second tumor appeared. The FDA forced Dusty to needlessly have three blood tests done every week. Twice he was taken off treatment because his sodium was 1 point too high. Dr. Burzynski felt that stopping the treatment was more dangerous than his slightly elevated sodium level, but the FDA overruled him. I would rather have Dr. Burzynski—a brilliant M.D./Ph.D. with a lifetime experience using antineoplastons—make that call than a faceless bureaucrat in faraway Rockville, MD, who has no experience with antineoplastons and has never met, much less treated, our son.

We are in a war against cancer, and the FDA never showed up. FDA claims that it is protecting cancer patients. We do not want your protection. Under your protection, cancer deaths climb every

year. In my opinion, the actions of your agency are un-American and unconstitutional. Our Constitution was supposed to assure people the liberty to make choices free and independent of Government bureaucrats. Thomas Jefferson wrote, "In questions of power let no more be heard of confidence in man, but bind him down from mischief by the chains of the Constitution." He also said, "The natural progress of things is for liberty to yield and for government to gain ground." The actions of the FDA are precisely the mischief Thomas Jefferson warned us against.

As Members of Congress, it is your duty to uphold the system of checks and balances. It is up to you to reign-in this FDA.

To the American citizens, I would say it is our duty to lead; our leaders' duty to follow. Perhaps we are all guilty of having not done our part in upholding the Constitution of the United States, and of letting our freedom slip.

Thank you, Congressman Burton and committee members, for hearing our concerns.

And I believe Dusty would like to say something also.

Mr. DUSTIN KUNNARI. Dr. Burzynski is my hero. I'm just a 6-year-old boy. If you take away my medicine, I might die.

[The prepared statement of Mr. Jack Kunnari follows:]

TESTIMONY OF JACK KUNNARI

FEBRUARY 4, 1998

Hello. My name is Jack Kunnari, and this is my son Dustin. It is a privilege to testify here today. But it also troubles me that we need Congressional hearings and new legislation to give us back our Constitutional rights. The Declaration of Independence states that "we are endowed by our creator with certain inalienable rights," including "Life, Liberty and the Pursuit of Happiness." That should also mean the pursuit of health. As I learned when my son Dustin became sick, this is a right we have lost.

Dustin was diagnosed exactly 4 years ago, at the age of 2-1/2, with a deadly medulloblastoma brain tumor the size of a golf ball. The neurosurgeon could only remove 75% of it. Radiation was out of the question because at his age, it would have left him a vegetable.

We were referred to the University of Minnesota for a clinical trial. A computer would randomly put Dustin on one of 3 highly-toxic chemotherapy drugs. According to the informed consent, the side effects of these drugs included bone pain, hearing loss, irreversible damage to kidney and bladder, destruction of his immune system, learning disabilities, sterility and leukemia. In addition, the doctors could not name a single child who had done well following any of these treatments.

When we told the doctors we would not subject him to such a cruel regimen with so little hope, they told us they could get a court order to treat Dustin as they wished. I told them to do what they had to, but they would not treat my son.

In April, 1994 we visited Dr. Burzynski's clinic in Houston. The difference between it and the hospital was like night and day. Dr. Burzynski's patients were full of life and hope. Unlike the cancer ward at the University, you could talk to patients

who were getting better. Dr Burzynski and his staff were very honest, courteous and professional. Dr. Burzynski made no promises, but said he had had good results with brain tumors, and agreed to treat Dustin for 6 weeks to see if Antineoplastons would have any effect.

After 6 weeks, the MRI showed Dustin's tumor was completely gone. But a year later a second tumor about one inch by one inch in size appeared. Dr. Burzynski increased Dustin's dose of Antineoplastons, which dissolved the tumor in 5 months.

Dustin continues in remission today, and is off intravenous treatment. The Antineoplastons caused no side effects whatsoever, and throughout the treatment Dustin was a healthy, happy, active child – no different from other children except for the backpack he always wore with his pump and the Antineoplastons.

From the time we went to Dr. Burzynski, the biggest threat to Dustin's life was not cancer, it was the FDA. In February of 1996, with cold disregard for the life of my son and Dr. Burzynski's other terminal cancer patients, FDA used legal maneuvers to stop them from receiving Antineoplastons. If not for quick and compassionate action by Congressman Joe Barton and his Investigations and Oversight Subcommittee, FDA would have succeeded. And Dustin would not be here today.

Why did FDA take this action? Not because it thinks the drug doesn't work. The current head of the FDA, Dr. Michael Friedman, has written that Dr. Burzynski's "human brain tumor responses are real." Not because those patients had a better treatment option available – Dustin had none. Incredibly, it was because these patients did not live in Texas, and had to cross state lines to take the drug back home with them. FDA claimed that was a crime, even if the drug was saving their lives.

As a result of the FDA's actions, Dr. Burzynski can now only treat patients with its approval. The FDA has final say over medical decisions concerning Dr. Burzynski's patients, not Dr. Burzynski. Some of its rules and decisions are arbitrary and against patients' interests. It terrifies me to realize that under current rules, Dustin would have had to stop treatment when his second tumor appeared. FDA forced Dustin to needlessly have 3 blood tests done every week. Twice he was taken off treatment because his sodium was one point too high. Dr. Burzynski felt that stopping the treatment was more dangerous than his slightly elevated sodium level, but the FDA overruled him. I would rather have Dr. Burzynski – a brilliant MD, Ph.D with a lifetime of experience using Antineoplastons – make that call than a faceless bureaucrat in faraway Rockville MD, who has no experience with Antineoplastons and has never treated our son.

FDA claims it is protecting cancer patients. We do not want your protection. Under your protection, cancer deaths climb every year. In my opinion, the actions of your agency are un-American and unconstitutional. Our Constitution was supposed to assure people the liberty to make choices free and independent of government bureaucrats.

Thomas Jefferson wrote, "In questions of power let no more be heard of confidence in man, but bind him down from mischief by the chains of the Constitution." He also said, "The natural progress of things is for liberty to yield and for government to gain ground." The FDA's actions are precisely the mischief Thomas Jefferson warned us against. As members of Congress it is your duty to uphold the system of checks and balances. It is up to you to reign in this FDA. To the American citizens, I would say it is our duty to lead and our

leaders' duty to follow. Perhaps we are all guilty of not having done our part in upholding the Constitution of the United States, and of letting our freedoms slip.

Thank you, Congressman Burton and committee members for hearing our concerns.

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APR 11 1995

Patient Name: DUSTIN KUNNARI DOB: 05/21/91

OUTPATIENT
Account Number: 00718756
Referring Physician: ROBERT T. RUTKA M.D.
Films and Additional Report To: S. BURZYNSKI M.D.
Date of Scan: 04/07/95
Type of Scan: MRI OF THE BRAIN

Pre-Scan Information: Resection posterior fossa medulloblastoma 02/28/94 St. Luke's Hospital. Patient is receiving experimental chemotherapy. Follow-up examinations at this institution que 3 to 4 months since 03/06/94.

Technical Information: Sagittal T1; axial two echo T2; axial, coronal, and sagittal post contrast T1 weighted images of the brain.

INTERPRETATION: The patient demonstrates regrowth of residual tumor in the left CP angle and far lateral lateral recess of the fourth ventricle. A 25 X 25 X 18 mm. tumor nidus fills in the caudal aspect of the left CP angle cistern posterior and inferior medial to the left internal auditory canal and left 7/8 neural complex. There is now modest mass effect on the lateral aspect of the upper medulla with a small "tongue" of tumor extending anterior to the left side of the upper caudal brainstem, as seen on axial image 6 on the post contrast T1 weighted images and in fact better seen on non-contrast axial proton density image 9. Proton density images suggest that the brainstem is in fact displaced slightly to the right. Tumor emerges from the lateral outlet of the fourth ventricular lateral recess and presents as a CP angle mass. There is no evidence for recurrence nor regrowth of the midline nor vermian component of the tumor and no demonstrated extension into the upper cervical canal. There is no evidence of residual nor recurrent tumor in the right ventricular lateral recess. Interestingly, the tumor does not enhance following contrast administration and in fact remains slightly hypointense to adjacent gray and white matter on all T1 weighted sequences (including the enhanced phase). This makes tumor detection difficult and the lesion is best appreciated on the proton density sequence where it is hyperintense to white matter and iso to slightly hyperintense to cortical gray matter.

The patients radiographic record is reviewed in retrospect. Original pre-operative scan demonstrates a midline tumor also extending laterally through the fourth ventricular lateral recesses to extend a short distance into the cerebellopontine angle cisterns, left greater than right. Operative note describes subtotal tumor removal with impression of extension into the left inferior cerebellar peduncle. It describes a "small rim" of tumor which could be visualized along the obex extending into the left inferior cerebellar peduncle which was cauterized with bipolar cautery.

Retrospective review of MRI studies beginning in June of 1994 through the current study are reviewed with specific attention to this area. Sagittal and axial images on 06/01/94 do demonstrate soft tissue asymmetry in this area with a small 7 to 8 mm. focus of tissue isointense with cerebellar gray matter. Retrospective measurements on 08/08/94 are 11 X 12 mm. and 19 X 19 mm. on 12/05/94. Again, current measurements are 25 X 25 X 18 mm.

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Patient Name: DUSTIN KUNNARI DOB: 05/21/91

OUTPATIENT
Account Number: 00718756
Referring Physician: ROBERT T. RUTKA M.D.
Films and Additional Report To: S. BURZYNSKI M.D.
Date of Scan: 05/22/95
Type of Scan: MRI OF THE BRAIN

Pre-Scan Information: Recheck left CP angle medulloblastoma. Comparison to recent study of 04/07/95, and previous examinations dating back to 02/25/94.

Technical Information: Sagittal T1; axial T2; coronal, axial, and sagittal post contrast T1 weighted images.

INTERPRETATION: Residual non-enhancing mass in the left cerebellar pontine angle emerging from the lateral recess and foramen of Luschka is again noted. The lesion is most conspicuous on proton density images and measures 23 X 23 mm. in dimension on that study. There is residual pontomedullary effacement laterally on the left with slight shift of the upper medulla to the right. A small nubbin of the tumor extends into the left fourth ventricular lateral recess. On sagittal views, the tumor extends anterior to the cerebellum and lies closely applied to the adjacent medial petrous margin just behind and inferior to the left cerebellar pontine angle. On T1 weighted images, tumor dimensions are slightly smaller measuring approximately 19 mm. in size. This presumably reflects marginal gliosis or infiltration of the tumor edges but otherwise, the lesion appears sharply demarcated from the adjacent normal brainstem and anterior cerebellar substance.

There is moderate residual hydrocephalus with ventricular shunt in place. Ventricular size is unchanged from the prior exam. There is mild posterior periventricular hyperintensity of the periventricular white matter consistent with posterior radiation effect.

No meningeal thickening nor enhancement is seen to implicate meningeal/subarachnoid spread of tumor.

CONCLUSION: Residual medulloblastoma and the left CP angle is unchanged in size and appearance since 04/07/95.

G. Alan Norris

G.A. Norris, M.D.
GAN/rkc
D&T: 05/22/95

RR
Jeremiah T Moore
5/25/95 RR
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Patient Name: DUSTIN KUNNARI DOB: 05/21/91

SEP - 1 1995

OUTPATIENT
Account Number: 00718756
Referring Physician: ROBERT T. RUTKA M.D.
Films and Additional Report To: S. BURZYNSKI M.D.
Date of Scan: 09/05/95
Type of Scan: MRI OF THE BRAIN

Pre-Scan Information: Follow-up brain tumor, left CP angle medulloblastoma.

Technical Information: Images were obtained sagittally with T1 weighting; axially with proton density and T2 weighting; axially with T1 weighting following gadolinium administration; sagittally with T1 weighting following gadolinium administration; and coronally with T1 weighting following gadolinium administration.

INTERPRETATION: The mass in the low cerebellar pontine angle adjacent to the foramen of Luschka has improved significantly from May 22, 1995. Only a small amount of residual tumor is seen at this site. Dilatation of the lateral third and fourth ventricles is again noted. This has remained unchanged from the previous examination. Shunt catheter is seen in the right frontal region. Probable venous angioma is seen in the left cerebellar hemisphere. No brainstem abnormalities are seen. Pituitary, optic chiasm, and basal cisterns all appear normal.

CONCLUSION: There has been significant regression in the cerebellar pontine angle mass on the left as compared to previous examination on May 22, 1995. Small amount of residual tumor persists.

Frank J. Suslavich, M.D.
FJS/rka
D&T: 09/05/95

Dictated But Not
Reviewed To
Expedite Mailing

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CONSENT FORM

CCG-9921
Multiagent Chemotherapy and Deferred Radiotherapy in Children Less Than 36 Months of Age With Malignant Brain Tumors

You are invited to be in a research study of chemotherapy for your child's brain tumor. Your child has been selected as a possible participant because of his/her age and type of brain tumor. We ask that you read this form and ask any questions you may have before agreeing to be in the study.

PURPOSE AND BENEFITS: Young children treated with radiation therapy for brain tumors may experience serious side effects from the radiation. The purpose of this study is to learn whether intensive chemotherapy following surgery can be used to treat tumors and therefore delay or avoid radiation therapy, and to determine the toxicities of two different drug programs.

Very young children with malignant brain tumors are at high risk that their tumors will continue to grow despite treatment with either chemotherapy or radiation or both. In order to develop more effective chemotherapeutic treatments, this study will randomize (assign by chance) children to receive one of two combinations of chemotherapy initially. One combination uses cytoxan, vincristine, VP 16 and cisplatin. The other regimen uses ifosfamide, vincristine, VP 16 and carboplatin. All of the drugs in either combination have been shown to be individually effective in some patients with brain tumors that have recurred following standard treatment, but we do not yet know how effective each combination of drugs is. One of the goals of the study is to determine which combination is most effective.

THERAPY PLAN: If you agree to participate in the study, we will do the following things.

Chemotherapy will begin as soon after surgery as possible, and no later than 28 days after surgery. Chemotherapy will be divided into two phases. The first phase, induction, will consist of five courses of drug treatment, each three weeks in duration (total of 105 days). Following the completion of the induction phase, if tumor remains and if in the judgment of your physician it can be removed surgically, an attempt will be made to do so. Your child will then proceed to receive maintenance chemotherapy, which will consist of eight courses, each 49 days in duration. Therefore, the total time in this study is approximately 1 1/4 years.

If your child has tumor persisting after the initial surgery and the induction chemotherapy, or if your child has metastatic disease (tumor spread throughout the brain and spinal cord) at the time of diagnosis, your child will receive irradiation. This will occur at the time your child reaches 36 months of age, or completes chemotherapy, whichever occurs first. If your child does not have metastatic disease at diagnosis and has no apparent tumor following the completion of induction chemotherapy, your child will not receive irradiation unless the tumor recurs.

RADIATION THERAPY (only if necessary): If your child does not have metastatic tumor at diagnosis, completes chemotherapy and has no evidence of remaining tumor, no radiation will be given unless the tumor begins to grow. If your child has completed induction chemotherapy with tumor still remaining and is over 36 months of age, or has completed maintenance chemotherapy if under 36 months of age, irradiation will be given at that time, and your child will be withdrawn from the study.

Irradiation will consist of craniospinal irradiation in patients with medulloblastoma or primitive neuroectodermal tumors or children with metastases throughout the central nervous system. For all other tumor types, the primary site only will be treated with radiation.

If the tumor grows in spite of chemotherapy, your child will receive irradiation even if he/she is less than 36 months of age.

PROCEDURES (before treatment): All patients, except those with brain stem tumors, will undergo a neurosurgical procedure in which an attempt will be made to surgically remove the tumor and, where this is not feasible, an attempt will be made to remove as much tumor as possible without placing the patient in jeopardy.

Computerized tomographic scans (CT scans) or magnetic resonance imaging scans (MRI scans) of the head with and without injection of contrast dye through a vein will be performed at diagnosis, within 72 hours after surgery, after the third course of induction, at the end of induction and at the beginning of 8 maintenance courses. After the completion of therapy, scans will be done every three months for two years following completion of radiation, then every six months for two years, then every year for six years. The location of the tumor and type of tumor will determine whether a CT or MRI is used.

A metrizamide myelogram (or spinal MRI), involving the injection of dye into the spinal fluid, will be done at diagnosis to determine if the tumor has spread (metastasized) from the brain.

A lumbar puncture and bone marrow aspirate will be done at the time of initial surgery to detect the presence of malignant cells in the bone marrow or spinal fluid. A bone marrow aspirate does not need to be done on patients with gliomas or brain stem tumors. A lumbar puncture (spinal tap) is done by inserting a needle in the back and removing about 1 teaspoonful of spinal fluid for examination. A bone marrow aspirate is done by inserting a needle into the hip bone through the skin and removing about 1/2 teaspoonful of bone marrow for examination.

An audiogram (hearing test) will be done at diagnosis for baseline information, prior to each course of induction, and at the end of induction.

A bone scan will be done at diagnosis on all patients (except those with gliomas or brain stem tumors) to rule out any bone involvement.

Blood counts and blood chemistries (kidney and liver function) will be done on blood obtained by finger stick at diagnosis for baseline information and then periodically throughout induction and maintenance therapy to monitor any side effects that might occur. The amount of blood taken is about 1 teaspoonful and will be repeated every three weeks. Neuropsychometric testing will be done twice during the course of treatment to determine your child's development and intelligence.

Twenty-four hour urine collection to measure creatinine clearance will be undertaken at diagnosis for baseline information and will be repeated prior to each course of cisplatin to monitor any toxicity that might occur. A Foley catheter will be inserted into the urethra and the urine collected into the attached bag. This is considered standard therapy for children of this age. All of the above tests would be considered standard observations of a child undergoing chemotherapy.

In addition to conducting clinical studies to improve the treatment of childhood cancer, CCG works

with laboratory researchers who are trying to understand why children get cancer and how cancer cells differ from normal cells. When a sample of your child's cancer is obtained for diagnosis and/or treatment, the part of the tumor which is not required by your child's doctors will be preserved for research studies. In some cases, when your doctor has ordered a blood test to help in the care of your child, an extra tube will be collected and saved for research. Samples for research purposes are usually obtained when your child is having a procedure performed that helps with his or her care. This would happen once or twice during the treatment.

RISKS AND BENEFITS: There is potential for toxicity with all the drugs and treatment described. As with any drugs, there may be unanticipated side effects. These drugs, however, have been in use so long that by careful adjustment of dosage and schedules, severe problems can usually be avoided. One regimen has cisplatin, vincristine, etopostide and cytoxan induction. The other regimen has carboplatin, vincristine, ifosfamide, and VP-16 for induction. Both have carboplatin, cytoxan, VP-16 and vincristine in maintenance. The major side effects from the drugs used in this protocol are listed below.

Vincristine: Jaw pain; constipation; hair loss; weakness, particularly in the hands and feet; local breakdown of skin if drug leaks from injection site; seizures and paresthesias (tingling, usually in hands or feet).

Cyclophosphamide: Nausea and vomiting, hair loss, bone marrow depression, bleeding from the bladder wall, a small but potential risk of developing a secondary case of acute myeloid leukemia (AML).

Mannitol: Loss of fluid from the body through urination.

Cisplatin: Hearing loss, bone marrow depression, decreased kidney function, nausea and vomiting, wheezing, decreased blood pressure.

VP-16: Nausea and vomiting, hair loss, bone marrow depression, a small but potential risk of developing a secondary case of acute myeloid leukemia (AML), wheezing, and decreased blood pressure at the time of drug infusion.

Carboplatin: Bone marrow depression, decreased kidney function, hearing loss.

Ifosfamide: Nausea and vomiting, hair loss, bone marrow depression, bleeding from the bladder wall, kidney damage.

Mesna: Abdominal pain, headache, joint pain, sleepiness, diarrhea, lowering of blood pressure.

G CSF: Bone pain which can be treated with pain medication, elevation of white blood cell enzymes, and enlarged spleen.

Radiation therapy toxicities may cause loss of appetite, some nausea and vomiting, irritability, tiredness and hair loss. Growth may be diminished due to effects on the bones of the spine which may be permanent. Long lasting side effects of this treatment include possible neurologic changes in intelligence or learning abilities, cataract development, decreased height, and possible late tumor production.

APPENDIX I

INDUCTION - REGIMEN A

The chemotherapy schedule is as follows:

1. Cisplatin (CDDP): 3.5 mg/kg into a vein, Day 0.
2. Vincristine (VCR): 0.05 mg/kg into a vein, Day 0, 7, 14.
3. Cyclophosphamide (CPM): 55 mg/kg into a vein, Days 1 and 2.
4. MESNA: Given into a vein 5 times per day, Days 1 and 2.
5. VP 16 2.5 mg/kg into a vein, Days 0, 1, and 2.

This phase of treatment will continue for 5 courses (15 weeks). Hospitalization is required during the administration of CPM and CDDP. Vincristine can be given in the clinic on an outpatient basis.

6. Granulocyte colony stimulating factor (G-CSF): 5 mcg/kg/day for at least 10 days beginning on Day 4. This is given as an injection under the skin.

INDUCTION - REGIMEN B

The chemotherapy schedule is as follows:

1. Carboplatin (CBDCA): 10 mg/kg into a vein, Day 0, 1.
2. Vincristine (VCR): 0.05 mg/kg into a vein, Days 0, 7, 14.
3. Ifosfamide (IFOS): 60 mg/kg into a vein, Days 0, 1, 2, 3, 4.
4. MESNA: Into a vein, Days 0, 1, 2, 3, 4;
5. VP-16: 1.5 mg/kg into a vein, Days 0, 1, 2, 3, 4.

Hospitalization will be required for administration of all chemotherapy except vincristine.

6. Granulocyte colony stimulating factor (G-CSF): 5 mcg/kg/day for at least 10 days beginning on Day 5. This is given as an injection under the skin.

MAINTENANCE CHEMOTHERAPY

Maintenance (8 Courses)

1. Carboplatin (CBDCA): 18 mg/kg into a vein over 1/2 hour, Day 0.
2. Cyclophosphamide (CPM): 65 mg/kg into a vein, Day 28.
3. VP-16: 2.5 mg/kg into a vein, Days 0, 1, 28, 29, administered over 1 hour.
4. Vincristine (VCR): 0.05 mg/kg into a vein, Days 0, 7, 14, 21.

Hospitalization will be required during the cyclophosphamide infusion only. Vincristine, carboplatin and VP-16 can be administered on an outpatient basis.

There may be no direct benefit to your participation; however, the aim of this research is to increase survival and decrease side effects of the treatment.

Although every precaution will be taken to minimize toxic effects of these treatments, their development is unpredictable, both in nature and severity, and may be life-threatening. In the event of adverse side effects, the usual careful medical management of your child will be undertaken to reverse the effects.

ALTERNATIVES: Alternatives to this treatment would be chemotherapy which would be similar or possibly the same without being enrolled on the study. Radiation therapy is also an alternative.

In the event that this research activity results in an injury, treatment will be available including first aid, emergency treatment, and follow-up care as needed. Payment for any such treatment must be provided by you or your third party payer, if any, such as health insurance, Medicare, etc. There are no additional costs to participation in this research program. None of the tests required are for research only, but standard observations for a child undergoing chemotherapy.

CONFIDENTIALITY: The records of this study will be kept private. In any sort of report we might publish, we will not include any information that will make it possible to identify any subject. Your records for this study may, however, be reviewed by the drug manufacturer or representatives of the Food and Drug Administration of the National Cancer Institute. To that extent, confidentiality is not absolute.

VOLUNTARY NATURE OF THE STUDY: Your decision whether or not to participate will not affect your current or future relations with the University. If you decide to participate, you are free to withdraw at any time without affecting those relationships or creating a danger to your child if stopped. If during the course of this study significant new findings are discovered which might influence your willingness to continue, the researchers will inform you of those developments. The researchers conducting this study are Dr. William G. Woods and _____ in the Department of Pediatrics. You may ask any questions you have now. If you have any questions later, you may contact them at (612) 626-2778. You will be given a copy of this form.

STATEMENT OF CONSENT

I have read the above information. I have asked questions and received answers. I consent to participation in this study.

_____	_____
Signature of Parent or Guardian	Date
_____	_____
Witness	Signature of Investigator

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January 5, 1996

RE: Stanislaw Burzynski, M.D.

TO WHOM IT MAY CONCERN:

I am a physician practicing medicine in southern Minnesota. Dustin Kunnari is my nephew and has been under the care of Stanislaw Burzynski, M.D., since April of 1994. He was diagnosed with a medulloblastoma at the age of 3. This is a very rare and aggressive tumor with an extremely poor prognosis. In February of 1994 he underwent surgical removal of most of the tumor. Some tumor remained, however, and treatment with radiation and/or chemotherapy was advised. This carried with it a significant morbidity and/or mortality. Following surgery, Dustin had no evident neurologic deficits.

Against my advice, my sister, Marianne Kunnari, and her husband, Jack, elected to pursue treatment with Dr. Burzynski. Dustin was initiated on therapy in April of 1994. Several months later, a repeat magnetic resonance scan showed no evident tumor remaining. Both his neurosurgeon and primary care doctor were amazed by the results of the MRI scan. Dustin continued to do extremely well, suffering no evident recurrence or neurologic deficits. He is basically a "normal" 3½ year old. In April of 1995, however, there was recurrence of the tumor. Dr. Burzynski increased the dose of medication; and the tumor subsequently, over the next several months, regressed, again with minimal to no side effects.

As I understand, Dr. Burzynski has come under investigation for his work with tumors in humans. I also understand that Dustin's future treatment will be determined by the court.

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I am requesting that Dr. Burzynski be allowed to continue to treat Dustin in a compassionate manner. Dustin has been, as far as can be determined, significantly benefited from this treatment and has remained a "normal" child under Dr. Burzynski's treatment. If this treatment is discontinued, the only alternative would be standard therapies, i.e., chemotherapy and radiation, both of which carry significant morbidity and mortality. The physicians evaluating him stated that he would certainly experience considerable brain damage and may end up incapacitated for a lifetime under these forms of treatments.

In summary, to allow this treatment to continue may perhaps offer this vivacious 4-year-old a normal lifetime and victory over the insurmountable enemy: brain cancer. This as opposed to a possible "cure of the tumor" but incapacitation of the child, resulting in a lifetime of institutional care. This would be an unforgivable and extremely unreasonable fate in this era of medical "advancement." I am begging the court to give him this chance.

Thank you for your consideration.

Sincerely,

Anthony F. Novak, M.D.

AFN/rmh

Mr. BURTON. Well, that's a pretty eloquent statement for such a young man.

Mr. JACK KUNNARI. Those were his own words.

Mr. BURTON. Very good. Very good. You may be a politician one day. [Laughter.]

Mr. JACK KUNNARI. I'll warn him against that. [Laughter.]

Mr. BURTON. You warned him against that? Well, I don't know. [Laughter.]

The politicians, for good or ill, may be the ones that will ultimately make the right decisions to change some of these things.

And I'll tell you, not on this subject, but most of the people that work in government and the Congress are pretty honorable people. We've got a few bad eggs, but not many. [Laughter.]

Let me just start off the questioning by asking, how much money did you have to spend on Dr. Burzynski's treatments?

Mr. JACK KUNNARI. I believe we spent about $10,000 a month.

Mr. BURTON. About $10,000 a month? Is that pretty standard for people who are going to his clinic down there?

Mr. JACK KUNNARI. I guess I'm not qualified to answer. I don't know for sure. I suppose it might vary from patient to patient.

Mr. BURTON. But that was—yes, ma'am?

Ms. SIEGEL. I think that's about what it is right now. The capsule treatment is much less than that. It's about $2,000. But the important thing to realize here is that chemotherapy is much more expensive than that.

Mr. BURTON. No, I understand.

Ms. SIEGEL. Right.

Mr. BURTON. I'm not—don't misunderstand; I'm just trying to get the facts here.

Ms. SIEGEL. Right.

Mr. BURTON. And the $10,000 a month is not covered by any health insurance that you have?

Mr. JACK KUNNARI. We were fortunate that our health insurance covered treatment for Dustin.

Mr. BURTON. It did?

Mr. JACK KUNNARI. Yes. As of May 1997, they have denied treatment, and we're going through an appeal process trying to get them to cover it again.

Mr. BURTON. Why is your health carrier, after having made the commitment to give you coverage for that, why have they rescinded that coverage? Is it because of the FDA's decision?

Mr. JACK KUNNARI. I don't know if it has anything to do with the FDA. The reason, we were told, they weren't covering treatment is because Dustin is just being monitored by telephone, telephonic services, and I've explained to them that it has been the same since 1994, and it's the same treatment. Now he's being monitored by a telephone——

Mr. BURTON. But his tumor is not—he doesn't have a tumor in his brain?

Mr. JACK KUNNARI. No, he's on a maintenance dose of capsules.

Mr. BURTON. And what does that maintenance dose cost you per month?

Mr. JACK KUNNARI. That's $2,000 a month.

Mr. BURTON. $2,000 a month? Pretty expensive. That's got to be causing you some real problems.

Mr. JACK KUNNARI. A little more than I have, yes.

Mr. BURTON. OK. But it's $2,000 a month now, and when he was in the regular program down there, it was running around $10,000?

Mr. JACK KUNNARI. Right.

Mr. BURTON. OK. Ms. Nippert, the cost that you incurred from your treatment offshore when you went to the Bahamas, what did that run?

Ms. NIPPERT. I believe the first 6 weeks was $10,000, and then after——

Mr. BURTON. Was that total or a week?

Ms. NIPPERT. No, that was just total for 6 weeks. After that, it was like $3,000 every 6 weeks. It was $150 a dose, and I took it three times a week.

Mr. BURTON. But it was very effective?

Ms. NIPPERT. It was for me, yes.

Mr. BURTON. Did the others have similar results, the ones that you referred to?

Ms. NIPPERT. I really didn't know. I was kind of alienated from the patients, but I did see them and meet them, the ones at the hearing, and they had had really dramatic results with it and were doing well before it was taken away.

Mr. BURTON. OK. When you went to Germany, what did they charge you over there?

Ms. NIPPERT. Initially, it was $10,000.

Mr. BURTON. Was that for what period of time?

Ms. NIPPERT. Three months.

Mr. BURTON. $10,000 for 3 months?

Ms. NIPPERT. The first 3 months, and then after that it was $3,000 for every 3 months.

Mr. BURTON. $3,000 every 3 months? So it was running $1,000 a month after that?

Ms. NIPPERT. About $1,000 a month, yes, sir, and none of it was covered by insurance.

Mr. BURTON. OK. That didn't include room and board? It just included the treatment?

Ms. NIPPERT. That's right.

Mr. BURTON. OK. Ms. Sherman, your treatment, you said, was $30,000, $25,000 to $30,000, for the vitamins? Is that——

Ms. SHERMAN. Just the vitamins; that wasn't the trips to the doctors or the transportation to and from New York included in that. And I'm still—I'm taking maintenance now, and that is $200 a week.

Mr. BURTON. $200 a week?

Ms. SHERMAN. Yes.

Mr. BURTON. And is this a chelation treatment?

Ms. SHERMAN. Chelation?

Mr. BURTON. Yes.

Ms. SHERMAN. No. No, this is just to make sure the tumor markers don't go up and that I stay——

Mr. BURTON. No, but I mean is it administered orally?

Ms. SHERMAN. Pardon me? No, it's intravenous.

Mr. BURTON. Intravenous.

Ms. SHERMAN. Intravenous, right.

Mr. BURTON. And what kind of vitamins were those? Were those A and E, like Ms. Fonfa?

Ms. SHERMAN. Well, there are many, many—yes, there are so many.

Mr. BURTON. You take a bunch of them?

Ms. SHERMAN. I brought my papers, but I'm sure I don't want to——

Mr. BURTON. Well, for the committee, I'd like to, if we could, have any information that you have, I'd like to make copies of it; my staff will be glad to do that, unless it's something you want to keep confidential, because——

Ms. SHERMAN. No. I just know that one doctor wouldn't even let me mention his name, though, in my report.

Mr. BURTON. I understand. I understand, and I can understand their concern, but we'd like to have that information, if we could, as much of it as possible, because we're going to have the FDA appearing and testifying. We're going to be asking them about your cases, at least I will be, and we want to have as much information, so we can present it to them, as possible.

Ms. SHERMAN. I would be happy to submit that.

Mr. BURTON. OK, make sure that we get copies of that.

[The information referred to follows:]

GENEVIEVE J. SHERMAN

CURRENT VITAMIN PROTOCOL

Basic Formula #1 - One ca½sule three times a day
Beta Natural - One capsule three times a day
Ester C - One Capsule three times a day
Quercetin - 250 mg.- Two times a day
GABA 3-- mg- One four times a day
Carnitine 250 mg - One four times a day
L. Glutathione 50 mg- One three times a day
Taurine 500 mg. Two three times a day
Pancreatic Glandular Tissue (Pork) One seven times a day
Hawthorne - Two capsules twice a day
Chromium Picolinate 200 mg - One Twice a day
Magnesium Oritate 500 mg - One four times a day
Essential Oils - One Ehree times a day
Super GLA - One twice a day
DHEA 50 mg - One a cay
CoQ10 (Liquid) One teaspoon twice a day
Thioctic 100 mg. - Two three times a day
CV#4 (Cardiovascular Formula) - One three times a day
Anti-Oxidant Formula- Two Three times a day

This vitamin formula is subject to change depending on next check-up with Dr. Atkins.

I BAG : 10 ML Glutathione in 50cc Normal Saline

DOCTORS' ORDER

Patient: Genevieve Sherman Date: 1/26/98

ANTI-CANCER FORMULA

I BAG:

250 ML	STERILE H2o
25 GMS	VIT C - Sodium Ascorbate
10 MEq (2 ME1/ML)	KCL (5mL)
2 ML (25 MGS/ML)	Adenosine Monophosphate
5 ML (0.465 MEq/ML)	Calcium Gluconate 10%
5 ML 200 MGS/ML	Magnesium Chloride 20%
1 ML (5 MGS/ML)	Zinc
3 ML (40 MCq/ML)	Selenium
1 ML (1MG/ML)	Manganese Chloride
1 AMP	Thymus
5 ML	Germanium

* Call me if you need instructions (212) 758 2110 x234 Julie Fosling

supplies sent for 24 IV therapies)

RATE OF INFUSION: 1-1/2 - 2 HRS TO RUN AT 60 GTTS/MIN

RC Atkins
Ordering M.D. or P.A. Signature

III BAG:
1 ampule Evrixor in 100 cc Normal Saline

This protocol to be run biweekly. The first treatment, 1 amp Evrixor can be given SQ (only the 1st one) & then IV throughout. For the 1st trx, you can give the 1st + 2nd bag also with SQ injection. (over)

During my 7½ years of the Anti-Cancer Formula, I have also received Essiac, Ilionea, 714 X (intravenously) and shark cartilage.

GP

Mr. BURTON. Ms. Siegel, now you also went to Dr. Burzynski?

Ms. SIEGEL. Right.

Mr. BURTON. And you're in California? You're a California resident?

Ms. SIEGEL. Yes.

Mr. BURTON. What kind of cost did you incur?

Ms. SIEGEL. When I started 7 years ago, it was quite a bit less, but it was probably about $5,000 a month then.

Mr. BURTON. $5,000 a month?

Ms. SIEGEL. Yes.

Mr. BURTON. It's more than that now?

Ms. SIEGEL. It's more than that now, yes.

Mr. BURTON. And you have to go down to his clinic to have this administered?

Ms. SIEGEL. Right. Because it was illegal for us to bring the medicine across State lines——

Mr. BURTON. Yes, I know.

Ms. SIEGEL [continuing]. We had to go every 2 months.

Mr. BURTON. But it's running about $5,000 a month now still?

Ms. SIEGEL. No, now I'm on the capsule form of the treatment, and it's about $3,000 a month.

Mr. BURTON. $3,000 a month.

Ms. SIEGEL. Or two.

Mr. BURTON. $2,000?

Ms. SIEGEL. Yes.

Mr. BURTON. OK. All right, let me see here. And you were terminally ill when you started taking it?

Ms. SIEGEL. I was. I was a stage four, low-grade, non-Hodgkins lymphoma. I was terminally ill.

Mr. BURTON. And it's gone now?

Ms. SIEGEL. I am totally cancer-free today.

Mr. BURTON. Ms. Fonfa, your vitamin was, you said, vitamin A and vitamin E?

Ms. FONFA. Yes.

Mr. BURTON. And what does that run per month?

Ms. FONFA. Well, actually, the vitamin A and vitamin E was not the sole treatment, but it's the trigger that reduced chestwall tumors that I had, but the vitamin A and vitamin E by themselves is something like $35 a month—nothing. But the basic——

Mr. BURTON. That was administered intravenously?

Ms. FONFA. No, it's taken through——

Mr. BURTON. Orally?

Ms. FONFA. Yes, I drink it with my vegetable juices.

Mr. BURTON. It's a liquid?

Ms. FONFA. Yes. But I was on the Gerson program, which I did in Mexico, which I mentioned cost me $27,000 for a 5-week stay, none of which was reimbursed. But my regular supplements during the time that I was actively fighting disease were more than $2,000 a month, none of which was reimbursed. At this point I'm back down to about $800 a month, using vitamins and supplements.

Mr. BURTON. And that's all out of pocket?

Ms. FONFA. Correct.

Mr. BURTON. And what's the status of your cancer now?

Ms. FONFA. Well, I haven't developed any tumors since December 1996. That's about all I can say.

Mr. BURTON. So you've had 2 years——

Ms. FONFA. So far, so good. As I'm sure everyone here will agree, we don't have very good detection methods, and the only time you know if you're in trouble is when you absolutely know you're in trouble. The rest of the time you just hope everything's OK, and we don't know for sure.

Mr. BURTON. In my wife's case, they missed—on the mammogram they missed her cancer for 7 or 8 years, and we discovered it by accident, and it was already in some of her lymph nodes. So there's a problem.

Do you have any questions?

Mr. MCHUGH. Mr. Chairman, I do not, but I did want to state for the record, and mostly so these good folks could hear the admiration I feel for them in their courage in fighting both the system and their diseases, and for the courage of appearing here today. I think Dustin does have a great future. He, obviously, is at great ease, but I'm not sure it's always that easy for these other good people. Just to let them know we do care very deeply about your personal experiences, and through your statements that we'll continue to go over carefully, hopefully we can weigh-in and make a difference to both you and the countless thousands of others, millions of others, that you're speaking for here today.

And thank you, Mr. Chairman, for caring enough to have this hearing.

Mr. BURTON. Thank you for those comments.

Let me just say that all the members of the committee, in fact, we'll get a "Dear Colleague" out to all the Members of the entire House about the bill that we're talking about, which would allow alternative treatments, and hopefully, we'll get a number of cosponsors off the committee and throughout the House of Representatives.

Mr. Souder, do you have any questions or comments?

Mr. SOUDER. No, I don't, but I want to reiterate the words of the chairman and Mr. McHugh. I've been working with a case in my district of a friend, actually, the wife of a man who ran against me, who developed a cancer, and now it looks like it's going to be terminal in the near future, but she was having similar problems with—there was a drug that looked like it might be able to help her. They had some preliminary tests, but she didn't qualify for the experiment, and they were worried about side effects. And you go, look, we're not worried about side effects at this point; if there's some hope, we want to try to expand that hope as much as possible. And I commend the chairman for that, and actually, the drug company that we worked with turned out to be very cooperative, and hopefully, more will do that as we work through this with FDA, too.

It's very helpful—I know sometimes it seems frustrating to come up and read a brief statement and wonder whether you've had an impact here, but this is how you can move public policy and this is how, hopefully, we can change some of the problems that we've got in our country. Thank you for taking the time, and thank you, Ms. Fonfa.

Ms. FONFA. Thank you.

Mr. BURTON. Will the gentleman yield to me?

Mr. SOUDER. I'd be happy to yield.

Mr. BURTON. I have just a couple of questions for those of you who are Dr. Burzynski's patients, Ms. Siegel and Mr. Kunnari. In an FDA briefing that was held yesterday—and I was not in attendance, but this is what I understood the FDA to say: The FDA told members of this committee that neither you nor any other patients were ever cutoff from your medication. Is that true?

Ms. SIEGEL. Well, that's not quite true. They were threatening—they have threatened many times to take us off the medication, but because of Congressman Barton, he helped us. And so I was able to continue to get my medication, but there are other patients who have been taken off. A gentleman by the name of David Smith, he had Hodgkins disease, and he was on antineoplastons treatment, was responding well. The FDA took him off the treatments. Their reasoning was that chemotherapy has often good results with it. Well, he did not have good results with it. In fact, he was dying on the chemotherapy, and yet the FDA made him go off of the treatment.

Mr. BURTON. It was his choice to stay on it?

Ms. SIEGEL. It was his choice to stay on this drug. He testified before Barton's committee, asked to be kept on this drug. No. We even—we fought for him to be able to stay on the drug. His wife testified, pleaded, and the FDA said no.

Mr. BURTON. Yes, Mr. Kunnari, did you have a comment?

Mr. JACK KUNNARI. Well, when we first started treatment with Dusty, we asked if Dusty qualified for any clinical studies or trials, and we were informed by Dr. Burzynski that he did not, because he was not quite 3 years old yet. So I guess, in reality, by the laws set up as they stand now, we were illegally treating Dustin, and then I mentioned in my testimony that we would have been cutoff treatment because we didn't live in the State of Texas, apart from Joe Barton's subcommittee there.

Mr. BURTON. Dr. Burzynski, as I understand it, was indicted, and the FDA made an announcement that Dr. Burzynski was not to treat any patients at all, in essence, cutting off all of his patients; is that correct?

Mr. JACK KUNNARI. Unless they lived in the State of Texas or they were involved in a clinical trial, all patients were cutoff treatment, from what I understand.

Mr. BURTON. And then, of course, Representative Barton got involved, and that helped solve the problem.

Let me ask one more question. The FDA also indicated that they're very concerned about dangerous side effects of Dr. Burzynski's medicine. What side effects have either one of you experienced?

Ms. SIEGEL. Well, I had no side effects, maybe a little tiredness, but I lived a completely normal life the whole time I was on his treatment, and I had three young children at the time. And, yes, I raised these kids. They're all in college now, which I thought I would never see. My three kids are all in college, and I thank God and Dr. Burzynski every day for that.

But the side effects, it's criminal for them to say that. It's not true.

Mr. BURTON. And Dustin, I understand, the treatment caused a 1 percent or 1 point above normal sodium level in Dustin, and they cited that as a reason, one of the reasons, why he shouldn't be treated; is that correct?

Mr. JACK KUNNARI. Correct. His sodium point was 1 point above normal.

Mr. BURTON. And you decided just to go ahead because you thought the risk you were willing to take?

Mr. JACK KUNNARI. Right. They wanted us to take him off treatment until his sodium level was stabilized.

Mr. BURTON. Let me ask you this, both of you, and then I'll yield to my colleague, Mr. Pappas: To your knowledge, have there ever been any complaints against Dr. Burzynski or lawsuits by patients who were treated by him?

Ms. SIEGEL. To my knowledge, never. There has never been one patient complaint filed against Dr. Burzynski in the whole 20 years that he's been treating patients.

Mr. BURTON. Mr. Pappas. Mr. Kunnari. Excuse me.

Mr. JACK KUNNARI. No, there hasn't been. In fact, everything we hear from patients that have succeeded with Dr. Burzynski's treatment, and for those who haven't been as fortunate, there has never been a complaint, only admiration for the man and praise for him.

Mr. BURTON. Mr. Pappas.

Mr. PAPPAS. Thank you, Mr. Chairman. I want to thank you for holding this hearing and for your interest.

For those of you who are here who are interested in this, the chairman has shown leadership behind the scenes with regard to this issue. Most people don't know about that, and I've had the opportunity to work with him. I have a friend who is a cancer survivor, and she had been involved in a program which—in another State; I'm from New Jersey—and which she had been going to, and the FDA had cut that off as well. A short time after that, I was sworn in and became involved in work with Mr. Burton. So we're all very, very fortunate, and the thousands of people in our country are fortunate, for his interest and his activity. I'm very happy to be a part of that and trying to support him.

I just want to echo what was said, to thank you folks for coming and telling your stories. Sometimes it's probably not easy, but it's important to have us and the staff people who are here advising the Members who are not here as to what your experience has been, and how our efforts, hopefully, can move that ball down the field to help other people, as well as people like yourselves. Thanks again for coming. Good luck, Dusty.

And I yield back.

Mr. BURTON. Excuse me. My counsel came in about another investigation we're involved with, not of this importance, however.

I don't have any more questions. Let me just say that we really appreciate your being here. I know a lot of you have come a long way, and you've expended an awful lot of money for your own treatment. So I know this is an additional expense that you really didn't need to incur, but I'll try to make sure, and I'm sure the rest of the members of the committee—I want to inform them that we'll try to make sure that your expense was not wasted. We're going to do our dead-level best to move some legislation that will help

other people, and the contribution that you've made today, hopefully, will help other people that you haven't even had the pleasure to meet. So thank you very much for being here, and have a safe trip home, and good luck in the future. And God bless you all.

Ms. SIEGEL. Thank you.

Ms. SHERMAN. Thank you.

Ms. NIPPERT. Thank you.

Ms. FONFA. Thank you.

Mr. JACK KUNNARI. Thank you much.

Mr. BURTON. Our next panel is Jonathan Emord, Charles Simone, Ralph Moss, Arnold Eggers, and Thomas Moore. Would you please come forward, as soon as everybody gets situated here?

Would you please stand, so you can be sworn?

[Witnesses sworn.]

Mr. BURTON. Have a seat.

OK, gentlemen, let's see, we started at the right end last time; let's start at the left end. We'll start with you, Mr. Emord. And if I mispronounce your names, please correct me. Mr. Emord. If you could try to keep your statements as close to 5 minutes as possible, we'd appreciate it.

STATEMENT OF JONATHAN W. EMORD, ESQUIRE

Mr. EMORD. Thank you, Mr. Chairman. Mr. Chairman and members of the committee, such as they are, I'm an attorney who practices constitutional and administrative law before the Federal courts and agencies. Among my clients are terminally ill cancer patients for whom the FDA's approved treatments have failed.

To understand their plight, and what to do about it, you need to put yourself in their situation. Imagine for a moment a horrible circumstance. Imagine that you, Mr. Chairman, you, members of the committee, are stricken with an incurable brain tumor. Imagine that you have undergone surgery, several rounds of chemotherapy and radiation therapy, to no avail. Your doctors have told you there's nothing more you can do. They predict you will not live beyond 6 months to a year. In so many words, they tell you that, barring an act of God, your fate is sealed.

What on earth can you do? You're left with two very basic choices. You can accept the conventional wisdom and prepare to die or you can fight for life against all odds and on your own terms. If you are like my clients, you will fight with every ounce of strength that you have; you will race against time, and the ravages of disease, to find and try every promising, experimental alternative available for your condition.

Unfortunately, although it is your life, your body, your cancer, and your future, the decision of whether you may try an experimental drug is not yours. In the very last analysis, that decision is the FDA's. The FDA will second-guess your physician's judgment and your own. Your physician may recommend an experimental drug. The corporate sponsor of that drug may agree to supply it, and the clinical investigator may agree to administer it, but if the FDA disagrees, you are out of luck.

It is a cruel, inhumane government, Mr. Chairman, that robs even one terminally ill patient of a potential cure and of the freedom to fight for life on his or her own terms. Yet, from time to

time, the FDA has done just that. Indeed, premature deaths have no doubt occurred because of FDA decisions not to allow access to experimental treatments.

Every day this Congress fails to change FDA law and policy to afford the terminally ill access to experimental treatments free of FDA interference, is another day that this Congress condones a loss of hope, of life's promise for the terminally ill. The Access to Medical Treatment Act is before this Congress, and the time has come to move it out of committee and pass it.

Consider one of my clients, Zachary McConnell, a boy of 8, diagnosed at 5 with a primitive neuro ectodermal tumor, a nearly fatal cancer that spreads its murderous tendrils through the brain with rapidity. At age 5, Zachary had to muster more courage and more strength than most adults ever have to. He suffered through brain surgery, rounds of chemotherapy, radiation treatment, seven blood transfusions, eight hospitalizations, nausea, vomiting, deep bone aches, high fevers, severe gastrointestinal stress, and a loss of one-half of his body weight.

Faced with conventional treatments that were not curative for Zachary's tumor and treatments that produced effects worse than did the disease, Shaun and Desiree McConnell, Zachary's parents, decided to fight for their child's life on their own terms with a promising, experimental alternative. On March 19, 1996, the experimental treatments began, and on May 23, 1996, the FDA ordered Zachary off those treatments, sending him back to the failed conventional drugs.

The McConnells were devastated. They could not believe that their Government had either the authority or the gall to deny them the right to fight for their boy's life. They vowed to oppose the decision through legal means with all the money and clout they and their friends could muster. They hired Washington lawyers; they hired a team of renowned scientific experts, and they pled their case to the FDA, to the media, and before Congress, begging the FDA to reverse its decision. After a month-and-a-half of constant, costly, time-consuming effort, the FDA buckled. With the McConnells' blessing, we have supplied the relevant documents to you, Mr. Chairman, for inclusion in the record.

The McConnells' remarkable campaign is beyond the finances of most terminally ill patients. Few have either the means or the strength to wage such a campaign. For them, when the FDA says no, the answer is final. For them, the FDA is an omnipotent force that has the power to deny freedom to fight for life and to consign innocent victims of disease to near certain death.

This system must change. The FDA Modernization Act that was referred to previously fails to correct the most basic flaws that exist in this system. We must protect patients from a force second only in its lethality to incurable disease, the FDA's denial of a terminally ill patient's access to promising, experimental alternatives.

Thank you, and I'm available for questions.

[The prepared statement of Mr. Emord and the documents referred to follow:]

Before the
U.S. HOUSE OF REPRESENTATIVES
House Commerce Committee
Subcommittee on Oversight and Investigations

Testimony of
Jonathan W. Emord, Esq.

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Unfortunately, although it is <u>your</u> life, <u>your</u> body, <u>your</u> cancer, and <u>your</u> future, the decision of whether you may try an experimental drug is not yours. In the very last analysis, that decision is the FDA's. The FDA will second guess your physicians' judgment and your own.

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This system must change. We must protect patients from a force second only, in its lethality, to incurable disease, the FDA's denial of a terminally ill patient's access to promising, experimental drugs. Thank you. I am available for questions.

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June 7, 1996

VIA TELECOPIER 301-594-0498
Robert J. DeLap, M.D., Ph.D.
Acting Director
Division of Oncology Drug Products
Office of Drug Evaluation 1
Center for Drug Evaluation and Research
Food and Drug Administration
Rockville, MD 20857

Re: IND 43,742; Antineoplastons Patient Zachary McConnell

Dear Dr. DeLap:

This firm has recently been retained to represent Zachary McConnell. Our letter responds to yours of May 23, 1996, as it pertains to Zachary. Earlier today I left word with you (on your voice mail) that we were acquiring information responsive to your inquiries. That information, consisting of several dozen MRI scans has arrived at our office. We are hopeful that upon your review of the scans you will be in a position to reconsider the initial determination to disallow Zachary authority to participate in an experimental protocol involving the administration of Antineoplastons by the Burzynski Research Institute of Houston, Texas.

At this juncture, I can present you with the following information.

Zachary is seven (born April 2, 1989). On August 26, 1995, he was diagnosed as having a Primitive Neural Ectodermal Tumor (PNET) by Dr. David S. Moss, a neurosurgeon with the Phoenix Children's Hospital. Zachary had a tumor on the right side of his brain. PNET, particularly when present at the location of Zachary's tumor, is almost always fatal and involves tumor recurrence in the overwhelming majority of cases. On August 28, Dr. Moss performed surgery to remove the tumor. A biopsy of the tumor was taken and confirmed that Zachary was suffering from PNET. The surgery was successful (a post surgery MRI revealed that there was a complete resection of all visible tumor on Zachary's brain). On February 7, 1996, Dr. Moss obtained a post-surgery MRI that showed the presence of residual tissue on Zachary's brain where the tumor was removed. Dr. Moss was unable to determine whether the residual tissue was scar tissue or new cancer growth.

On August 29, 1995, a team of Oncologists assessed Zachary's condition and determined that he was at high risk of a recurrence of the tumor. Because of the high risk of recurrence and the poor prognosis, especially for someone so young, they recommended that Zachary become a participant in a Phase Two experimental clinical trial known as the "Groupwide Pilot Study of Neoadjuvant Chemotherapy followed by Cranial-Spinal Hyperfractionate Radio Therapy in Patients with Newly Diagnosed High Risk PNET." That protocol involves five courses of aggressive chemotherapy followed by radiation that would involve Zachary's entire cranial-spinal area. On September 14, 1995, Zachary began the chemotherapy. Zachary experienced horrific pain as a result of the treatments. He was subjected to 7 blood transfusions and was hospitalized 8 times. His white and red blood count went to zero on several occasions. He suffered from multiple bacterial and viral infections. He exerpienced total hair loss on his head and face. He suffered severe gastrointestinal distress, nausea, vomiting, deep bone aches, and high fevers. He suffered severe mood swings and headaches. He lost 40% of his total body weight. Zachary began his radiation treatment program on March 4. Within two hours of receiving the treatment, he suffered a severe headache followed by continuous vomiting.

Mr. and Mrs. McConnell have decided not to continue this treatment because their small child has suffered immeasurably and does not appear able to withstand more trauma to his body. Moreover, their physician has informed them that the radiation therapy could cause Zachary to experience a loss in IQ points and could be accompanied by severe damage to major organs surrounding the radiated area, hearing loss, spinal deformities, sterility, risks of secondary cancer, growth abnormalities, cataracts, permanent hair loss, memory loss, and loss of other cognitive functions that cannot be measured by IQ tests, loss of balance, and death due to necrosis.

On March 19, 1996, Zachary began receiving Antineoplastons treatments at the Burzynski Institute. On April 26, 1996, an MRI revealed that the residual tissue on his brain, present in the February 7 MRI, disappeared. Zachary has had no adverse reactions to the antineoplastons treatments.

A copy of the affidavit of Shawn McConnell, Zachary's father, is attached as Exhibit A. We have also attached as Exhibit B relevant additional documents for your review.

In your letter you request a copy of the MRIs. We have the original MRIs in our offices and would be pleased to hand deliver them to your office for review at your earliest convenience. Because they are the originals and may be required on short notice by Zachary's physicians, we will not be able to part with them and will need to be present during your review. In your letter you request a copy of a report from Zachary's radiation oncologist indicating that the tumor is not *potentially* curable with radiation therapy. We know of no radiation oncologist in the United States who would represent that the application of radiation to a brain tumor would not be *potentially* curative. While there is a slim chance that radiation could save Zachary, the *probability* is that it will not and,

indeed, the reality is that he has suffered excruciating pain from the one radiation treatment he did receive. Moreover, a team of Oncologists believed it in the child's best interests to enter an experimental protocol involving intensive and high dose chemotherapy and radiation *after* a complete resection of the tumor: on the view that recurrence of the tumor is so likely and is so likely to be lethal that experimental treatments were warranted. The fact is that young Zachary has not been able to cope with the suffering brought on by these treatments and his parents believe that completion of the current regimen could kill Zachary (not an unreasonable belief based on the scientific evidence documenting death to necrosis as a side effect). It would therefore appear that another experimental treatment is warranted, one that Zachary can withstand. The treatment of choice for the McConnells is Antineoplastons and the track record thus far has been a positive one: any potential cancerous mass present in the February 7 MRI disappeared under the Antineoplastons treatments.

Please inform me at your earliest convenience of a time when you would like to review the MRI scans. As soon as possible thereafter (in light of the critical need for treatment for Zachary), we would appreciate receiving word from you of your decision on our request for reconsideration of the agency's May 23 decision to disallow Zachary authority to continue receiving Antineoplastons therapy under the Burzynski treatment program. I look forward to receiving your call.

Sincerely,

Jonathan W. Emord

cc: Shawn and Desiree McConnell;
Steve Siegel, President of the Burzynski Patient Group;
Kathryn Cook, Esq., Office of the Chief Counsel

EXHIBIT A

AFFIDAVIT OF SHAWN R. McCONNELL

I, Shawn R. McConnell, declare under penalty of perjury that the following is true and correct to the best of my knowledge, information and belief:

1. I reside at 17107 East Calaveras Avenue, Fountain Hills, Arizona 85268.

2. On August 26, 1995, my six year old son Zachary McConnell, was preliminary diagnosed as having a Primitive Neural Ectodermal Tumor (PNET) by Dr. David S. Moss, a neurosurgeon with the Phoenix Children's Hospital.

3. That preliminary diagnosis was made after a MRI taken at the Biltmore Imagining Center showed a tumor on the right side of Zachary's brain.

4. On August 28, 1995, Dr. Moss performed surgery to remove the tumor. At that time a biopsy of the tumor was taken and confirmed that Zachary was in fact suffering from PNET.

5. The surgery was successful and my wife, Desiree D. McConnell, and I were informed that the post surgery MRI showed that there was a complete resection of all visible tumor on Zachary's brain.

6. On August 29, 1995 a team of Oncologists met with my wife and I to determine what the next step in Zachary's care should be. At that meeting we were informed that Zachary was an appropriate candidate to follow the protocol for Phase Two of the clinical study known as Groupwide Pilot Study of Neoadjuvant Chemotherapy followed by Cranial-Spinal Hyperfractionate Radio Therapy in Patients with Newly Diagnosed High Risk PNET. We were informed that therapy under this protocol would include five courses of aggressive Chemotherapy followed by radiation that would involve Zachary's entire cranial-spinal area.

7. On September 14, 1995, Zachary began aggressive chemotherapy under the supervision of a team of Oncologists. That team consisted of Dr. Moss, Neurosurgeon of the Phoenix Children's Hospital; Dr. Etzel, Zachary's primary Oncologist of the Phoenix Children's Hospital; Dr. Sapozink, Radiation Oncologist of the Good Samaritan Hospital; and Dr. Kaplan, Neurologist of the Phoenix Children's Hospital.

8. The team of Oncologists, under Dr. Etzel's supervision, scheduled Zachary for 5 courses of chemotherapy. Each course was to last 3-4 weeks and required Zachary to take 5 different chemotherapy drugs.

9. During the course of the aggressive chemotherapy Zachary experienced several severe side-effects. Zachary was subjected to 7 blood transfusions and was hospitalized 8 times due to the chemotherapy. His white and red blood count went to zero on several occasions while he was undergoing the therapy. Zachary suffered from multiple bacterial and viral infections as a result of the low white blood count. As a result of the multiple bacterial and viral infections Zachary was required to take large doses of antibiotics such as vancomyacin and gentomyacin which created other health problems. Zachary lost his high pitch hearing due to the chemotherapy. Zachary experienced total hair loss on his head and face due to the chemotherapy. During his course of chemotherapy treatment Zachary suffered severe gastrointestinal distress, nausea, vomiting, deep bone aches and high fevers because of the aggressive treatment. Due to the chemotherapy Zachary suffered severe mood swings and headaches due to anemia. He also lost 40% of his total body weight during chemotherapy.

10. Dr. Etzel completed Zachary's last course of chemotherapy the first week of January 1996.

11. On February 7, 1996, Dr. Moss ordered a second post-surgery MRI that showed residual tissue on Zachary's brain where the tumor was removed. Dr. Moss was unable to determine whether the tissue was scar tissue or growth of another tumor.

12. After five weeks of rest, Dr. Sapozink, scheduled Zachary's radiation treatment for the second week of February. Because of a reoccurring infection in Zachary's gastrointestinal track, the radiation treatment did not begin as scheduled.

13. During the month of February, my wife and I met with Dr. Sapozink to discuss the possible side effects of radiation therapy. Dr. Sapozink informed us that Zachary could experience a loss in IQ points, severe damage to major organs surrounding the radiated area, hearing loss, spinal deformities, sterility, risks of secondary cancer, growth abnormalities, eye cataracts, permanent hair loss, memory loss, loss of other cognitive functions that can not be measured by IQ tests, loss of balance and death due to necrosis.

14. During the first week of February Zachary took an IQ test administered by Dr. Wood at Phoenix Children's Hospital. The results of the test showed that Zachary has a superior IQ. Dr. Sapozink stated that because Zachary has a superior IQ, he could stand to lose the few points that might occur under the radiation treatment.

15. On March 4, 1996, under the supervision of Dr. Sapozink, Zachary was given one session of radiation treatment. Within two hours after being subjected to the radiation, Zachary suffered a severe headache followed by continuous vomiting.

16. Dr. Sapozink was unable to explain to us why Zachary had experienced such a severe reaction to the radiation treatment.

17. After hearing the risks associated with radiation therapy and witnessing Zachary's reaction to the treatment, my wife and I decided against the radiation treatment and began researching alternative means of treating our son's condition.

18. After extensive research we discovered Dr. Burzynski's Clinic in Houston, Texas.

19. On March 18, 1996, Zachary had his first visit with Dr. Burzynski.

20. On March 19, 1996, Zachary began antineoplastons therapy.

21. After reviewing Zachary's health records, and giving him blood tests, Dr. Burzynski recommended that Zachary begin his treatment by taking small doses of antineoplastons to ensure that the drug did not cause Zachary any adverse reaction.

22. After determining that the treatment did not have an adverse effect on Zachary, Dr. Burzynski scheduled 8-12 months of antineoplastons treatment to combat any recurring tumors. That schedule included twelve intravenous treatments per day, every four hours of 60cc of A-10 and 18cc of AS2-1 per treatment.

23. On April 26, 1996, Zachary was given a MRI that showed that the residual tissue on his brain present on the February 7 MRI was no longer present.

24. Since Zachary has been under the antineoplastons treatment he has had no symptoms of cancer. He has returned to the young, active child we knew before his battle with cancer. Zachary now has energy and is able to attend school and play with is friends. He no longer suffers from bone pain, his hair has completely returned and he has returned, to his normal body weight.

25. On May 23, 1996, we were informed by the Burzynski Clinic that the FDA stated that Zachary could no longer use the antineoplastons treatment. It is our understanding that Zachary cannot use the treatment because he does not currently have a residual tumor.

26. Zachary ran out of his daily dose of antineoplastons therapy on May 25, 1996.

27. We would like to continue the antineoplastons therapy for Zachary because of his positive response under the therapy. We are also concerned about the possibility of a recurring tumor. When Zachary was first diagnosed with cancer the Oncologists repeatedly told us that without aggressive follow-up treatment the brain tumor would return.

28 The team of Oncologists informed us that they believe that Zachary only had a 20-40% chance of surviving another five years, and that he was too poor a risk for them to realistically assure us of a long term cure with conventional therapies.

29 I was repeatedly informed by Zachary's doctors that the primary goal of any cancer therapy is to extinguish tumor cells the first time out and that a recurrent tumor is notoriously more tenacious, and thus more deadly than the original tumor

30 After being informed of the risks accompanying the radiation volume, target (Zachary's entire cranio-spinal area) and adjuvant chemotherapy factor for Zachary, my wife and I do not believe the risk/benefit ratio to be acceptable

31. Dr Moss, Zachary's neurosurgeon, supports our decision to make our own informed decisions regarding Zachary's continuing therapy options.

Executed on 6-7-96

Shawn McConnell

EXHIBIT B

PATIENT CONSENT, AUTHORIZATION, WAIVER, AND RELEASE

I, the undersigned, whose full name is ZACHARY McCONNELL, being either the Patient or the authorized representative of the Patient (hereinafter "PATIENT"), hereby REQUEST, AUTHORIZE AND CONSENT TO treatment of PATIENT'S medical condition by Stanislaw R. Burzynski, M.D., Ph.D., and such other assistants, agents, servants, and employees as he may designate; and I hereby fully RELEASE Stanislaw R. Burzynski, M.D., Ph.D., the Burzynski Research Institute, and such other of his assistants, agents, servants, and employees as he may designate, from all liability which may be alleged or adjudged against them in connection with PATIENT'S treatment, and, in connection herewith, I specifically understand the following, to-wit:

The treatment REQUESTED, AUTHORIZED, and CONSENTED TO is the administration to PATIENT of the Antineoplastons referred to as A1, A2, A3, A4, A5, AS2-1, AS2-5, and A10, or any other drugs or medicines, such as conventional chemotherapy drugs (possibly in combination with Antineoplaston drugs), in either liquid or capsule form, as either a natural or synthetic product, and with the same to be administered, at Dr. Burzynski's discretion, intramuscularly, intravenously, orally, and/or topically.

Dr. Burzynski has explained to PATIENT the nature of these substances and that these substances and their use in treating cancer or other conditions such as that which PATIENT has been diagnosed as having, is purely experimental. PATIENT specifically understands that the use of such substances as a prescriptive drug has not yet been approved by any State or Federal regulatory agency, nor has its use been approved or adopted by any single or group of Medical Institutions at this time.

Under Federal Law (as determined in a prior legal proceeding between Dr. Burzynski and the Food & Drug Administration), Dr. Burzynski is only permitted to treat patients with Antineoplastons in the State of Texas. Neither he nor anyone associated with him is permitted to ship his medication out of the state. PATIENT understands and agrees that in the event PATIENT permanently moves out of the boundaries of the State of Texas, Dr. Burzynski and the Burzynski Research Institute will not ship or send PATIENT Antineoplaston drugs.

Dr. Burzynski has explained to PATIENT, and PATIENT understands, the risks associated with the use of the substances and any alternative treatments available to PATIENT. No assurances have been made to the PATIENT by anyone as to any results expected to be obtained from such treatment. PATIENT hereby DENIES that any assurances or warranties were made pertaining to results or successes of the treatment. PATIENT has been told, and specifically understands, that neither success nor improvement in PATIENT'S condition is warranted or guaranteed by such treatment. PATIENT understands that the reverse may be true and that PATIENT'S condition may worsen as a result of the treatment, or that PATIENT may react adversely to the treatment.

PATIENT understands that Dr. Burzynski makes no claims that Antineoplaston drugs will cure or stabilize cancer or any other medical condition. PATIENT also understands that the U.S. Food and Drug Administration and the National cancer Institute claim that this drug is not yet proven to be effective in the treatment of cancer.

PATIENT CONSENT, AUTHORIZATION, WAIVER, AND RELEASE

PATIENT Agrees to deposit the sum of $ 6000.00 as an initial deposit with the Burzynski Clinic to start treatment with Antineoplastons, which sum of money will be retained by the Clinic until treatment is completed, even if PATIENT'S insurance carrier determines in advance that it will pay 100% of the cost of this treatment. PATIENT understands that the reason for this is in the unfortunate event that PATIENT'S insurance carrier initially determines it will pay 100% for the treatment and then decides thereafter that it will not pay. PATIENT understands that if Dr. Burzynski and the Burzynski Research Institute are not paid for their services and drugs, they will have the sole authority and decision to terminate this treatment, no matter how cruel or unfortunate this may seem.

In signing the CONSENT, AUTHORIZATION, WAIVER, AND RELEASE, and in requesting this treatment, PATIENT agrees and SPECIFICALLY STATES that he does RELEASE Dr. Burzynski and all of his agents, servants, employees, associates, affiliates, and parent or subsidiary companies, including the Burzynski Research Institute, from any and all liability for all claims for damages which PATIENT, anyone on PATIENT'S behalf, or anyone on the behalf of the PATIENT's estate may have because of, arising out of, or related to the Antineoplaston treatment hereinabove described, or any other treatment or drugs prescribed to PATIENT by Dr. Burzynski.

In connection with the treatment hereinabove described, PATIENT consents to the publication and re-publication of information and/or photographs relating to PATIENT'S case in professional journals or medical books and to the use of such information for any other purpose Dr. Burzynski may deem proper in the interest of medical education, knowledge, or research; provided, however, that it is specifically understood that in any publication or use, PATIENT shall not be identified by name.

I, the undersigned, either as a PATIENT or legal representative of a PATIENT, am over the age of eighteen (18) years and do certify that I am of sound mind and I have read this form in its entirety, that the statements contained herein are true, and that I authorize and request those individuals above to render such treatment to PATIENT.

PATIENT acknowledges that no publication, media event, or representation made by any person or entity, has induced PATIENT to undergo the treatment to be rendered and that this agreement is entered into by PATIENT'S own free act and will.

This PATIENT CONSENT, AUTHORIZATION, WAIVER AND RELEASE is being executed not as a prerequisite for treatment, nor as a result of any representations to the contrary, but, rather, to acknowledge PATIENT'S understanding of the experimental nature of the treatment; and that, hope and prayers notwithstanding, no promises, guarantees, nor warranties for success of the treatment have been made.

PATIENT CONSENT, AUTHORIZATION, WAIVER, AND RELEASE

DATED this 18 day of MARCH, 1996

PATIENT NAME:

Printed Name

X Denise McConnell
Patient's Signature

Patient's Permanent Address:
P.O. Box 17352
Fountain Hills, Az
85269

Patient's Texas Address While Undergoing Antineoplaston Treatment:
Homestead
Room #223 Ext. #1159?

TIME: 5:20 a.m./p.m. Relationship: mother

Witness:
Gabriela Howard

SUBSCRIBED AND SWORN TO before me, the undersigned authority, by the said Zachary McConnell on this the 18th day of March, 1996, to certify which witness my hand and seal of office.

Cheryl A. Owens
Notary Public in and for the State of TEXAS

CHERYL A. OWENS
MY COMMISSION EXPIRES
August 11, 1900
SEAL

My Commission Expires:
8-11-96

Typed or Printed Name of Notary:
Cheryl A. Owens

BURZYNSKI
RESEARCH INSTITUTE, INC.

May 10, 1996

Mr. Paul Zimmerman, CSO
FOOD AND DRUG ADMINISTRATION
CDER Oncology Group (HFD-150)
Attn: 3rd Floor Document Room
1451 Rockville Pike
Rockville, MD 20852
Send by Federal Express

RE: IND #43,742

Dear Mr. Zimmerman,

In response to your fax of April 26, 1996, please find attached the listing of an additional patient with his diagnosis and description of the treatment. The patient is MCCONNELL, Zachary and is six years old. The patient is already in complete response and we feel that this is extremely important to continue his treatment because he may develop early tumor recurrence.

Sincerely,

S. R. Burzynski, M.D., Ph.D.

SRB/cf

12000 RICHMOND AVENUE • HOUSTON, TEXAS 77082-2431 • (713) 597-0111 • FAX (713) 597-1166

SUMMARY

Patient's Name: MCCONNELL, Zachary

Diagnosis: Primitive neuroectodermal tumor of the right cerebral hemisphere of the brain (PNET)

Treatment: Antineoplaston A10 and Antineoplaston AS2-1 injections began on March 19, 1996 and are administered daily. The dosage of Antineoplaston A10 was gradually increased to 5.45 g/kg/day and AS2-1 to 0.44 g/kg/day. No other anticancer treatment has been given to this patient since March 19, 1996.

May 10, 1996

S. R. Burzynski, M.D., Ph.D.

SRB/ef

BURZYNSKI
RESEARCH INSTITUTE, INC.

May 17, 1996

VIA FEDERAL EXPRESS

Paul Zimmerman, C.S.O.
FDA/CDER Oncology, HFD-150
1451 Rockville Pike
Rockville, MD 20852

RE: IND #43,742

Dear Mr. Zimmerman:

I am providing you additional information regarding the following patients:

Susan Dubin
Albert Frolander
Zachary McConnell
Patricia McPherson
Janice Miller
Patricia Petroski
David Smith

Sincerely yours,

Stanislaw B. Burzynski, M.D., Ph.D

12000 RICHMOND AVENUE • HOUSTON, TEXAS 77082-2431 • (713) 597-0111 • FAX (713) 597-1166

June 13, 1996

Catherine M. Cook, Esq.
Associate Chief Counsel for Enforcement
Office of the Chief Counsel
Food and Drug Administration
5600 Fishers Lane, GCF-1
Rockville, MD 20857

Re: IND #43,742; Antineoplastons Patient Zachary McConnell

Dear Ms. Cook:

We have been sent a copy of your letter to attorney Jonathan W. Emord of June 10, 1996. We have also been sent a copy of Mr. Emord's letter (with attachments) of June 7, 1996 concerning antineoplaston patient Zachary McConnell. In your letter, you state that you are not at liberty to review or respond to Mr. Emord's letter (submitted in response to Dr. DeLap's letter of May 23, 1996) because neither Mr. Emord nor his client are the IND sponsor. You further state that the deadline for receipt of materials responsive to your May 23 letter (wherein you tentatively conclude that Zachary McConnell may not continue receiving antineoplastons) would be extended until June 24, 1996.

We have reviewed Mr. Emord's letter and the attachments thereto. We believe they contain the most thorough response possible to the May 23 letter. We, therefore, formally resubmit them to you (see the attached copy) as our official response to Dr. DeLap's May 23 letter (as that letter pertains to Zachary McConnell). Moreover, we ask that you have Dr. DeLap confer with Mr. Emord concerning the MRI scans of Zachary McConnell now possessed by Mr. Emord, consistent with his request in the May 23 letter for that review.

Please respond to us at your earliest convenience. We need not restate the urgency of the request (see Mr. Emord's letter for details).

Sincerely,

S. R. Burzynski, M.D., Ph.D.
President

SRB/cf

cc: Jonathan W. Emord, Esq.
Shawn and Desiree McConnell
Steve Siegel, President of Burzynski Patient Group

12000 RICHMOND AVENUE • HOUSTON, TEXAS 77082-2431 • (713) 597-0111 • FAX (713) 597-1166

July 10, 1996

Robert J. DeLap, M.D., Ph.D.
Director, Division of Oncology Drug Products
Office of Drug Evaluation I
Center for Drug Evaluation and Research
Food and Drug Administration
Rockville, MD 20857

RE: IND 43,742; Antineoplastons Patient Zachary McConnell

Dear Dr. DeLap:

This letter responds to the agency's of June 24, 1996. In the June 24 letter, you asked us to supply two kinds of information: (1) "a letter from a physician with specialized training and experience in the treatment of brain tumors who has reviewed the latest literature on treatment of PNET of the brain in children and the details of Zachary's case (indicating that failure to administer the radiation therapy to this child as previously planned would not significantly affect his chances of being cured, considering the surgery and chemotherapy he has already received)" and (2) the "radiologist's" reports interpreting all of the brain scans performed between February 7 and June 21, 1996."

Attached as Exhibit A is the analysis of Dr. Charles B. Simone, M.MS., M.D., an internist (trained at the Cleveland Clinic 1975-1977), medical oncologist (trained at the National Cancer Institute 1977-1982), tumor immunologist (trained at the National Cancer Institute 1977-1982), and radiation oncologist (trained at the University of Pennsylvania 1982-1985). We selected Dr. Simone based on his recognized achievements in the field of oncology and radiation oncology. Dr. Simone's work is recognized by Sloan Kettering and NCI. He served as oncologist to former President Ronald Reagan, to former Vice President Hubert Humphrey, and to several prominent members of Congress. We believe Dr. Simone well-qualified to assess the extent to which the failure to administer radiation therapy will affect Zachary's chance of being cured, considering the surgery and chemotherapy he has already received. Following a thorough review of the patient's clinical history, his MRIs, and his medical reports, along with all published scientific literature on treatments of juvenile PNETs, Dr. Simone finds no scientific basis for concluding that failure to administer radiation therapy may adversely affect Zachary's chance of survival, based on his initial adverse reactions to two fractions of radiation. Dr. Simone concludes that:

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> ...[F]ailure to administer the radiation therapy to this patient as previously planned cannot be shown by any available scientific measure to affect in any significant way Zachary McConnell's chances of being cured (considering the surgery and chemotherapy he has already received). Given my detailed assessment of this child's clinical history and of the scientific literature concerning PNET survival, I find no appropriate scientific basis for the conclusion that radiation therapy would be curative or would even improve his chance of survival. I am convinced that if the child suffers as adverse a reaction to continued radiation therapy as he did the first two fractions, his chance of survival may be reduced.

Exhibit B includes copies of all MRIs from February 7, 1996 until the present. Those reports reveal that following the complete resection of Zachary's PNET on August 28, 1995, one event of significance occurred in a February 7, 1996 MRI. That MRI was taken subsequent to Zachary's receipt of all sessions of chemotherapy but before commencement of Antineoplastons treatments. The MRI included evidence of a minor enhancement in the location of the original tumor, which was interpreted as either scar tissue, blood vessels, or a possible tumor recurrence. Subsequently on March 4, 1996, Zachary received two fractions of radiation therapy. From March 29, 1996 until the present, Zachary has received Antineoplastons treatments. None of the two subsequent MRIs (April 25, 1996 and June 20, 1996) has revealed the presence of the minor enhancement found on February 7, 1996. Indeed, none has revealed any tumor recurrence whatsoever.

Now that the requested information is before the agency, we respectfully request a response at the earliest possible moment. If consideration of our request requires more than twenty-four hours' time, please confirm that during the pendency of your review we may continue to supply Zachary Antineoplastons' treatments.

Sincerely,

S. R. Burzynski, M.D., Ph.D.

cc: Shawn and Desiree McConnell
Steve Siegel, President, Burzynski Patient Organization
Jonathan W. Emord, Esq.
Kathryn Cook, Esq.

EXHIBIT A

S I M O N E
Protective Cancer Institute
123 Franklin Corner Road Suite 108 Lawrenceville, NJ 08648
609-896-2646 Fax 609-883-7173

July 3, 1996

Shawn and Desiree McConnell
17107 East Calaveras
Fountain Hills, AZ 85268

Dear Mr. and Mrs. McConnell,

I am an Internist (trained at the Cleveland Clinic 1975-1977), a Medical Oncologist (trained at the National Cancer Institute 1977-1982), Tumor Immunologist (NCI 1977-1982), and Radiation Oncologist (University of Pennsylvania (1982-1985) with expertise in nutrition, cancer and other disease prevention.

I am the author of ***Cancer and Nutrition, A Ten Point Plan to Reduce Your Risk of Getting Cancer*** (325 pages, 1982 McGraw-Hill, revised 1992 and 1994 Avery Publishing). I also authored ***Breast Health*** (410 pages, 1995 Avery Publishing), and ***Shark Cartilage and Cancer*** (1995 Paradigm Press).

I am investigating lifestyle changes, including nutrient supplementation, with the use of shark cartilage in the treatment of advanced cancers. The study protocol is sponsored by the National Institutes of Health and approved by the Food and Drug Administration.

I have testified as an expert witness in the areas of cancer and lifestyle for the State of New Jersey 1995. I have also testified for the United States Senate and the House of Representatives in 1995, 1994, and 1993 as an expert in the fields of cancer, nutrition, cancer prevention, the benefits of food supplementation, and medical care costs reduction. I have testified as an expert in a deposition concerning the benefits of food supplements in relation to disease and disease prevention recently (Sheri Lieberman, Ph.D. vs American Dietician Association).

STATEMENT:

I was asked to review and comment on Zachary McConnell, who is 7 years old diagnosed with a rare brain malignancy, Primitive Neuroectodermal Tumor (PNET) located in the right frontal temporoparietal region. On August 28, 1995, a complete resection of all visible tumor was performed and a post surgical MRI scan done on August 30, 1995 revealed no visible tumor.

The patient was placed in a Phase II non-randomized clinical trial, CCG-9931, Groupwide Pilot Study of Neoadjuvant Chemotherapy followed by Cranial-Spinal

Hyperfractionated Radiotherapy in Patients with Newly Diagnosed High Risk PNET. From September 14, 1995 to early January 1996, the patient underwent and completed the prescribed five cycles of chemotherapy consisting of vincristine, etoposide, carboplatin, cisplatin, and cyclophosphamide. Because of the severe side effects of these agents he had seven blood transfusions, was hospitalized on eight different occasions for side effects (three times for administration of chemotherapy and five times for side effects), was put on both oral and intravenous antibiotics for sepsis and other infections, had profound neutropenia, permanent loss of some hearing, and loss of considerable body weight for him (weight before chemotherapy was 18.8 kilograms, weight after the five cycles of chemotherapy, 16.8 kilograms), and also experienced nausea, high fevers, vomiting, gastroenteritis, and mood swings.

On September 7, 1995, October 31, 1995, and November 30, 1995, follow-up MRI scans were performed, each of which revealed no visible tumor. On February 7, 1996 another post surgical MRI scan revealed the presence of a minor enhancement in the location of the original tumor, which was interpreted as either scar tissue, blood vessels, or a possible tumor recurrence.

The first radiation treatment of this investigational protocol did not begin until March 4, 1996 at which time Zachary suffered a severe headache and continuous vomiting. The parents decided to terminate Zachary's participation in the study due to all of the side effects that he experienced with the one fraction of radiation and the prior chemotherapy. The parents then did their own investigation on other treatments, went to Dr. Burzynski, and began on Antineoplastons on March 19, 1996. The patient received reduced doses of Antineoplastons for the first ten days followed by standard doses March 29, 1996 and thereafter. On April 25, 1996 an MRI scan revealed no visible tumor mass. The mass seen on the February 6, 1996 MRI was not present on the April 25, 1996 scan. Zachary has received Antineoplastons treatments from March 19 until the present. On June 20, 1996, a follow-up MRI was done. It again showed no visible tumor mass.

I am asked to comment on whether standard therapy, i.e. radiation therapy, would be beneficial to the patient rather than have the patient continue on the Antineoplastons protocol.

The first point is that there is no *standard therapy* for PNET. In fact, the Groupwide Pilot Study is an unproven investigational protocol. That study and others like it are experimental, performed in an attempt to determine what treatment(s) could most benefit patients with PNET.

It is my opinion as a medical oncologist, tumor immunologist, and radiation oncologist, that there is no scientific way to predict whether the failure to administer radiation therapy to Zachary McConnell will have a "significant" effect on his chances of "cure." As we know, the word "cure" as applied to cancer care denotes a contrived definition - the number of patients alive at the end of a certain

period of time; the worse the tumor, the shorter that period of time is simply to make our statistics "look good." If, for instance, a person lives five years and one day, that person is considered "cured" but is dead nonetheless.

Zachary McConnell was enrolled in an investigational protocol and received experimental chemotherapies and one fraction of radiation therapy. The radiation therapy was never completed. He then received Antineoplastons. These events make an estimate of "affect on [his] chances of being cured" with radiation impossible to determine with any certainty. There are several reasons for this.

First, the original investigational protocol (above) that Zachary was scheduled to receive was terminated by his parents after he completed all the chemotherapy and after he received only two fractions (a single day: one in the morning and one in the afternoon) of a hyperfractionated (two radiation treatments per day) schedule over several weeks. Therefore, one cannot rely upon the percentage rate of "cure" associated with the Groupwide Pilot Study due to the fact that treatment has been interrupted for over three months. Moreover, another variable, Antineoplastons treatment, in the intervening three months since the cessation of radiation therapy, further complicates the picture, making prediction impossible.

Hence, it is thus necessary to consider radiation therapy as a new treatment and independent of the original investigational chemotherapy protocol.

Second, the Groupwide Pilot Study is itself an experimental investigational protocol, not "standard" therapy. It has neither been proven safe or efficacious and, thus, it is not *predictably* curable as a treatment for juvenile PNET.

Third, Zachary had severe reactions to the chemotherapy and unusually severe and adverse reactions to the two fractions of radiation treatment administered. Those reactions make it extremely difficult to predict whether the continuation of radiation would prove to be curative or would result in either death from the side effects of treatment or from complications associated with them.

Fourth, radiation treatments to Zachary now must be considered as an independent modality and as a *salvage* modality since there was evidence of possible tumor recurrence after chemotherapy. Therefore, radiation therapy, when administered to children under age 10 with recurrent PNET, will not change survival.

Through CANCERLIT, I have reviewed the literature for PNET clinical trials. The pertinent studies will be briefly discussed. The only randomized published peer-reviewed study I found, which is the best type of clinical study, was that of Dr. Cohen and others from the Cleveland Clinic Foundation in Ohio. They randomized 55 patients to receive either: (1) craniospinal radiation therapy followed by eight cycles of CCNU, vincristine, and prednisone; or, (2) two cycles of "eight drugs in one day" chemotherapy followed by the same radiation therapy, followed by eight additional cycles of "eight drugs in one day" chemotherapy.

There was no difference in survival in either group. (Cohen, BH, et al. 1995. Prognostic factors and treatment results for supratentorial PNET in children using radiation and chemotherapy: a Children's Cancer Group randomized trial. *J Clin Oncol* 13(7):1687-96).

At a meeting presentation, Dr Boyett (et al) reviewed 203 children who were randomized to receive either: (1) "eight drugs in one day" chemotherapy before and after radiation therapy; or, (2) vincristine during radiation followed by vincristine, CCNU, and prednisone after radiation therapy. Group one's treatment ("eight drugs in one day" before and after radiation therapy) was less effective with regard to progression-free survival. (Boyett, J, et al. 1995. Progression-free survival and risk factors for PNET of the posterior fossa in children: Report of the Childrens Cancer Group [CCG] randomized trial CCG-921 Meeting Abstract. *Proc Annu Meet Am Soc Clin Oncol* 14:A283).

At another meeting presentation, Dr. S Skapek (et al) presented findings of a retrospective review of 20 children treated for PNET. One patient had surgery alone, one patient had surgery and chemotherapy, eight patients had surgery and radiation therapy, and ten patients had surgery, chemotherapy, and radiation therapy. The median overall survival was 64 months. The group that was slightly better had surgery, chemotherapy, and radiation therapy in sequential order. However, cheomotherapy did not change survival when it was used to salvage a tumor recurrence. (Skapek, et al. 1994. Clincial outcome in children with CNS PNET outside of the posterior fossa. Meeting Abstract. *Proc Annu Meet Am Soc Clin Oncol.* 13:A497).

Zachary is now not the typical patient. He had chemotherapy, possible recurrent tumor after the chemotherapy, two fractions of hyperfractionated radiation therapy, and Antineoplastons. A patient with PNET should participate in an investigational protocol so that information could be utilized for future patients. *Generally* any treatment will produce a response or not in a period of about 2 to 2 1/2 months. Therefore, if Antineoplastons fail the patient, conventional radiation therapy could always be utilized. Antineoplastons treatment were begun on March 19, 1996 and have continued to the present with varying dosage levels. Nevertheless, during this three month period there has been no visible sign of tumor recurrence. In addition, it is significant that the MRI enhancement observed on February 6, 1996 before the Antineoplastons treatments began, is no longer present following Antineoplastons administration.

Based on all of the foregoing, I conclude that failure to administer the radiation therapy to this patient as previously planned cannot be shown by any available scientific measure to affect in any significant way Zachary McConnell's chances of being cured (considering the surgery and chemotherapy he has already received). Given my detailed assessment of this child's clinical history and of the scientific literature concerning PNET survival, I find no appropriate scientific basis for the conclusion that radiation therapy would be curative or would even

improve his chance of survival. I am convinced that if the child suffers as adverse a reaction to continued radiation therapy as he did to the first two fractions, his chance of survival may be reduced.

Executed on July 3, 1996. Charles B Simone MD

Charles B. Simone, M.D.
Director

CURRICULUM VITAE

Name: Charles B. Simone, M.MS., M.D.

Present Address: 16 Balsam Court
Lawrenceville, New Jersey 08648
609-853-4147

Date and Place of Birth: June 21, 1949 in Trenton, N.J.

Marital Status: Married, two children.

Military: 1977-1982 Commander, U.S. Navy, Public Health Service

Education:

1967-1971 - B.A. (Biological Sciences) - Rutgers University, New Brunswick, N.J.

1971-1975 - M.MS. and M.D. - Rutgers Medical College Piscataway, N.J.

Positions Held:

1967-1971 Research Assistant to Ralph J. DeFalco, Professor of Immunology, Rutgers University, New Brunswick.

1970-1972 Consultant for criminal investigations requiring immunological corroborations.

1971-1972 Acting Chairman of Rutgers University Serological Museum.

1975 Research Appointment with Robert A. Good, M.D., Ph.D., President and Director of Memorial Sloan Kettering Cancer Hospital, New York City.

1975-1976 Internship, Department of Medicine, The Cleveland Clinic Foundation, Cleveland, Ohio.

1976-1977 First Year Assistant Resident, Department of Medicine, The Cleveland Clinic, Cleveland, Ohio.

1977-1979 Clinical Associate, Immunology Branch, National Cancer Institute, NIH, Bethesda, Maryland.

1978-1980 Clinical Assistant Professor of Medicine, George Washington University School of Medicine, Washington, D.C.

1979-1980 Clinical Associate, Medicine Branch, National Cancer Institute, NIH, Bethesda, Maryland.

1980-1982 Investigator, Clinical Pharmacology Oncology

Branch, National Cancer Institute, NIH, Bethesda.

1980-pres Founder, Simone Protective Cancer Center

1982-1985 Radiation Therapy Department, Hospital of the University of Pennsylvania, 3400 Spruce Street, Philadelphia, Pennsylvania 19104.

1984-1989 Consultant, New Jersey Education Oncology Program.

1984-1987 Advisor, U.S. Postal Service, Preventive Health.

1984-pres Speaker for the American Cancer Society

1985-1989 Associate Professor, Radiation Therapy and Nuclear Medicine Department, Thomas Jefferson University Hospital, Philadelphia, PA.

1985-1988 Chief Breast Section, Radiation Therapy and Nuclear Medicine Department, Thomas Jefferson University Hospital, Philadelphia, PA.

1985-1989 Consultant, Immunobiochemistry for BASF

1985-1988 Chairman, Departmental Publications Review Committee

1986-1991 Consultant for Hoffmann-LaRoche, Nutley, NJ

1986-1988 Member, Jefferson Hospital Nutrition Committee

1986-1988 Speaker for Jefferson Educational Program

1988-pres Medical Advisor to N.J. Governor - Substance Abuse

1989-pres Consultant to Spain

1989-pres Consultant to Cambodia

1989-pres Consultant to Russia

1990-pres Medical Advisor to National Alliance of Breast Cancer Organizations

Certification:

Diplomate of the National Board of Medical Examiners 1976

American Board of Internal Medicine, Eligible 1976

Medical Oncology Subspecialty Board, Eligible 1980

Allergy and Immunology Board, Eligible 1980

Radiation Therapy Subspecialty Board, Eligible 1983

Awards:

Honorable Mention Award - SAMA Research Forum (April 1973)

Visiting Professor in Rheumatology, Cleveland Clinic (1978)

Visiting Professor in Clinical Immunology, University of Hawaii (1979)

Elected into New York Academy of Sciences 1983

Elected into American College of Immunologists 1983

American Academy of Sciences 1984

Elected, Who's Who in Frontiers of Science and Technology 1984

Elected, Contemporary Authors 1984

Author Citation Award, Nineteenth Annual New Jersey Writers Conference, NJ, Institute of Technology, March 1986

Visiting Professor in Immunology/Oncology, Cleveland Clinic 1987 and 1989

Invited/Special Lectures:

Lecture to Radiation Therapeutic Oncology Group 1983

Keynote Speaker - 18th Annual Congress, AACIA 1984

Keynote Speaker - Annual Cancer Symposium, University of Louisville 1985

Speaker - New Jersey State Justice Department 1985

Speaker - United States Arsenal, Picattiny, NJ 1985

Keynote Speaker - New Jersey Superintendents' and Principals' Convention 1985

Keynote Speaker - New York Open Center 1986

Keynote Speaker - New Jersey Superintendents' and Principals' Convention 1986

Keynote Speaker - New Jersey State Kiwanis Club

Speaker for Jefferson Outreach

Keynote Speaker - NJ Superintendents' and Principals' Convention 1989

Societies:

New York Academy of Sciences

American College of Immunologists

American Academy of Sciences

Contemporary Authors

Military Service:

1977-1982 Commander, U.S. Public Health Service, Navy.

Licensed to Practice Medicine:

New Jersey
Pennsylvania
Ohio
Maryland

BIBLIOGRAPHY

1. Katz, P., C.B. Simone, P.A. Henkart, and A.S. Fauci. 1981. Mechanisms of antibody-dependent cellular cytotoxicity. The use of effector cells from chronic granulomatous disease patients as investigative probes. J. Clin. Invest. 65:55-63.

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Implant at Time of Lumpectomy for Breast Cancer. Radiology. Nov 1987 Supplement.

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15. Simone, C.B., and V. Simone. 1992. A Healthy Start In press.

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18. Simone, C.B. 1991. Directed human effector cells kill human cancer in vivo in nude mice. Submitted.

17. Simone, C.B. 1992. Anti-Thy 1.2-directed effector cells cause marked T cell reduction in Balb/c mice. Submitted.

18. Simone, C.B. 1990. AIDS, A Complete Review. Manuscript submitted.

EXHIBIT B

Good Samaritan Regional Medical Center
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Phoenix, AZ 85006

Phoenix Children's Hospital
1111 East McDowell Road
Phoenix, Arizona 85006

MAGNETIC RESONANCE IMAGING

PATIENT NAME: MCCONNELL,ZACHARY REED DATE OF EXAM: 02/06/96

EXAM: MRI BRAIN/CERVICAL SPINE MRN: 67-28-43

ORDERING PHYSICIAN: ETZL, MICHAEL M. DATE OF BIRTH: 04/02/1989 AGE: 06

X-RAY NO. 672843 ACCOUNT NO: 350150 LOCATION: AC SEX: M

FINDINGS

MRI BRAIN

CLINICAL HISTORY: Follow-up PNET.

Routine MR imaging of the brain was performed pre and post [illegible] administration. Post surgical changes involving the insular cortex are noted. Mild encephalomalacic changes are present as well as hemosiderin deposition in the brain parenchyma. There has been no change since the previous study of 10/31/95. No abnormal foci of contrast enhancement to suggest tumor recurrence is seen.

MRI CERVICAL SPINE

Sagittal and axial imaging of the cervical spine shows the [illegible] bodies to be normal in signal intensity. No abnormal contrast enhancement is seen within the cervical spinal canal. The [illegible] normal in signal intensity.

IMPRESSION

Post surgical changes in the right insular cortex. No evidence of tumor recurrence.

SCHIMEL,SANDRA R
DDADT 02-06-96/02-06-96
SS /DC

c: ETZL,MICHAEL M

Sandra Schimel, M.D.

CHART COPY

RADIOLOGIST/NUCLEAR PHYSICIAN

MAGNETIC RESONANCE IMAGING

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S. David Mintz, M.D

PULMONOLOGY
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Gerald D. Gang, M.D
Peggy J. Redford, M.D

April 9, 1996

Michael M. Etzl, Jr., M.D
Phoenix Children's Hospital
909 East Brill Street
Phoenix, Arizona 85006

RE: MC CONNELL, ZACHARY
MR#: 67-28-43
DOB: 04/02/89

Dear Dr. Etzl:

I saw Zachary in follow up in Neurosurgery Clinic today. Zachary underwent craniotomy for resection of primitive neuroectodermal tumor and has been doing exceptionally well since surgery.

He underwent a follow up MRI scan in February which demonstrates a small cystic area in the sylvian region on the right side where the tumor had been resected. The brain tissue has reconstituted itself in the surrounding areas with a very good appearance. There is a minor amount of enhancing membranous appearing tissue in the depths of the tumor bed which may very well represent scar tissue or blood vessels in the piaraschnoid surface. These are the same vessels that I felt I saw at surgery and I have not seen anything to indicate that there is any tumor growth. The spine was negative on the MRI as well.

With his neurologic condition being back to normal and no focal deficits and no worsening of his condition since that time, I am very pleased to note how well he has done and to note the good course which has followed since surgery. He initiated chemotherapy, but did not do well with the initial doses and seemed to have a number of side effects. His family has chosen another avenue for treatment with doctors in Texas regarding antineoplastics.

The recommendation I have made today is that follow up MRI scan with and without contrast be performed. If we see any increase in the enhancement or any signs of tumor regrowth in the tumor bed, I would at least stress the importance of focal radiation in that area. After this MRI scan, if it remains negative, our next surveillance would be somewhere between four and six months after that

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[illegible]

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MEDICAL EDUCATION
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NEPHROLOGY
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NEUROLOGY
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Saunder M. Barnes, M.D.
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April 25, 1996

Michael Etzl, M.D.
909 East Brill Street
Phoenix, Arizona 85006

RE: MCCONNELL, ZACHARY
MR#: 67-28-43
DOB: 04/02/89

Dear Dr. Etzl:

I appreciated seeing Zachary in follow up in Neurosurgery Clinic today. His MRI scan was obtained to get a follow up of the previously enhancing part of the tumor bed which I felt was possibly either scar tissue or collection of blood vessels and hopefully would dissipate. The MRI today is clear. There is no evidence of any kind of enhancement in the tumor cavity. There is no evidence of any kind of recurrence. The entire cerebrum looks good with no enhancing lesions and the tumor cavity has diminished to the point where it is almost nonvisible.

Neurologically Zachary is doing very well. They are returning to Texas for continuation of his antineoplastic therapy. I would like to obtain another MRI scan with and without contrast in four months from now. I will make the arrangements and will have my office contact the family.

I greatly appreciated the opportunity of seeing Zachary. I will keep you posted as to his progress.

Sincerely,

S. David Moss, M.D.
Pediatric Neurosurgeon

cc: Michael D. Sapozink, M.D.
1111 East McDowell Road
Phoenix, Arizona 85006

The Parents of Zachary McConnell
16507 East Fayette Drive
Fountain Hills, Arizona 85268

Southwest
Neuro-Imaging
2141 East Camelback Road
Suite 110
Phoenix, Arizona 85016
(602) 954-6229
FAX (602) 954-6018

John A. Hodak, M.D.
Richard A. Flom, M.D.
Burton P. Drayer, M.D.
C. Roger Bird, M.D.
Evan K. Fram, M.D.
Bruce L. Dean, M.D.
Joseph E. Heiserman, M.D.
John P. Karis, M.D.

PATIENT NAME: McConnell, Zachary R.

NEURORADIOLOGIC CONSULTATION: 6/20/96

MRI OF THE BRAIN WITH GADOLINIUM:

MR SEQUENCES: The exam is dated 04/25/96. A series of T1, intermediate and T2-weighted axial series were obtained, as well as a pre-contrast sagittal T1-weighted sequence. Post-contrast scans were obtained in the coronal, sagittal and axial planes.

FINDINGS: There has been interval postsurgical resection and treatment for the right-sided tumor. There is residual hemosiderin in the external capsule and minimal encephalomalacia. There is no abnormal enhancement following the intravenous administration of contrast material. There is an incidentally empty sella. There are no drop metastasis demonstrated in the upper portion of the cervical spine down to the level of C4.

CONCLUSIONS:

1. Exam from Good Samaritan dated 04/25/96 is overread at the request of the patient as a courtesy and no charge to the patient.

2. Postoperative section of the right-sided mass and post therapy changes.

3. Residual hemosiderin deposition in the external capsule consistent with postoperative findings or prior hemorrhage related to the tumor.

4. No abnormal enhancement demonstrated, and no apparent recurrent mass.

5. No evidence of hydrocephalus or apparent drop metastases throughout the ventricular system, upper cervical spine, or floor of the anterior fossa.

Bruce L. Dean

Bruce L. Dean, M.D.

dd: 06/20/96 dt: 06/20/96 by: emc/bk

Biltmore Advanced Imaging Center

RADIOLOGY FINAL REPORT

PATIENT NAME: McConnell, Zachary R.

PATIENT #: 01-67-00

DOB: 06-02-89 AGE: M-7

REFERRING PHYSICIAN: Stanislaw Burzynski, M.D.

MRI OF THE BRAIN WITH GADOLINIUM: 06-20-96

CLINICAL HISTORY: Permeative neuroectodermal tumor.

MRI SEQUENCES: T1, intermediate and T2-weighted axial scans were obtained, as well as a T1-weighted sagittal sequence. Post-contrast scans were obtained in the axial and coronal planes.

FINDINGS: Skull base and masticator spaces appear to be unremarkable. There are prominent retropharyngeal nodes consistent with patient's age. Prior postoperative changes are demonstrated on the right. There is a region of hemosiderin deposition in the external capsule on the right. There is no evidence of recurrent mass or abnormal enhancement. The coronal scans reveal no apparent recurrent mass or lesion. The orbits appear to be symmetric without evidence of mass, and there is no evidence of a pineal tumor.

CONCLUSIONS:

1. Hemosiderin deposition in the right external capsule.
2. No abnormal enhancement or mass.
3. No evidence of recurrent tumor or mass.

Bruce K. Dean

Bruce K. Dean, M.D.

[illegible] 06/20/96
[illegible] 06/20/96
by: emc/bk
Outcome Code: 2C

2141 E. Camelback Road, Suite 110 Phoenix, AZ 85016 (602) 954-0954

Biltmore Advanced Imaging Center

RADIOLOGY FINAL REPORT

PATIENT NAME: McConnell, Zachary R.

PATIENT #: 01-67-00

DOB: 04-02-89 AGE: M-6

REFERRING PHYSICIAN: Carol A. Foster, M.D.

BRAIN MAGNETIC RESONANCE SCAN: 08-26-95

HISTORY: Migraine. Dizziness.

PROCEDURE: Sagittal and axial T1-weighted imaging, as well as axial, intermediate and T2-weighted imaging and a FLAIR image.

FINDINGS: There is a mixed signal mass in the right frontal lobe, which has a rather discrete core of decreased signal intensity laterally and a larger core medially that is of higher signal intensity. There is also perilesional edema of the white matter, resulting in a mass of approximately 6 x 8 cm. Shift to midline right to left, approximately 1.5 cm. No hydrocephalus. The remainder of the signal intensity of the brain are normal. No abnormal vascular flow voids. The character of the lesion would suggest a primary brain tumor and in view of the mixed signal intensities and the presence of a rather well-demarcated rim raises the possibility of gangliogliomas.

SUMMARY:

1. Mixed signal mass, right frontal lobe with subfalcial herniation.

2. Dr. Foster notified and patient referred to Good Samaritan Hospital.

Richard A. Flom, M.D.

Richard A. Flom, M.D.

dd: 08/26/95
dt: 08/26/95
by: emc/dd
Outcome Code: 3C

2141 E. Camelback Road, Suite 110 Phoenix, AZ 85016 (602) 954-0954

Phoenix Children's Hospital

OUTPATIENT SPECIALTY CARE CENTER

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September 7, 1995

Michael Etzl, Jr., M.D.
909 E. Brill Street
Phoenix, AZ 85006

Re: MCCONNELL, ZACHARY
DOB: 04/02/89
MRN: 67-28-43

Dear Doctor Etzl:

I saw Zachary McConnell in follow up in the Neurosurgery Clinic today. He has been diagnosed with a primitive neuroectodermal tumor with some neuroblastoma and rhabdosacroma like elements within the tumor.

I believe that our follow-up MRI today shows that the cavity where the tumor was located is decompressing fine. There are some enhancing areas in the medial wall of the tumor border. This area had a distinct capsular membrane with tumor easily dissected and removed from the surrounding brain. The biopsy down deep in this area is negative for tumor. I believe it may be related to a post decompression infarction area of enhancement and probably results in some scar tissue formation. I do not necessarily believe that this is malignant tumor. Observation will be our only way of telling at this point.

We need to go ahead and proceed with our radiation and chemotherapy protocols. We should follow up this tumor with another MRI scan in six weeks with contrast. His staples were removed. His recovery is excellent and he appears to be neurologically intact. There is a small subgaleal fluid collection which will resolve in the next couple of weeks. My office will make arrangements for follow up.

I appreciated the opportunity of seeing him today. I will see

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GASTROENTEROLOGY
239-5780
Kirby R. Ingelus, M.D.
Michael T. Sheehan, M.D.
Mitchell D. Shub, M.D.
Gary L. Sicker, M.D.

HEMATOLOGY/ONCOLOGY
239-5763
Paul V. Benaroo, M.D.
Michael M. Etzel, Jr., M.D.
Dee Anne Singer, M.D.
Terry B. Wood, M.D.

INPATIENT PEDIATRICS
239-3148
David M. Sullivan, M.D.

MEDICAL EDUCATION
239-5781
Mark L. Ulster, M.D.
Mitchell D. Shub, M.D.

NEPHROLOGY
239-5784
Mark L. Ulster, M.D.
David L. Wadley, M.D.

NEUROLOGY
239-3455
[illegible]
Richard [illegible], M.D.
Alan M. [illegible], M.D.

NEUROSURGERY
239-4860
[illegible]
S. David Moss, M.D.

PULMONOLOGY
239-5778
[illegible]
[illegible], M.D.
[illegible], M.D.

Michael Etzl, Jr., M.D.
Re: MCCONNELL, ZACHARY
September 7, 1995
Page 2

him as he is readmitted to the hospital and appreciate working with you.

Sincerely,

S. David Moss, M.D.
Pediatric Neurosurgery

SDM/TL987

909 East Bell Street • Phoenix, Arizona 85006

Phoenix Children's Hospital

OUTPATIENT SPECIALTY CARE CENTER

AMBULATORY PEDIATRICS
239-4522
Paul S. Bergeson, M.D.
Julie H. Ernst, M.D.
Janice P. Piatt, M.D.
Ray C. Pinckard, M.D.
Jeffrey C. Weiss, M.D.

BEHAVIOR/DEVELOPMENT
239-3751
Eric Benjamin, M.D.
Thomas R. Dillerstromer, Ph.D.
Randall K. Ricardi, D.O.
Karlsson Roth, Ph.D.
David L. Wodrich, Ph.D.

CRITICAL CARE
239-3563
Paul R. Bakerman, M.D.
David H. Beyda, M.D.
Paul H. Liu, M.D.
Janet W. Tallez, M.D.

ENDOCRINOLOGY
239-4644
Robert D. Clemons, M.D.
Khalid S. Hasan, M.D.
Alvin H. Perelman, M.D.

GASTROENTEROLOGY
239-5780
Kristy R. Ingebo, M.D.
Michael T. Shelton, M.D.
Mitchell D. Shub, M.D.
Gary H. Silber, M.D.

HEMATOLOGY/ONCOLOGY
239-3788
Paul V. Baranko, M.D.
Michael M. Etzl, Jr., M.D.
Cara Anne Singer, M.D.
Terry S. Wood, M.D.

INPATIENT PEDIATRICS
7-3146
David M. Solomon, M.D.

MEDICAL EDUCATION
239-5781
Melvin L. Cohen, M.D.
Mitchell D. Shub, M.D.

NEPHROLOGY
239-5784
Melvin L. Cohen, M.D.
Drew C. Cohen, M.D.

NEUROLOGY
239-4855
Saunder M. Barnes, M.D.
Ronald O. Hadden, M.D.
Allen M. Kaplan, M.D.

NEUROSURGERY
239-4880
Kim H. Marmaring, M.D.
S. David Moss, M.D.

PULMONOLOGY
239-5778
Barry J. Fisher, M.D.
Gerald D. Gong, M.D.
Peggy J. Radford, M.D.

October 31, 1995

Michael Etzl, Jr., M.D.
909 East Brill Street
Phoenix, Arizona 85006

Re: MCCONNELL, ZACHARY
DOB: 04/02/89
MRN: 67-28-43

Dear Doctor Etzl:

I appreciated the opportunity of seeing Zachary in follow up in Neurosurgery Clinic today. Zachary has had a resection of a right temporal parietal primitive neuroectodermal tumor that may have had some components of rhabdomyosarcoma involved. I am extremely pleased to note that the MRI scan is totally clear of any enhancement consistent with any kind of tumor. The cavity is completely obliterated and the brain has reassumed its normal position. I am extremely pleased to see the postoperative configuration of the brain. I am also noting a small subdural chronic effusion over the high right parietal convexity which may at some point be a centimeter thick. This may be causing him some irritability in changing his behavior. It may also cause headaches. Some of the will resolve on their own, but many may need drainage and a shunting procedure. I would like to see him back in four weeks with another CT scan of the head to see if this effusion is enlarging or if it has remained stable. I will keep you posted as to our progress and appreciate working with you.

Sincerely,

S. Davis Moss, M.D.
Pediatric Neurosurgery

SDM/TL909

cc: Mr. and Mrs. McConnell
16507 East Fayette Drive
Fountain Hills, Arizona 85268

909 East Brill Street • Phoenix Arizona 85006

Phoenix Children's Hospital

OUTPATIENT SPECIALTY CARE CENTER

AMBULATORY PEDIATRICS
239-4822
Paul S. Bergeson, M.D.
Jose H. Ernst, M.D.
Janice P. Pratt, M.D.
Kay C. Pinckard, M.D.
Jeffrey C. Weiss, M.D.

BEHAVIOR/DEVELOPMENT
239-6731
Eric Benjamin, M.D.
Thomas P. DiBartolomeo, Ph.D.
Randal K. Ricardi, D.O.
Karlsson Roth, Ph.D.
David L. Woodrich, Ph.D.

CRITICAL CARE
239-3563
Paul R. Bekerman, M.D.
David H. Beyda, M.D.
Paul H. Liu, M.D.
David W. Tekez, M.D.

ENDOCRINOLOGY
239-4844
Robert D. Clemons, M.D.
Khaled S. Hasan, M.D.
Alvin H. Perelman, M.D.

GASTROENTEROLOGY
239-5780
Kristy R. Ingabo, M.D.
Michael T. Shelton, M.D.
Michael G. Shub, M.D.
Gary H. Silber, M.D.

HEMATOLOGY/ONCOLOGY
239-5785
Paul V. Barasso, M.D.
Michael M. Etzl, Jr., M.D.
Dale Anne Singer, M.D.
Terry S. Wood, M.D.

INPATIENT PEDIATRICS
239-3146
David M. Solomon, M.D.

MEDICAL EDUCATION
239-6781
Melvin L. Cohen, M.D.
Michael G. Shub, M.D.

NEPHROLOGY
239-5784
Melvin L. Cohen, M.D.
Drew C. Cutler, M.D.

NEUROLOGY
239-4655
Saunder M. Bernes, M.D.
Ronald C. Hadden, M.D.
Allen M. Kaplan, M.D.

NEUROSURGERY
239-4880
Kimmy Mamelling, M.D.
S. David Moss, M.D.

PULMONOLOGY
239-5778
Barry J. Fisher, M.D.
Gerald C. Gong, M.D.
Peggy J. Radford, M.D.

November 30, 1995

Michael Etzl, M.D.
909 East Brill Street
Phoenix, Arizona 85006

Re: MCCONNELL, ZACHARY
DOB: 04/02/89
MRN: 67-28-43

Dear Doctor Etzl.

I appreciated seeing Zachary in follow-up in Neurosurgery Clinic today. His subdural effusion is completely resolved with and without contrast CT today. I see nothing of any concern with any kind of tumor enhancement and the cavity where the tumor was removed is completely collapsed. Overall I am thrilled to see how well Zachary is doing. I am anxious to see him again in three months after his radiation and chemotherapy are completed so that we can assess the contrast scan at that time. He has no neurologic deficits that I can discern and I am pleased to see him again. I wish him well over the holidays and follow-up arrangements will be made. Thank you very much.

Sincerely,

S. David Moss, M.D.
Pediatric Neurosurgery

SDM/TL909

cc: Mr. and Mrs. McConnell
16507 East Fayette Drive
Fountain Hills, Arizona 85268

COPY

909 East Brill Street • Phoenix Arizona 85006

Mr. BURTON. Thank you, Mr. Chairman.
Dr. Simone.

STATEMENT OF CHARLES B. SIMONE, M.D., ONCOLOGIST AND CANCER RESEARCHER

Dr. SIMONE. I have a graph that I'd like to review, if that's possible. It's supposed to be put up on the screen.

Mr. BURTON. Sure.

Dr. SIMONE. Good. I may have to get up and—they have the wrong graph, but I'll use this one.

Mr. BURTON. No, that's all right. Just a second; we'll get the right graph. Do you have the other graphs there? Are they numbered in any way, Doctor? Just 1 second. Just 1 second; we'll get it correct.

Well, let me just give you some of my background.

Why don't you pick that up from the counsel here? OK? Thank you. Careful, you might become a Member of this place, walking up that close. [Laughter.]

Dr. SIMONE. I want to thank you, Chairman Burton and the other Members of the Congress here, who are here——

Mr. BURTON. Well, let me just say, I know that it's disconcerting not to have all the Members here, like you've seen on television when we have these scandal hearings, but I will tell you this, that they will be informed about this. I give you my word.

Dr. SIMONE. That is important.

Mr. BURTON. I want to make sure that everybody knows about it.

Dr. SIMONE. Good.

I'm a medical oncologist. I trained at the National Cancer Institute. I'm also an immunologist, trained there as well, and I'm a radiation oncologist, having trained at the University of Pennsylvania. So I'm very well-grounded in conventional medicine, as you know.

But billions of dollars have been invested in cancer treatments over the years, and since 1971, when President Nixon declared war on cancer, the incidence of cancer actually went up. Cancer will emerge the No. 1 killer of people in this country by the year 2000, and two of every five people in this room will get cancer.

The key thing with any cancer treatment, whatever it is, is whether it's going to cure a patient—that is, extend the life of a patient. Let's just concentrate a little bit on the graph. You can see one line dramatically going up. That's the death rate from lung cancer. So since 1930 to the present time, we've made little or no progress in that, obviously. Only one cancer has come down, and that's stomach cancer, the line going down from left to right. Because of refrigeration, less food additives, less stomach cancer.

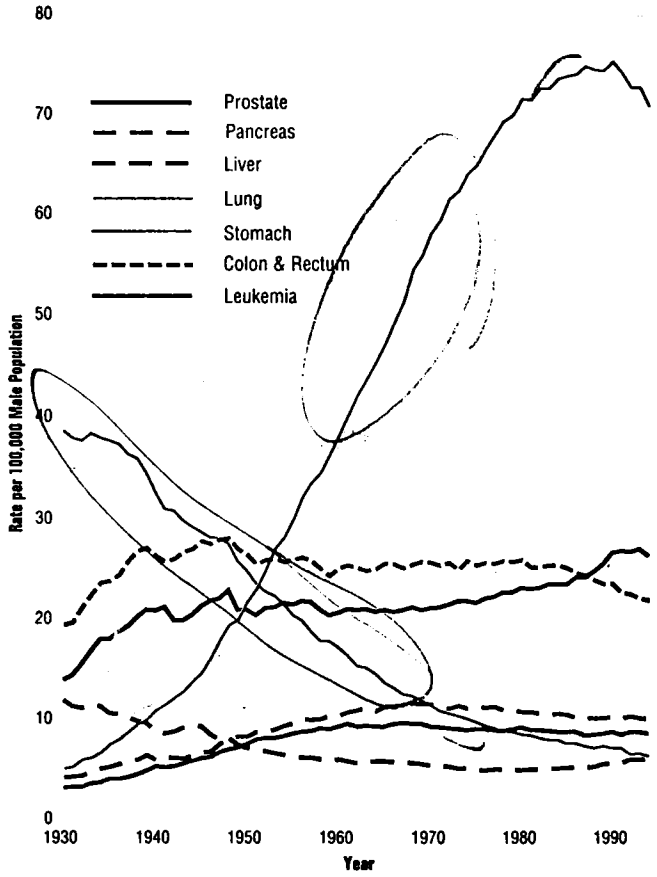
But the important, salient feature of that graph—and it's a very busy graph—but the important thing about this whole conference today is that all the other lines are horizontal, which means one thing: Since 1930, we made little or no progress in the treatment

of adult cancers, and that is despite surgery; that is despite radiation therapy in the twenties and thirties; that's despite combination chemotherapy that began in the sixties, and immunotherapies in the seventies.

[The chart referred to follows:]

CA CANCER J CLIN 1998;48:6-29

Figure 4
Age-Adjusted Cancer Death Rates*
for Males by Site, United States, 1930-1994



Note: Due to changes in the ICD coding, numerator information has changed over time. Rates for cancer of the liver, lung, and colon & rectum are affected by these coding changes.

*Rates are per 100,000 population and are age-adjusted to the 1970 US standard population.

Data source: Vital Statistics of the United States, 1997.

Dr. SIMONE. All the fancy MRI scans, CAT scans, all the treatments, all the diagnosis we've made and done, we've made little or no progress, which means a couple of things.

There are people here talking about prostate cancer and breast cancer. A person who gets breast cancer today will live essentially as long as a woman who got it in 1930. A person who got prostate cancer today will live essentially as long as a person who got it in 1930. That's the cold, hard facts that we have to deal with. And despite all the hoopla, the lobby groups, the media, there are the facts. Because of that, we really need to turn our attention to other forms of treatment, whatever they are, and that's what we need to look at.

There's been a number of issues. I think the Honorable Waxman talked about that we should not pay for treatments that don't work. Well, we've done lots of treatments there, as you can see, from the thirties to the present, and little or no effect has been made on lifespan. So maybe we shouldn't pay for those treatments either, if we're being consistent about what we talk about.

Efficacy is required, and I think we should all adhere to scientific method. I firmly agree with that. I'm a rigorous scientist in everything I do, and we're doing a current study now, sponsored originally through the Office of Alternative Medicine, but also permitted through the FDA. So we're rigorously looking at a few treatments and complying with all the regulations.

There are a number of issues that I think the FDA should look at. First of all, in the USA Today it is reported that the FDA discovered a new drug, licensed it to another group, a pharmaceutical group, and now that pharmaceutical group has determined that they probably will have saved $100 million, and the taxpayers are due those moneys.

I think other things that the FDA should look at, there should be easier access, as you pointed out, Chairman Burton, for terminally ill patients, no matter what the issue is. Whatever drugs are currently available under study in this country, they should be available, as other people have pointed out, too.

I think all physicians should have access to off-label provisions of drugs. That will help them use the drugs that are currently available for any types of treatments that they wish.

There should be a new time limit for FDA review of new applications, provided that the drug had prior approval in the United Kingdom or in the European Medicines Evaluation Agency. These are two key things. If drugs have passed muster in other well-controlled countries, there should be little entry problems into this country. There's lots of data out there to support that.

I think we should discontinue the FDA's discovery research because we have many other governmental agencies that look at this. If there's a big backlog, as it says there is, let's hire some additional staff in the short term, a few million bucks, whatever it might be, to hire the staff to clear the backlogs to start anew.

I think we need to rescind the FDA's regulatory authority within a single State that was just broadened in its scope in the new bill. I think, also, informed consent should really be part of everybody who's getting cancer treatment. I'm a cancer doctor. So I'm talking about cancer care. If informed consent were truly given to a pa-

tient, they might think twice or three times about these treatments that produce those lines in the graph.

And misinformation abounds. We constantly hear about misinformation all the time. In fact, the New York Times reported a physician who was interviewed from Memorial Sloan-Kettering saying misinformation about certain vitamins and minerals in the treatment of cancer, that they interfere with chemotherapy; folic acid interferes with a particular chemotherapeutic agent, and these are simply not true. This is wrong information.

I ran out of time, and I went over hastily. So I just wanted to go through the key points——

Mr. BURTON. How much more time do you need?

Dr. SIMONE. Well, I've actually truncated my talk. The whole talk really explores all these issues in detail.

Mr. BURTON. Well, maybe when we get to questions and answers, you can elaborate a little bit more, but what we'd like to do is we'd like to have any information you have for the record, so we can review them, condense them down. We'd like for you to condense them down as much as possible, because Members of Congress, when we submit this to them—and we'll try to get it out to as many Members as possible—when we submit this information to them, I can tell you, because we have huge volumes of things to go over——

Dr. SIMONE. Sure.

Mr. BURTON [continuing]. With our staffs on a regular basis, that I like to use the KISS method; you know, keep it simple, so that we can make sure that they digest as much as possible.

Dr. SIMONE. I think the simple thing is that we have done lots of treatments in the last 7 years—minimal effects as far as lifespan promotion. We need to look at other issues, whatever they may be, wherever they are.

[The prepared statement of Dr. Simone follows:]

Brief Background of Charles B. Simone, M.MS., M.D.

Charles B. Simone, M.MS., M.D. graduated from Rutgers Medical College (1971-1975). He is an **Internist** (trained at the Cleveland Clinic 1975-1977), **Medical Oncologist** (trained at the National Cancer Institute 1977-1982), **Tumor Immunologist** (trained at the National Cancer Institute 1977-1982), and a **Radiation Oncologist** (trained at the University of Pennsylvania 1982-1985). Working with Senator Harkin, he helped to shape the Office of Alternative Medicine, National Institutes of Health. In addition, he also helped to organize a Department of Alternative Medicine for Columbia and Mt. Sinai in NYC.

While at the NCI, his basic science research uncovered the fundamental mechanism of how human white blood cells kill, helped show how "complement" proteins aid in killing, demonstrated how adriamycin works, and developed *directed effector cells.*

One of the first patients he consulted with at the NCI was a senior statesman who was dying of malnutrition secondary to his cancer. Later, a man his own age with a newly pregnant wife came to him at the NCI and asked to be kept alive for the birth of his child. An intensive course of chemotherapy cleaned out the cancer cells, but the patient failed to improve. "I decided at last resort to put him on high doses of vitamins and minerals that quickly produced a temporary recovery.." The man lived to see the birth of his son.

He began devoting some of his time investigating the effects of nutrition on cancer and the possibilities of cancer and disease prevention. The result was ***Cancer and Nutrition, A Ten Point Plan to Reduce Your Risk of Getting Cancer*** (1981 McGraw-Hill, revised 1994 Avery). He also wrote ***Breast Health, Shark Cartilage and Cancer, Prostate Health, KidStart,*** and ***Sports and Nutrition***.

He has testified as an expert in the fields of cancer, nutrition, nutritional supplementation, disease prevention, and medical care cost reduction before the United States Senate and the United States House of Representatives of 1993, 1994, and 1995.

THE NEED FOR CHANGE

Billions of dollars have been invested in cancer research and treatment since 1971 when President Nixon declared War on Cancer. Each month, it seems, new therapies are trumpeted. Some show promise, most fizzle quickly.

Cancer will emerge as the number one cause of death in the United States by the year 2000. Despite the enormous effort to combat cancer, the number of new cases of nearly every form of cancer has increased annually over the last century. Still worse, from 1930 to the present, despite the introduction of radiation therapy, chemotherapy, and immunotherapy with biological response modifiers, despite CT scans, MR scans, and all the other new medical technology – life spans for almost every form of ***adult*** cancer except cervical cancer and lung cancer have remained constant, which means that there has been no significant progress in cancer treatment (Figure 1 attached, data from the National Cancer Institute SEER Program, published by the American Cancer Society each January).

"Cure" is largely elusive or statistically disguised. "Cure" means surviving 5 years after treatment - if death occurs at 5 years and one day, the "cure" is unaltered in the statistical record.

The chilling prospect remains - by the year 2000, two of every five Americans will develop cancer. And most will die.

Because of these dismal survival statistics with existing conventional treatments, we need to redirect our attention to two important areas. *Prevention* of cancer and other diseases; and pursue totally *New Substances* or *New Modalities* that show scientific merit for treatment even though they may not yet be approved by the Food and Drug Administration for widespread use.

FDA REFORM ??

Because FDA funding must be used for more food safety work, it has been estimated that there will be a 60% to 70% reduction in funds that would otherwise be assigned to tenure-track scientists in the Division of Viral Products, Center for Biologics Evaluation and Research (Feinstone SM, Lewis AM, Markoff LJ, Carbone K, Golding H.[all lab chiefs in that Division] *Science*. January 9, 1998; 279:157-159.

> We certainly do not want to lose excellent scientists, however, the dollars earmarked for research should be used only for evaluation of. Other governmental agencies are organized for research.

Henry I. Miller, from the Hoover Institution, Stanford University, writes about FDA Reform in the same cited *Science* article:

> 'First, it calls for "promptly and efficiently reviewing clinical research" "in a timely manner." But these words will not have any impact on the agency's 30 year tradition of risk aversion and foot-dragging.
>
> Second, it calls on FDA to develop a plan by the year 2000 to clear the legendary backlog of products awaiting approval. Congress here makes itself a hostage to an endless series of demands for additional resources the FDA will say it needs to do this.
>
> Third, it codifies many policies that are already in place, giving the impression of a lengthy list of improvements.
>
> The most important provision offers drug companies greater latitude in supplying scientifically sound information to doctors about drugs' "off label" uses (those not yet approved by FDA). Companies are currently prohibited from distributing such critical information. But even this improvement comes at a high price: substantial additional paperwork to convince FDA that formal applications for approval of the new uses are forthcoming.

[This provision of "off label" uses is very valuable for the patient. CB Simone, M.D.]

A welcome provision permits manufacturers to submit "health care economics information," such as data on a drug's cost-effectiveness, to hospitals and HMOs.

The bill contains other minor improvements, such as loosened restrictions on health claims for food products and expanded use of third party, including academic institutions, to review medical devices.

However, one provision actually *increases* the scope of FDA's regulation by expanding its jurisdiction to activities that occur completely within a single state – small-scale research by an academic or a practicing physician testing an innovative therapy.

Many critical reforms recommended by blue-ribbon panels are absent. These include reducing the redundancy of regulation of early-stage clinical trials and a binding reciprocity provision that, for example, would limit the duration of FDA review of a new drug to a maximum of, say 60 days after its approval in the United Kingdom or by the European Medicines Evaluation Agency (thereafter, the FDA would have to show cause why the drug should not be marketed in the United States, or it would automatically be approved).

Following Congress's failure to accomplish significant FDA reform, the costs of drug development (already averaging more than $500 million to bring a single product to market) will continue to rise, fewer drugs will be developed, and market competition will erode. Patients will suffer higher prices and benefit from fewer breakthrough drugs.'

FDA Should Not Be In The Drug Discovery Business

"FDA finds potential cancer treatment" (USA Today 12-12-97)

The Associated Press reported in USA Today on December 12, 1997 that the FDA "found a promising new treatment for cancer and licensed it" to Neopharm Inc, an Illinois biotechnology company. The private firm will need FDA approval to sell it. Obviously, the FDA has a conflict of interest for this approval. The chief executive of Neopharm, William Govier, said "his company may have saved $100 million in drug-development work by merely licensing the FDA's discovery."

The FDA Reform Bill passed by Congress includes requirements to speed the review of new vaccines and drugs and to reauthorize the Prescription Drug User Fee Act. To accomplish this, the FDA will tap "user fees" that are charges to companies that submit products for FDA review and approval.

However, the FDA uses about $10 million a year (USA Today article) in industry fees to fund their research labs. The biotechnology industry protested the new drug discovered by the FDA. "It should stick to regulation and leave discovery to industry."

The taxpayer has essentially funded a private company's Research and Development. That company will have saved over $100 million. The taxpayer should be compensated and the FDA should deal only with regulation and not discovery. There are many other Federal research labs that are in the business of discovery.

The Dietary Supplement Health and Education Act of 1994

Having helped to write some of the key language for the Dietary Supplement Health and Education Act of 1994 with Senators Harkin and Hatch, I was very disappointed in the proposed statements of the President's Commission on Dietary Supplement Labels. The Commission issued a draft report for public comment before it made its final recommendations to the President, Congress, and the FDA.

The report was a disaster. It completely ignored the subject Congress created the Commission to address: namely, "how best to provide truthful and non-misleading information to consumers so that such consumers may make informed health care choices." Instead, the Commission simply placed its stamp of approval on the FDA's current prior restraint on all health claims, except those pre-approved and recommended the adoption of safety, reporting, and OTC botanical regulations that are beyond the scope of its delegated authority. The tragedy is compounded by the fact that the Commission's recommendations are required, by law, to be published by FDA as proposed rules, making it possible that the agency will adopt one or more of the suggestions.

Government Interference With Choice of Treatment by* Informed *Patients

I have been called upon many times by patient advocates to determine whether the treatment outcome desired by the patient's guardians or patient will equal or be better than the outcome of existing conventional treatment. Often times, these patients or their guardians find themselves entangled with legal issues because the physician wants to impose the conventional treatment indicating "it will save the patient's life," or "it will cure you." Or, the patient has received all conventional treatment without success and then wants to try an Investigation New Drug approved by the FDA for

research purposes. The patient attempts to obtain this drug but finds himself or herself ineligible according to the strict research criteria.

In these instances, when a patient has an unwanted treatment imposed upon him or her by a governmental agency, or when he or she desires an Investigational New Drug, I review the medical records to make a determination of the various *effective* treatment options, and whether any one option is superior to another, and importantly, whether a particular treatment option will increase life span. Remember that a critical measurement in cancer care is whether a specific treatment will increase the life span of the patient. Examples:

> You have already heard the eloquent and heart wrenching story of Zachary McConnell by Jonathan Emord, Esq. Zachary, age 5, had a rare brain tumor and was enrolled in a FDA approved Investigational New Drug protocol. While under this treatment the FDA decided to stop the protocol. His parents fought this decision legally.
>
> An eleven year old girl, EU, diagnosed with non-metastatic high-grade osteosarcoma of the left distal femur was treated with three cycles of appropriate chemotherapy with adequate doses for osteosarcoma. Her mother, with whom I spoke, wanted no further chemotherapy and wanted her daughter to proceed to surgery. Her physicians wanted her to have several more months of chemotherapy before the surgery. After reviewing all the data, including the patient's records and imaging scans, I was convinced from the published medical journal articles that the patient should proceed with surgery because no benefit in lifespan or local control is achieved when more than three cycles of chemotherapy is administered before surgery. Any delay in surgery brought a higher risk for distant metastasis. The judge in the case overturned the court order requiring the patient to have more chemotherapy.

TRUE FDA REFORM SHOULD INCLUDE

- *Easier Access to Drugs for terminally ill patients* and once a patient is enrolled in an FDA approved protocol, never stop that treatment.
- *Allow physicians to have access to "off label" information.*
- *Assign New Time Limit for FDA Review* of Applications, and limit FDA review of a new drug to 60 days if that drug has prior approval in United Kingdom or European Medicines Evaluation Agency.
- *Discontinue FDA's Discovery Research,* and Recover for the American taxpayer the $100 million saved by Neopharm, Inc., and other monies possibly gleaned in a similar fashion by other companies.
- *In the short term, hire additional staff to clear the backlog of products that await approval.*
- *Rescind FDA's regulatory authority within a single state.*

- *Answers to the Following:*
  1) How many applications per year are submitted for protocol approval (Investigational New Drug, New Drug Approval, etc)?
  2) How many of these are approved and in what period of time?
  3) How many potential applicants stop the process after attempting to complete the paperwork for the application.
  4) How many applications are submitted by "professional" application writers - attorneys, past FDA people, etc.
  5) To how many potential applicants does the hiring of these "professionals" present an impediment?
  6) What is the average cost of getting an application ready for submission to FDA?
  7) What is the average length of time needed to complete an application by the applicant?
  8) How effective is the FDA at helping potential applicants to complete the application if an applicant requests help from the FDA?
  9) How many patients request from the FDA an "off-label use" treatment or one that has an IND for which they may not be eligible?
  10) How many of those patients receive such treatment?
- *Informed Consent* could be a very powerful tool for the patient. If the concept of Informed Consent was truly enforced and fully explained, patients would then understand the limitations of many treatments.
- *Misinformation* is sometimes given to patients. A glaring example is found in a Sunday front page New York Times article on October 26, 1997 entitled 'Vitamin Mania, Millions Take a Gamble on Health.' Larry Norton, M.D., a staff person from Memorial Sloan Kettering, a highly regarded institution, was interviewed and stated:

> "Research at his institution showed that large doses of vitamin C could blunt the beneficial effects of chemotherapy for breast cancer. The research showed that breast cancer cells had large numbers of receptors, or docking places, for vitamin C, suggesting that the vitamin acted like a tonic for cancer cells.
>
> And a recent experiment showed that free radicals, chemicals that damage cells in ways that may lead to cancer, are also necessary for some of the mechanism that stop cancer once it gets going. So a substance like vitamin C, in large doses, could have unpredictable effects. It is also known that folic acid can negate the effects of methotrexate, a drug used to treat cancer."

This "information" is absolutely incorrect. Over 200 peer-reviewed scientific articles have been published in medical journals in the 1970s, 1980s, and 1990s. Summarized in books and medical journals, the correct information shows that nutritional modification, including the use of certain nutrients, and proper lifestyle can

dramatically decrease the morbidity and side effects of chemotherapy and radiation therapy as well as increase response rates. There have even been some reports that nutritional and lifestyle modification actually increase survival.

Simone, CB. Cancer and Nutrition (1992 revised Avery Publisher).
Simone, CB. Breast Health (1994 Avery Publisher)
Simone CB, Simone NL, Simone CB II. Oncology care augmented with nutritional and lifestyle modification. *J Ortho Mol Med.* 1997; 12(4):197-206.
Simone CB, Simone NL, Simone CB II. Folic acid does not interfere with methotrexate. *Lancet.* 1997; 350:1556.
Simone CB, Simone NL, Simone CB II. Lifestyle modification in oncology care. In: Prasad, KN ed. Cancer and Nutrition. 1997. Amsterdam, Netherlands, IOS Press.
Simone CB. Nutritional and Lifestyle Modification in Oncology Care. In: Torosian M. Integrated Cancer Management: Surgery, Medical Oncology, and Radiation Oncology. 1998. Marcel Dekkar, New York. (University of Pennsylvania)

Summary

The patient's well being must come first in all instances. Patients need to have access to treatments and information that potentially may benefit them. The FDA should serve the public and not be an obstruction.

Charles B. Simone, M.D.

Mr. BURTON. Thank you, Doctor. Dr. Moss. If you could hold the microphone as closely as possible, it would be helpful.

STATEMENT OF RALPH W. MOSS, Ph.D., JOURNALIST

Dr. MOSS. My name is Ralph Moss. I want to thank you, Mr. Chairman, and members of the committee, for allowing me to speak here today.

Mr. Chairman, Congress was deceived when the war on cancer was launched in 1971. Experts swore under oath that they would deliver a cure for cancer in time for the Bicentennial. Well, that's ancient history, but Congress continues to be fooled by a new generation of "experts" who testify that the war on cancer is being won, and that all we need to do is to trust them to conquer this terrible disease.

Recently, Richard Klausner, M.D., the Director of the National Cancer Institute, appeared before this Congress and claimed that we have turned the corner in the fight against cancer. He promised advances in genetics were ushering in a golden age of research. However, I believe that the rosy picture he paints is misleading, and I think that the statistics that Dr. Simone has shown you prove that.

There are many, many things that are wrong with the war on cancer. I have submitted nine pages of single-spaced testimony, and I'm just going to touch very briefly on some of the areas that I cover in that testimony.

For one thing, we know that about half of all cancers are still incurable by conventional methods, and the best that conventional medicine has to offer in those cases is palliation. So what are patients supposed to do?

The National Cancer Institute and the Food and Drug Administration—in fact, a whole industry—tries to get patients to enroll in clinical trials of chemotherapeutic agents, but we know from studies that there's very little chance of therapeutic benefit to patients in such trials. Studies in both the United States and Japan have shown that only about 1 percent of patients in Phase One clinical trials have a complete response to the treatment, and only about 5 percent have any response at all. You may think, "Well, 5 percent, that's not so bad," but a response is simply a shrinkage of a tumor for 1 month or more; a complete response is a complete shrinkage for 1 month or more. So you cannot correlate a complete response of cancer with a cure or significant increase in life. This is a kind of sleight of hand that's practiced all the time in the field of oncology. The doctor says "response" or "remission," and the patient hears the word "cure" in their head and thinks that they're going to get some extension of life.

In addition, there is great danger for patients in some of these clinical trials. There's one clinical trial I know of in which 42 percent of the participants were killed by the treatment itself, and this went unnoticed by the media or in public debate.

Another regimen called ICE, 13 patients, 8 percent of the total, died as a consequence of the treatment, so-called "treatment deaths." The scientists in charge had the nerve to conclude that this regimen was "well-tolerated with acceptable side effects and predictable organ toxicity." Acceptable to whom? Not to the pa-

tients who died after contracting raging bacterial infections, capillary leak syndrome, bleeding inside the brain, or irreversible kidney failure.

So we need alternatives, and there are alternatives. There are over 100, possibly 200, different treatments with some substantiation in the medical literature. On a recent trip to Germany, I was astonished to see the scope and freedom with which many progressive oncologists there treat cancer. They use a combination of the conventional approaches with such things as tumor vaccines; mistletoe therapy; local, regional, and whole-body hypothermia; thymus and other organ extracts; fever therapy; orthomolecular and antioxidant therapy; psychoneuroimmunology, and many, many other things. And their government not only allows such approaches, but encourages and pays for them as well.

We have a fiasco going on in this country, and that is the National Cancer Institute. The National Cancer Institute does some good things, of course, but also is in charge of disseminating information to the public on the nature of these nontoxic alternative treatments, and they issue statements on each of these treatments, and the statements are filled with error. They have not been peer-reviewed, and we don't know who has written these and we don't know what process they were created under. I served on the Advisory Council to the Office of Alternative Medicine, and at that time the OAM tried to find out simply who writes these things and what's the process by which they're vetted and made to be sure that these are accurate, and we never could find out.

And these statements, Mr. Chairman, must be immediately withdrawn, and new statements should be drawn up that are factual and unbiased. There is a good model for this: the University of Texas School of Public Health has posted such statements, excellent statements, on the Internet, which could be used.

But my big request to you, Mr. Chairman, and to the Congress and to the committee, is that you will focus your attention on something that happened last August, and it happened at the NIH, and was called POMES, P-O-M-E-S, which stands for the Practice Outcomes Monitoring and Evaluation Systems. This was a meeting of over 100 leaders of the cancer field, and there were heads of comprehensive cancer centers there and chairmen of departments at Memorial Sloan-Kettering, and the head of the American Health Foundation. I was proud and honored to be, myself, included in that group of 100. The National Cancer Institute paid for this meeting, and some of their representatives were there, although sadly, Dr. Klausner did not see fit to come.

And after days of heated debate, we arrived at guidelines by which alternative or complementary cancer treatments could be evaluated. People from orthodox medicine, people from nonconventional medicine were there, and it was, believe me, very heated. We needed mediators to come in and solve some of our problems, but we did hammer out certain guidelines, including an oversight board that could oversee the way in which these clinical trials of nontoxic treatments would be conducted.

This POMES process is being blocked by the National Cancer Institute. It's being bureaucratically stopped, and now we have gone through 6 more months, and we've seen another 270,000 people die

in this country from cancer, while they sit on their hands and refuse to do anything.

So I know that Dr. Klausner makes a nice presentation, and he came here and he asked for $2.2 billion, and Vice President Gore has said that he wants to increase the funding to $4.7 billion by the year 2003 simply for cancer research at the National Cancer Institute.

We, as citizens, have no power to make the FDA or the National Cancer Institute do anything; the power is with you, our elected representatives. My request, my urgent request, to you is that you block the appropriations to the National Cancer Institute until they agree to implement the POMES process. All we want is to have fair, impartial, unbiased evaluations done through the rigorous scientific method of the alternative treatments, and that will solve the problems that Mr. Waxman alluded to and that the other people have alluded to.

We want science, but we want the science to be applied to, and appropriately applied to, the methods that show the greatest promise for conquering cancer, which are, in fact, the alternative and nontoxic treatments. Thank you.

[The prepared statement of Dr. Moss follows:]

Ralph W. Moss, Congressional Testimony 2/4/98

TESTIMONY TO THE HOUSE COMMITTEE ON
GOVERNMENT REFORM AND OVERSIGHT
DAN BURTON (R-IN), CHAIRMAN
By Ralph W. Moss, Ph.D.

2/4/98

Mr. Chairman and Members of the Committee,

Congress was deceived when the war on cancer was launched in 1971. Experts swore under oath that they would deliver a cure for cancer in time for the Bicentennial (1976). That is ancient history. But Congress continues to be fooled by a new generation of "experts" who testify that the war on cancer is being won, and that all we need to do is trust them to conquer this terrible disease.

Recently, Dr. Richard Klausner, M.D., director of the National Cancer Institute (NCI), appeared before this Congress and claimed that we have turned the corner in the fight against cancer. He promised that advances in genetics were ushering in a golden age of research. However, I believe that the rosy picture he paints is misleading.

Back in 1962, 278,000 Americans died of cancer.

Last year, cancer deaths were over 560,000, double the figure of 35 years ago. Certainly, part of this increase is due to the growth and aging of the population. But even when one adjusts for these factors, the overall U.S. mortality rate from cancer increased over 10 percent from 1950 to 1991. And the incidence rate during that time increased nearly 50 percent.

There has been a leveling off in recent years. But we have still witnessed a tremendous worsening of the cancer situation throughout this century. In particular, the rates of lung cancer have risen astronomically, more than 500 percent among women. There has been a tripling in the incidence of melanoma, and nearly a doubling of cases of prostate cancer and multiple myeloma.1

BREAST CANCER STATISTICS

Many of us are understandably alarmed at the prevalence of breast cancer in America today. When Pres. Nixon launched the war on cancer in 1971, a woman's lifetime risk for contracting breast cancer was one in fourteen. Today, it is one in eight. Between 1973 and 1992, the incidence of breast cancer rapidly increased by 34 percent, and among black women by 47 percent. And the chances of being cured have not improved very much. Since 1960, nearly one million American women have died of breast cancer. Dr. Klausner has made much of the recent leveling off or even downturns in some of the cancer statistics. These are encouraging.

However, a slight downturn in mortality does not make up for millions of personal tragedies.

WHEN THE DIAGNOSIS IS CANCER

Let us consider what happens to a person who is diagnosed with cancer.

First of all, there are the so-called "proven" methods, surgery, radiation therapy and chemotherapy. Sometimes these are brutal methods, that involve the loss or damage of body parts and functions. Surgery is an ancient approach, known to the Egyptians, Greeks and Romans. It is a sad commentary that this is still the mainstay of therapy. New ideas are urgently needed in the treatment of even so-called "curable" cancers.

But what about those patients whose tumors are inoperable or widespread at the time they are discovered? Similarly, what about the patients whose tumors have returned after being "successfully" treated with "curative" therapies?

Such cancers are, by and large, incurable with today's conventional methods. The best that conventional medicine has to offer is palliation. And despite the war on cancer about half of all cancer patients will eventually find themselves in this deplorable position.

What are they supposed to do?

THE PITFALLS OF CLINICAL TRIALS

If you read the statements of the NCI, they urgently appeal to cancer patients to join their clinical trials. This message is picked up and amplified by all the beneficiaries of the war on cancer. You can even see it on billboards in airports. A "clinical trial" is made to sound very attractive to cancer patients. However, as the President's Commission for the Study of Ethical Problems in Medicine stated (in 1983), "Patients who are asked to participate in tests of new anticancer drugs" should "not be misled about the likelihood (or remoteness) of any therapeutic benefit they might derive."

In fact, there is little chance of therapeutic benefit to patients in such trials. Studies in both the United States and Japan have shown that only about one percent of patients in Phase I clinical trials have a complete response to the treatment, and only about 5 percent have any response at all.

You may think that five percent is not bad odds when you are in a desperate situation. But here you have to understand some of the peculiar terminology of the field. For a "response" is not a "cure." Far from it. The FDA defines a response as the shrinkage of 50 percent or more of the measurable tumors for a period of one month or more.

It is a change in size of a mass. This might be important, if the tumor is painfully pressing on a nerve or another vital structure. But usually such shrinkages are absolutely meaningless to the patient. It is essentially a numbers game played among oncologists - who can shrink tumors the most. In the majority of cases, these temporary shrinkages do not correlate with an increase in median overall survival, which is the most meaningful measurement of patient benefit in such

trials.

Sometimes, in fact, a high response rate actually correlates with a lower period of survival. It may do more harm than good.

"TREATMENT DEATHS"

I want to call your attention to the fact that these trials can be very dangerous for patients. The drugs approved by the FDA for treating cancer are all toxic. Some of them have astonishing toxicity, especially when given in combination. In one clinical trial of drugs on patients with the leukemia-like myelodysplastic syndrome, 42 percent of participants were killed by the treatment itself.

In another study, of a three-drug regimen called "ICE," 13 patients (8 percent of the total) died as a consequence of the treatment itself, so-called "treatment deaths." But the scientists in charge had the nerve to conclude that this regimen was "well tolerated, with acceptable...side effects and predictable organ toxicity."

Acceptable to whom? Not the patients who died after contracting raging bacterial infections, capillary leak syndrome, bleeding inside the brain, and irreversible kidney failure - all caused by these drugs. And certainly not their families.

This is the "scientific" approach of the NCI. Not surprisingly, there is tremendous resistance among patients and doctors to such trials. Only three to five percent of cancer patients go into them. Many oncologists want nothing to do with them. In fact, just 10 percent of all oncologists enroll 80 percent of the patients in clinical trials.4 In New York, oncologists have given their patients small doses of standard chemotherapy to make them ineligible for useless clinical trials.

LOOKING FOR ALTERNATIVES

Drugs that don't work, clinical trials that measure meaningless shrinkages, doctors who think that horrible side effects are perfectly acceptable...no wonder cancer patients today are desperately looking for alternatives. They are exploring the realm of unapproved, complementary, non-toxic treatments in record numbers.

You can be sure that one of the reasons the NCI and FDA so hate these alternative treatments is that they siphon away "adventurous" patients who might otherwise go into clinical trials.

Historically, all of the agencies involved in the war on cancer have lied about the nature of these alternatives. They have painted a distorted picture of them as quackery. They have pre-judged them, refusing to carry out the most basic tests that could evaluate their efficacy. Tests were only performed under duress (often because the Congress insisted) and these tests were at best ill-conceived and at worst marked by outright fraud.

Yet the history of medicine tells us that many treatments and techniques once considered

"alternative" or "fraudulent" later became an established part of the mainstream. Radiation and chemotherapy themselves started out on the fringe. Acupuncture was derided as "quackupuncture" for decades. But a recent Consensus Conference of the National Institutes of Health endorsed its use for such conditions as pain and nausea related to cancer. The Office of Alternative Medicine (OAM) was established by Congress at the National Institutes of Health precisely because of the historic failure of the NCI to fulfill its mission and examine all possible options in the fight against cancer. But little progress has been made because of the intransigent attitudes of the cancer establishment.

Are there frauds among the alternatives? Certainly. How can we separate the wheat from the chaff? We need good research, with open-minded attitudes and adequate funding, to carry out studies of these alternatives. The OAM is ready to perform these studies. But the NCI stands in the way. Along with its police partner, the FDA, it is the great roadblock to the examination of promising new ways of treating cancer.

GREAT PROMISE

Dr. Klausner is betting on the genetic revolution to produce a cure for cancer. Even some geneticists warn that cancer breakthroughs, if they do come from this field, may be decades away. I believe there is enormous potential in the various alternative and complementary approaches to cancer.

In my book, Cancer Therapy (1992), I discuss over 100 such methods. One could add another hundred or so of promise. These include vitamin and mineral regimens, herbal formulas, unusual drugs from land and sea, immunological techniques, electromagnetic treatments, and utilization of the mind-body connection.

On a recent trip to Germany I was astonished to see the scope and freedom with which many progressive oncologists treat cancer. They use a combination of the conventional approaches with such things as tumor vaccines; mistletoe therapy; local, regional and whole-body hyperthermia; thymus and other organ extracts; fever therapy; orthomolecular and antioxidant therapies; psychoneuroimmunology; music and art therapy; sports and physical therapy; and many, many others. Their government not only allows such approaches, but encourage and pay for them as well.

It is astonishing that the average American oncologist knows little or nothing about any of these approaches. The FDA has done everything in its power to block their development over here. The NCI has not seriously examined a single one of these. Our war on cancer has fallen woefully behind developments in other parts of the world, not just Germany but Japan, China, and many other countries as well.

The approach of the war on cancer has been relentlessly that of chemotherapy. Reliable estimates put the sales of cancer therapeutics at over $12.3 billion this year.5 Most of that is controlled by American firms. And so it has been a big business success story, with double-digit growth rates every year for over a decade. But it has done little for the cancer patient.

The FDA has approved approximately 40 drugs for the treatment of cancer. But it has never approved a non-toxic agent or one that was not patented by a major pharmaceutical company. The approved drugs are all toxic and many of them cause second cancers in those who are lucky enough to survive the treatment. And the NCI, FDA, and comprehensive cancer centers are tied by a thousand strings to the multi-billion dollar pharmaceutical industry. Recently, a top FDA official went to work for Elan Pharmaceuticals. But this is nothing new. Two past directors of the FDA became drug company officials, as did Dr. Klausner's predecessor at the NCI. It is a time-honored tradition, the "revolving door."

Meanwhile, the FDA spends a good deal of its resources hunting down and harassing those who use innovative methods in treating cancer.

They have carried out a vendetta against Dr. Stanislaw R. Burzynski, MD, PhD, a Texas physician who has used non-toxic peptides in the treatment of brain cancer and other kinds of malignancy. They have repeatedly raided his clinic, seized his records, harassed his patients. In 1995, they instigated charges that would have put him in federal prison for life. Luckily, the jury saw otherwise and Dr. Burzynski is a free man. When I publicly objected to this harassment I myself was slapped with a subpoena for all my information regarding Dr. Burzynski. When I pointed out the illegality of this request, and indicated my willingness to fight the FDA, the subpoena was just as suddenly quashed by the U.S. Attorney.

FDA has also impeded the work of Dr. Georg Springer of the Finch Medical School, who has developed a promising vaccine for breast cancer. It has hindered the work of Arnold Eggers, M.D., of Downstate Medical School, who has a promising treatment based on concepts first proposed by William B. Coley a century ago. And it has used its resources to attack the distributors of non-toxic medications. The most recent victim was a distributor of the non-toxic drug hydrazine sulfate, who was raided by FDA enforcement agents on January 16, 1998.

The approach of the NCI and FDA is overwhelmingly in support of toxic chemotherapy. They have abrogated their duties as the defenders and protectors of the cancer patients. They function today on behalf of the industry they were supposed to challenge and oversee. They are the drug testing and law enforcement arms of a vast $100 billion a year business, the cancer industry.

C.I.S. FIASCO

The promotion of toxic treatments and the venomous hatred of alternatives is not restricted to court battles. Both FDA and NCI are active in the court of public opinion, trying to destroy confidence in any non-toxic or less-toxic treatment.

Their main vehicle in this regard is the Cancer Information Service of the NCI. Their reckless attacks on alternative and complementary treatments are disseminated at taxpayer's expense via print, fax, and especially the Internet.

Their statements are filled with prejudice, errors and innuendo. Each one contains an "advertisement" for NCI's clinical trials. When I was an advisor to the Office of Alternative

Medicine, I tried to find out exactly who wrote these erroneous statements and what sort of "peer review" they possibly could have undergone before being released. I never could find out. It is clear that no bona fide experts were involved in their creation, and that the proponents of such methods were not consulted or even interviewed before these statements were drawn up and released.

These harmful, hateful statements have become an integral part of the "war on cancer" which, quite frankly, more often looks like a "war on alternative practitioners" than a war on any disease. Treatment approaches that threaten the hegemony of the drug industry are prone to vicious attack.

The NCI's statements on alternative and complementary cancer treatments should be immediately withdrawn. New statements that are factual and unbiased, should be drawn up for release by the Cancer Information Service.

The statements that have already been prepared by Dr. Mary Ann Richardson and her group at the University of Texas School of Public Health could provide a good starting point for these new statements.

REFORM OF FDA

In addition, the FDA should be reformed so that it no longer exerts a stranglehold on innovators in cancer treatment and diagnosis. That is why I strongly support passage of the Access to Medical Treatment Act and urge you all to cosponsor this important legislation.

The FDA does little to protect citizens from the ravages of chemotherapy, which is overwhelmingly given without any proof of patient benefit. In the past, FDA at least paid lip service to the idea that anticancer drugs should extend life or improve quality of life. But in 1996, they caved in and agreed that new drugs could be approved based on partial remissions in clinical trials. Such partial remissions are nothing but the shrinkages of tumors. As we have shown, such temporary and partial shrinkages do not necessarily lead to improvements in survival or quality of life.6

POMES

Finally, Mr. Chairman and members of the committee, I have an urgent request.

In August, 1997, the Office of Alternative Medicine (OAM) in conjunction with the National Cancer Institute (NCI) convened a meeting in Bethesda, MD to consider how they could evaluate the practices of doctors who use unconventional methods to treat cancer. The name of this meeting was POMES, which stands for "Practice Outcomes Monitoring and Evaluation Systems." Over 100 leaders of the cancer field attended, including not just alternative researchers and practitioners, but the director of the Comprehensive Cancer Center of the University of Wisconsin, the president of the American Health Foundation, two department chairs from Memorial Sloan-Kettering Cancer Center, representatives from major food companies, and many

others.

There were great hopes for this meeting, since we were told that it was funded by Dr. Klausner's office at the NCI. Perhaps this signaled a change in attitude at NCI, the change we have all been waiting for. But not only was Dr. Klausner unable to attend, but his key deputy, Robert Wittes, M.D., Director of the Division of Cancer Treatment, Diagnosis and Centers, also failed to put in an anticipated appearance. The FDA and NCI scientists who did appear lacked decision-making power in this area.

After several days of heated discussion, the participants finally hammered out statements that could lay the basis for future evaluations of alternative cancer treatments. It felt like history in the making. These guidelines called for the creation of an Oversight Board, a body of experienced people who could guarantee a "level playing field" in the evaluation of alternative practices. No longer would NCI have complete power to serve as lawyer, judge and jury in every case.

Most of the participants left that meeting excited by the prospects before us. Then, silence. Since August, we have not received a single official communication regarding POMES. Has POMES died a natural death...or did someone kill it?

I know for a fact that the problem does not lie with the Office of Alternative Medicine, whose leaders remain enthusiastic about the prospect of fairly evaluating such treatments. I can only conclude, therefore, that the roadblock is the top leadership of the NCI and possibly the NIH as well.

You have to ask yourself why these high-placed medical leaders so fear an impartial test of unconventional approaches to cancer? Why do they hate the idea of an impartial Oversight Board, which could detect fraud or malfeasance on either side of the cancer controversy?

Perhaps they are afraid of the competitive threat such non-toxic and less-toxic methods might pose to the cancer industry? Do they fear the ridicule of prejudiced colleagues? Or perhaps they fear the repercussions in Congress, if it turns out that an effective treatment for cancer was overlooked - or even suppressed - by NCI and FDA?

Mr. Chairman, I urgently appeal to you to help revive POMES.

I am sure you agree that patients and their caregivers need reliable information about the safety and potential effectiveness of alternative and complementary cancer treatments.

Many American citizens are impatient with the foot-dragging at NCI and the obstructionism of the FDA. Yet we as individual citizens have no way to force these agencies and individuals to act properly or fairly. It is up to you, our elected representatives, to do that. There is no time to waste. Since August, another 270,000 Americans have died of cancer. Many of them were desperately seeking reliable scientific information on alternatives at the time they died.

The Congress created the OAM to bring about the fair evaluation of alternative methods. We

appreciate the fact that you have increased OAM's funding to $20 million this year. It is a heartening vote of confidence in the future of this field. And, in some respects, under the leadership of Wayne Jonas, M.D., it has done a brilliant job. But OAM by itself does not have the political clout to force the testing of alternative cancer treatments. That is the main reason that OAM has not carried out a single evaluation of a controversial cancer treatment. It has not and it will not, because at every turn, the NCI has been there, insisting on a major role. It now turns out that the role NCI wanted was to block and obstruct such trials from taking place.

BLOCK NCI'S APPROPRIATIONS!

Just one month ago, Dr. Klausner appeared before the Appropriations Committee and requested $2.2 billion for his agency for fiscal year 1998. This is an increase of $61 million over last year. I am here to ask you to do everything in your power to block that appropriation until NCI changes its attitude towards alternative and complementary treatments. As a first step they should actively implement the POMES process.

In his speech to Congress, Dr. Klausner stated that "there is no one intervention or even one type of intervention that will successfully conquer the many diseases we call cancer. Our approach must be open and broad-based."

Fine words! But it happens to be the exact opposite of the course that NCI is actually pursuing. It is only an aroused Congress that can make Drs. Klausner and Wittes open the doors of NCI to alternative treatments. They must not be allowed to serve as a branch of the pharmaceutical industry, but must be convinced to test a wide variety of treatments, as they are currently practiced around the world. If these individuals will not comply, they should be replaced by open-minded scientists who will.

Mr. Chairman, for the 1.2 million Americans and the 9 million people worldwide who will develop cancer this year, such reforms cannot come a moment too soon.

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1 Ries, LAG, et al. SEER cancer statistics review. 1973-1991, Tables and Graphs, Bethesda, NCI, 1994.
2 J Clin Oncol 1996;14:287.
3 J Clin Oncol 1995;13:323
4 J Clin Oncol 1994;12:1796
5 Frost & Sullivan, World cancer therapeutics markets [executive summary]. Mountain View, CA: Frost & Sullivan, 1993. Cited in Moss, Questioning Chemotherapy, p.75.
6 Stout, H and McGinley L. Cancer drugs to get speedier FDA review. The Wall Street Journal, March 29, 1996.

Mr. BURTON. I will tell you right now that we will talk to, and probably have before the committee, the people who are sitting on that proposal over at National Cancer Institute.

Dr. MOSS. Thank you.

Mr. BURTON. I've already instructed our staff to have them appear before the committee, and we will do that. I will ask them why they are sitting on their hands.

Dr. MOSS. Thank you very much.

Mr. BURTON. If they don't give us a satisfactory answer, I'll sic you on them. [Laughter.]

I'm not being flippant. We will look into it.

Mr. Eggers.

STATEMENT OF ARNOLD E. EGGERS, M.D., CANCER RESEARCHER

Dr. EGGERS. Yes, thank you. I have worked for the past 25 years, with some interruptions, on a study of tumor immunology with a view to developing a vaccine treatment for cancer. I began this project as a medical student at Columbia University, took residency training at a New York Hospital, which is Cornell University, and then at the Hospital of the University of Pennsylvania, went to NIH in the National Cancer Institute for 3 years, came back to Columbia to finish residency, and have worked as an attending, first at Columbia and now at SUNY-Health Science Center at Brooklyn.

The purpose of describing my training is to emphasize that I have good academic credentials and that this is my life work. It is a long-term commitment to one idea: reproducing the spontaneous remissions which are sometimes seen in cancer patients following bacterial infections. My vaccine is a kind of nontoxic immune stimulation, a kind of alternative medicine, if you like. The final version of the vaccine treatment has produced good results. There's a CT scan of a response of a brain tumor patient included in the paperwork I submitted, which shows a brain cancer shrinking from a large ring-shaped lesion down to a small dot. Three out of four brain cancer patients who received the final version of the treatment went into remission. Out of 180 injections of this and previous versions of the vaccine, there was only 1 patient with a side effect, 1 case of an allergic reaction, which was not fatal.

Now the man on the street might say that this looks like a promising, new treatment which should be supported by our Government in the war on cancer, but the man in the street does not know how the system works. Having already received approval from the FDA in 1989 to treat brain cancer patients, I applied in 1994 for permission to treat other kinds of solid tumors as well, and was put on clinical hold. It is now almost 4 years later; the clinical hold is still in effect, and we are still dialoging.

Most of this time has been spent discussing technical minutiae.

Mr. BURTON. Excuse me. I missed it—how long did you say, Doctor, that this has been going on?

Dr. EGGERS. Almost 4 years now, 4 years in April.

Mr. BURTON. And you're dealing with——

Dr. EGGERS. The FDA.

Mr. BURTON. The FDA.

Dr. EGGERS. Yes.

Mr. BURTON. Can you give this committee—I hate to interrupt you—can you give this committee the people that you have been contacting over there who have not been responding? We'd like to have that.

Dr. EGGERS. Yes, I can supply that. I will.

Mr. BURTON. Thank you.

Dr. EGGERS. The FDA inspected my records and issued a warning letter which said, quote, "Deviations in the conduct of this study appear to be the result of your lack of understanding of the procedures and requirements that govern the use of investigational new drugs." It is important to emphasize that their citations were, in general, appropriate and correct from their point of view. They found valid deviations from their rules.

Just to give one example, they cited me for incorrect patient consent forms. It turns out there are 17 elements of informed consent which require one-and-a-half pages of small print just to list. My consent forms, although approved by the local hospital ethics committee, were equivalent to a violation of statutory law. As you know, the FDA has sent at least 16 people to Federal penitentiary in the last 10 years for violations of their rules, which have the power of statutory law. I appealed to an ombudsperson at the FDA. She told me that if I wanted to have any hope of meeting regulatory requirements, I needed to hire a professional FDA consultant, one of the people drug companies hire to interface with the FDA. This is way beyond my means financially.

In all of this, no one has acted with malice. On one side, you have a university scientist approaching the regulatory process with good with, and on the other side, professional bureaucrats approaching their jobs with good will. Yet, between us, we could not make the system work in 4 years.

The man in the street might want to know what went wrong? But I think the problem lies with the system, and not with individual bureaucrats, who are only doing their job, and in most cases doing it well.

As everyone knows, the FDA was established in its current form by the Kefauver-Harris Drug Amendment in 1962, as a response to the thalidomide tragedy in Europe in which 2,000 to 3,000 mothers who took this particular sleeping pill gave birth to children with serious birth defects. An FDA employee, Frances Kelsey, became a national hero by blocking legal entry of the drug into the United States. It is important to note that the drug was a sleeping pill, and no one dies from insomnia. The mistake in thinking behind Kefauver-Harris is that it fails to distinguish between fatal and nonfatal diseases. In the case of nonfatal diseases like insomnia or acne, you want to protect people from unnecessary side effects. This was what thalidomide was all about.

In the case of fatal diseases like cancer, the situation is more complicated. In deciding if Government regulation is worthwhile, you have to compare the number of people who die from toxic side effects of inadequately screened new medicines against the number of people who die waiting for the release of successful new medicines. The bureaucratic process saves on the one hand by screaming for toxic, but takes lives on the other hand by delaying access to treatment.

Cancer kills 500,000 people a year in the United States. A 1-year bureaucratic delay in releasing a cure for cancer would necessarily kill 500,000 people. These are the people who would still be alive if the Government hadn't blocked their access to treatment in their lifetime.

These days, toxic side effects of drugs are quickly discovered and publicized or extremely rare, and it is inconceivable that 500,000 people could be killed by a dangerous new treatment before the alarm was called. I believe this arithmetic or statistic argument shows the error of the current system, which guarantees that there will be a vast, unnecessary loss of life if ever cancer is cured.

I had submitted suggestions about how to change the current system, which are actually very parallel to Congressman Bedell's thoughts, and I fully support his ideas.

Thank you.

[The prepared statement of Dr. Eggers follows:]

Mr. Chairman and Members of the Committee:

I have worked for the past twenty-five years, with some interruptions, on a study of tumor immunology with a view to developing a vaccine treatment for cancer.

I began this project as a medical student at Columbia University, took residency training at New York Hospital, which is Cornell University, and at the Hospital of the University of Pennsylvania, went to NIH in the National Cancer institute for three years, came back to Columbia to finish residency, and have worked as an attending first at Columbia and now at SUNY-Health Science Center at Brooklyn.

The purpose of describing my training is to emphasize that I have good academic credentials and that this is my life's work. It is a long-term commitment to one idea: reproducing the spontaneous remissions which are sometimes seen in cancer patients following bacterial infections. My vaccine is a kind of non-toxic immune stimulation. The final version of the vaccine treatment produced the results which you see before you. Three out of four brain cancer patients who received the final version of the treatment went into remission.

Out of 180 injections of this and previous versions of the vaccine there was only patient with aside-effect--one case of an allergic reaction which was not fatal. Now the man in the street might say that this looks like a promising new treatment which should be supported by our government in the war on cancer, but the man in the street does not know how the system works. Having already received approval from the FDA in 1989 to treat brain cancer patients, I applied in 1994 for permission to treat other kinds of solid tumors as well and was put on clinical hold. It is now almost four years later, the clinical hold is still in effect, and we are still dialoguing. Most of this time has been spent discussing technical minutiae. The FDA inspected my records and issued a warning letter which said "deviations in the conduct of this study appear to be the result of your lack of understanding of the procedures and requirements that govern the use of investigational new drugs."

It is important to emphasize that their citations were in general appropriate and correct from their point of view. They found valid deviations from the rules. Just to give one example, they cited me for incorrect patient consent forms. It turns out there are seventeen elements of informed consent which require one and a half pages of small print just to list. My consent forms, although approved by the local hospital ethics committee, constituted a violation of statutory law. As you know, the FDA has sent at least sixteen people to federal penitentiary in the last ten years for violations of their rules, which have the power of statutory law. I appealed to an ombudsperson at the FDA. She told me that if I wanted to have any hope of meeting regulatory requirements, I needed to hire a professional FDA consultant, one of the people drug companies hire to interface with the FDA. This is way beyond my means financially.

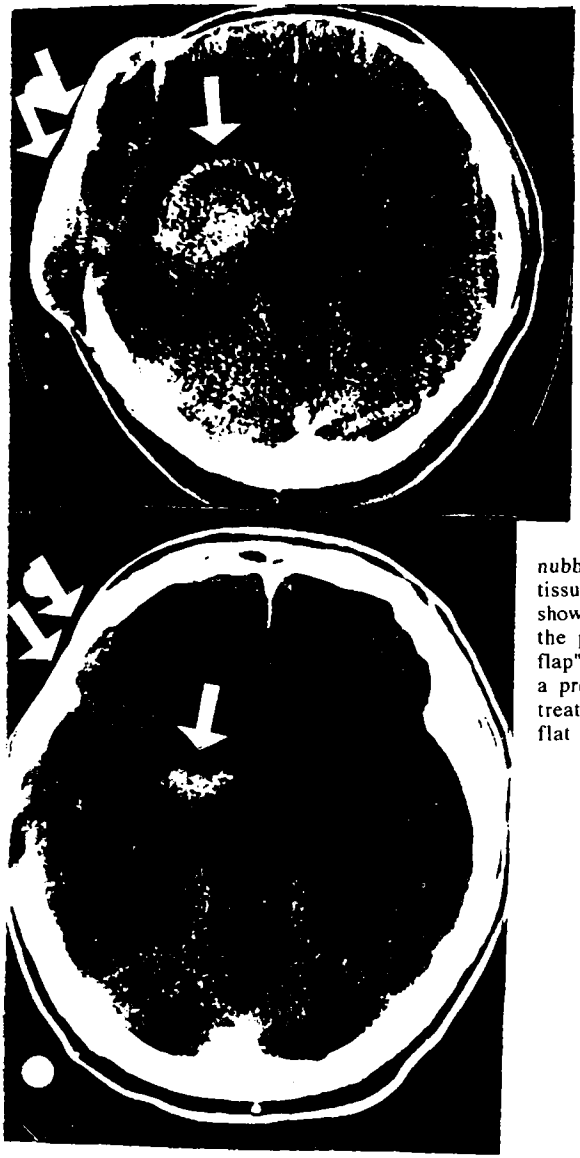
In all of this, no one has acted with malice. On one side you have a university scientist approaching the regulatory process with good will and on the other side professional bureaucrats approaching their jobs with goodwill. Yet between us we could not make the system work in four

years. The man in the street might want to know what went wrong. I think the problem lies with the system and not with individual bureaucrats, who are only doing their job, and in most cases doing it well.

As everyone knows, the FDA was established in its current form by the Kefauver-Harris Drug Amendment in 1962 as a response to the thalidomide tragedy in Europe, in which 2000-3000 mothers who took this particular sleeping pill gave birth to children with serious birth defects. An FDA employee, Frances Kelsey, became a national hero by blocking legal entry of the drug into the US. It is important to note that the drug was a sleeping pill, and no one dies from insomnia. The mistake in thinking behind Kefauver-Harris is that it fails to distinguish between fatal and non-fatal diseases. In the case of non-fatal diseases like insomnia or acne, you want to protect people from unnecessary side-effects.

This was what thalidomide was about. In the case of fatal diseases like cancer, the situation is more complicated. In deciding if government regulation is worthwhile, you have to compare the number of people who die from toxic side-effects of inadequately-screened new medicines with the number of people who die waiting for the release of successful new medicines. The bureaucratic process saves lives on the one hand by screening for toxicity but takes lives on the other hand by delaying access to treatment.

Cancer kills 500,000 people a year in the U.S. A one-year bureaucratic delay in releasing a cure for cancer would necessarily kill 500,000 people. These are the people who would still be alive if the government hadn't blocked their access to treatment in their lifetime. These days, toxic side-effects of drugs are quickly discovered and publicized, or else extremely rare, and it is inconceivable that 500,000 people could be killed by a dangerous new treatment before the alarm was called.I believe this arithmetic or statistical argument shows the error of the current system, which guarantees that there will be a vast unnecessary loss of life if ever cancer is cured.



RESPONSE OF BRAIN CANCER PATIENT TO IMMUNE STIMULANT VACCINE

The top CT scan of the brain is pre-treatment and the bottom scan is post-treatment.

The single arrows show the tumor itself, which collapses from a large irregularly-shaped ring into a small nubbin of residual scar tissue. The double arrows show a section of the skull, the post-operative "bone flap", which is elevated from a pressure effect before treatment but goes down flat after treatment.

Intralymphatic immunotherapy of glioblastoma

ARNOLD E. EGGERS, MD; JOHN I. MILLER, MD; SALVATORE SCLAFANI, MD

Patients with solid tumors have been reported to undergo remission with immunotherapy, either after treatment with autologous tumor cell vaccine or after administration of lymphokines.[1] Another example of immune rejection of tumors occurs in renal transplant recipients bearing non-major histocompatibility complex-matched tumors. Cessation of immunosuppression leads to immune regression of the tumor in the majority of these patients.[2] In the case of glioblastoma, an extract of *Serratia marcescens* was found to induce remission in 3 of 19 patients with recurrent tumors.[3] This report describes a patient with recurrent glioblastoma who underwent more or less complete remission of a brain tumor after immunotherapy; however, spinal cord metastasis developed.

CASE REPORT

A 46-year-old man was admitted to University Hospital of Brooklyn for evaluation of recurrent glioblastoma. He had undergone undergone subtotal removal of a right temporal lobe tumor four and a half months previously. Because of early recurrence at the end of radiotherapy (5,000 cGy), he underwent a second subtotal removal 60 days after the first operation. Pathologic findings confirmed the presence of recurrent glioblastoma. He was given an additional 1,000 cGy, and four weeks after completion of the second course of radiotherapy or 139 days after the first operation, he was started on immunotherapy, at which time he had a second relapse confirmed by clinical and computed tomography (CT) criteria.

Informed consent was obtained, and all procedures were approved by the hospital's Institutional Review Board. The patient received three treatments (days 0, 14, and 42; average dose of 6.5×10^6 cells) of vaccine prepared from autologous tumor cells grown in tissue culture, inactivated with mitomycin, and linked sequentially to a glycylglycylcystamide spacer and the adjuvant muramyl dipeptide, as according to the methods of Eggers et al.[4] The vaccine was administered intralymphatically via dorsal pedal lymphatics, as described previously by Wiseman et al.[5] The patient's only other medication was phenytoin. He was not on steroids.

Within two weeks of starting treatment, the patient became more alert, and the bulging free bone plate covering his craniotomy site began to recede into place. A CT scan taken at nine weeks showed significant decrease in the size of the tumor: the ring-enhancing lesion having collapsed into a small nubbin of residual contrast-enhancement at the site of surgical debulking on the medial aspect of the middle fossa (Fig 1). A repeat scan at 14 weeks showed no change in this pattern, with the patient remaining alert and interactive. No side effects of treatment were seen.

During the course of immunotherapy, progressive spinal cord compression developed. A magnetic resonance imaging (MRI) scan of the spinal cord showed a very faintly gadolinium-enhancing tumor extending from C6 to T2 (Fig 1). Surgical exploration disclosed an intramedullary tumor with a large exophytic component, which was debulked; pathologic assessment revealed it to be glioblastoma. This was interpreted as a metastasis, although a second primary tumor could not be ruled out. The patient succumbed to pulmonary complications of the progressively growing spinal cord tumor shortly after the 14-week scan.

DISCUSSION

Immunotherapy of glioblastoma has been attempted previously in numerous cases with limited success, as reviewed by Couldwell et al.[6] Bloom et al[7] described a randomized controlled study with irradiated autologous tumor cells injected subcutaneously, which led to negative results. There are apparently no reports of glioblastoma regression induced by active specific immunization.

The approach to immunotherapy illustrated in this case report is based on work in a murine fibrosarcoma model, in which animals were immunized against syngeneic tumor cells linked covalently through a spacer molecule to the adjuvant muramyl dipeptide, a simplified form of the monomer of the mycobacterial cell wall.[8] Cytotoxic lymphocytes were detected with a short-term, chromium release assay, and the growth of small tumors could be slowed or reversed with immunotherapy. Preliminary data from human glioblastoma patients innoculated subcutaneously with a similar vaccine have demonstrated the ability to immunize patients against tumor-associated antigens, as detected with a short-term, chromium-release assay performed with peripheral blood lymphocytes and autologous tumor cell targets, but no definite therapeutic effect.[2] Cytotoxic lymphocytes appear to have T-cell markers and require major histocompatibility complex (MHC) matching at the sensitization but not effector stage of immunity. The presence of T-cell markers does not prove that cytolysis occurs via the T-cell receptor. This kind of adjuvant-induced immunity resembles the MHC-unrestricted "promiscuous" killing by T cells described by other workers.[9] No toxicities have been associated with treatment except occasional urticaria around the injection site, which has been treated successfully with diphenhydramine. The putative advantage of intralymphatic versus subcutaneous administration of vaccine is probably a dose effect because injection into dorsal pedal lymphatics directly accesses the largest lymph node chain in the body. Intralymphatic injection of tumor vaccine has been reported to induce occasional remission of solid tumors outside of the central nervous system.[3]

An important issue in this case is whether the observed partial regression (ie, the regression of the brain tumor only) can be explained as a delayed effect of radiotherapy of the brain. Graeb et al[10] reported that three out of 47 glioma patients undergoing radiotherapy experienced transient CT worsening on CT scan followed by gradual improvement occurring over a period of

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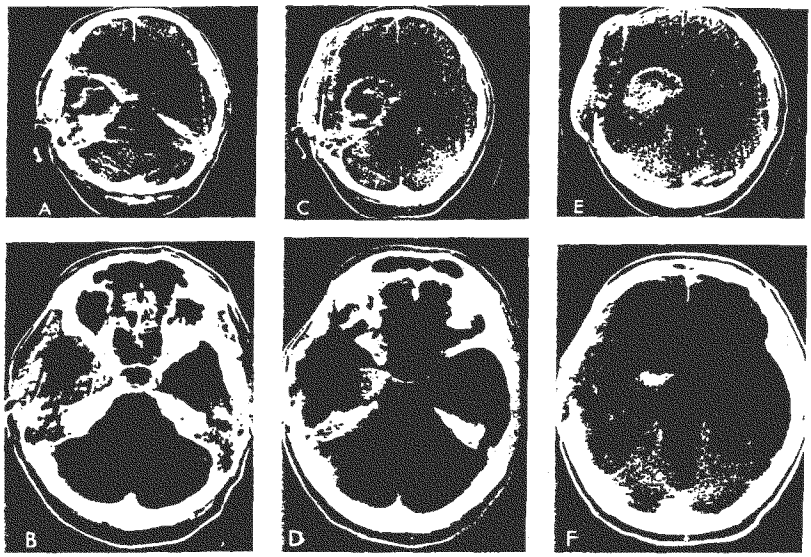

FIGURE 1 CT scans of brain tumor. Sections of scan taken four weeks after completion of second course of radiotherapy (1,000 cGy), at the time of starting immunotherapy (A,C,E). Corresponding sections of scan taken nine weeks later (B,D,F).

seven to 18 months. This kind of delayed radiation effect cannot be ruled out in this case, but it seems unlikely because of surgical documentation of early tumor recurrence, which implied radioresistance, and because of the rapidity of clinical improvement after the initiation of immunotherapy. On the other hand, it cannot be stated on the basis of one case that this is an efficacious treatment for glioblastoma, and further studies are warranted before any claims can be made in this regard.

An hypothetical alternative explanation for the mixed clinical response in this case may relate to the concept of "immunologic privilege." The central nervous system is said to be an "immunologically privileged site," a place where graft or tumor rejection is impeded, where lymphatic drainage is poor, and where lymphocytes do not normally circulate.[11,12] Lymphocyte access is thought to be regulated by the blood-brain barrier, which is maintained by tight junctions between endothelial cells, as well as by special physiologic properties of the endothelial cells and surrounding pericytes and astrocytic end-feet.[12,13] In this patient, the primary tumor, which showed marked contrast enhancement on neuroimaging, signifying breakdown of the blood-brain barrier, regressed simultaneously with growth of a spinal cord metastasis, which retained an intact blood-brain barrier. One can hypothesize that breakdown of the blood-brain barrier on neuroimaging correlates with improved lymphocyte access, although the latter may require more than just opening of tight junctions. Why endothelial cells in different parts of the central nervous system should have reacted differently to the same tumor is unknown. The alternative explanation that the spinal tumor was a second primary with different tumor-specific antigens that were not expressed on vaccine cells prepared from the first tumor is unlikely because immunity in this system has been shown to have little specificity, at least as measured by in vitro assays.[7]

Another noteworthy feature of this case was the spinal cord lesion. Clinically apparent spinal cord metastasis is a rare complication of glioblastoma[14]—tumor spread via cerebrospinal fluid being more characteristic of medulloblastoma and pinealoma.[15] Clinical spinal cord involvement has been reported in four out of 85 patients with intracranial glioblastoma, all of these cases having involved a primary tumor in the posterior fossa.[14] However, it can be argued that this patient outlived the natural history of his disease and that if immunotherapy had not been effective against the primary tumor, the spinal cord lesion would not have had time to develop.

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Mr. BURTON. Thank you very much, Doctor. And I apologize to Dr. Simone and you, Doctor, because they've got "Mr." up there, and I don't know how that happened. We'll have to shoot somebody on the staff. [Laughter.]

Are you a doctor or are you not a doctor, Mr. Moore?

Mr. MOORE. No, health policy is my field.

Mr. BURTON. OK. Well, then I have you correct. Mr. Moore, you're recognized.

STATEMENT OF THOMAS J. MOORE, SENIOR FELLOW, HEALTH POLICY INSTITUTE, GEORGE WASHINGTON UNIVERSITY

Mr. MOORE. Mr. Chairman, the basic issue today is whether consumers, especially those with serious or life-threatening illnesses, ought to have the right to any drug or alternative medicine, even though that drug had not been proven safe and effective, and had been approved by the Food and Drug Administration.

I'd like to tell you a story of what might happen if that should be the case. In this age of media hype, it's quite plausible that literally millions of Americans could be persuaded to take a pill every day that they hoped would prevent cancer, especially if it included a natural substance or a vitamin. Suppose that long after millions of people were popping this cancer-prevention pill the proper, extensive, expensive, randomized clinical trials were finally conducted to see if the hoped-for benefits in fact existed. Now suppose that those clinical trials showed that these anti-cancer pills either didn't work at all or they actually caused lung cancer. Millions of Americans would be spending their hard-earned money on a remedy that at best was ineffective and at worst might give them cancer.

Am I telling you a fanciful, alarmist story? This is a true story. The natural chemical was the beta carotene supplement, and like so many new ideas, it sounded promising, but proved to be worthless or harmful when actually tested.

We have dreamed of having powerful medicines since the dawn of human history. But the sad history teaches us that, for most of the last 7,000 years, most of the drugs were hazardous, poisonous, or at best, merely unpleasant. Real progress began only when we began to use randomized clinical trials to separate the beneficial drugs from those that were ineffective and harmful. Our current regulatory scheme, our current law, and the FDA are built on that vital principle.

This morning you have heard some dramatic stories from individuals who believe they were greatly helped, perhaps even saved, by a treatment that is not available in the United States. The question is, therefore, should Americans have access to a medical treatment if there are individuals who can personally testify that it is valuable?

The most simple test case might be a remedy for obesity. Here seems to be a treatment that every consumer can judge. You either lose weight or you don't. Suppose that for our test case the FDA had approved these drugs. So they had been subject to at least modest levels of safety testing. Should not then the consumer, rather than Government regulators or health authorities, be the judge of this treatment? You all ought to know the answer to this ques-

tion because this episode also happened. It is popularly called the fen-phen debacle, and the result may turn out to be one of the greatest drug disasters our Nation has ever experienced.

Last September, the diet drugs Pondimin and Redux were hastily withdrawn after the FDA received evidence that an astonishing 31 percent of the people tested showed some evidence of damage to their heart valves. Did the consumers notice? Could they judge for themselves? They could not. Until it became very severe, the heart damage had no symptoms. Did their doctors notice? They did not. Pondimin was on the market for more than 20 years before two alert medical workers in North Dakota spotted something suspicious.

What is the lesson, what is the first lesson of this drug debacle? It not only takes systematic, scientific testing, but continuing surveillance to discover serious adverse effects that may already, as we speak, be harming millions of people. This is exactly why society and this Congress has erected the safeguards that are now being examined in this hearing.

Another aspect of this issue is much more difficult. Should people with an advanced cancer or Parkinson's disease have the right to any treatment they choose? Some of these people might not live long enough for the kind of drug testing that I believe is so important to protecting the public. Should not they be entitled to take any risks they choose? On the surface, I believe the case for individual liberty seems compelling. However, another example will illustrate the dark problems underlying this seemingly straightforward idea.

Suppose you are dying of cancer, and I offer you this ghoulish shell game. In one of my hands, I have hidden a treatment that might save your life. In the other hand is a quack treatment that's probably going to make you so sick you can't get out of bed; it will actually shorten your remaining days of life. And I'm giving you a free choice here; pick which hand you want; go ahead. Pick. Do some research. Check me out and see which hand you would like to choose. This is not a meaningful choice. Without extensive drug testing, we just can't tell which hand holds the dangerous poison and which conceals the life-saving drug.

I want people to have choices, too, but they should be real choices involving scientific data about how much harm and how much good various treatment alternatives can be expected to achieve. We really have only one solution to the problem that is of concern to people who have testified today. We need policies that will promote and pay for more drug testing, not new loopholes that would endanger the safety of millions of people. I agree with the testimony that there are important alternative therapies that are falling through the cracks of our system as we have designed it today, but I don't think the answer is to repeal the safeguards that are so important to public health.

[The prepared statement of Mr. Moore follows:]

Prepared Statement of Thomas J. Moore

Mr. Chairman, members of the committee.

The key issue today is an important one. Should consumers, especially those with a serious or life threatening illness, have the right to any drug or alternative medicine even though it has not been proven safe and effective and approved by the Food and Drug Administration?

Let me tell a story of what could happen if that were the case. In this age of media hype, it is plausible that literally millions of Americans could be persuaded to take a pill every day that they hoped would prevent cancer–especially if it included some natural ingredient or a vitamin.

Suppose that long after millions of people were popping this cancer prevention pill, proper, expensive randomized clinical trials were finally conducted to see if the hoped for benefits in fact existed.

Now suppose that those clinical trials–the only real scientific evidence we have whether drugs work or not–showed that these anti-cancer pills either didn't work at all–or actually caused lung cancer. Millions of Americans would be spending their hard earned money on a remedy that at best was ineffective–and at worst could give them cancer.

Am I telling you a fanciful, alarmist story? This is a true story. It already happened–and the treatment involved was beta-carotene supplement. Like so many new drug treatments, it sounded promising but proved to be worthless or harmful when tested.[1 2]

Humans have dreamed of powerful medicines since the dawn of history. But for most of the last seven thousand years consumers were mostly victims of hazardous, poisonous, or merely unpleasant drugs. The era of modern beneficial drugs began only a few decades ago when society

began to insist that drugs be tested for safety and efficacy in well controlled clinical investigations. Real progress began only when we used randomized clinical trials to separate beneficial drugs from those that were worthless or harmful.

This morning you have heard some dramatic stories from individuals who believe they were greatly helped–perhaps saved--by a treatment that is not available in the United States. The question therefore is should Americans have access to a medical treatment if there are individuals who can personally testify that it is valuable?

The most simple test case would be a remedy for obesity. Here seems to be a treatment every consumer can judge. Either you lose weight or you don't. Suppose for our test case that the FDA had approved the drugs–so they had been subject to at least modest levels of safety testing. Should not then the consumer–and not government regulators or health authorities–be the judge this treatment?

You all ought to know the answer to this question. This episode also happened, and the result may turn out to be one of the greatest drug disasters that our nation has experienced. Last September the diet drugs Pondimin and Redux were hastily withdrawn after the FDA received evidence that an astonishing 31 percent of the people tested showed some evidence of damage to their heart valves. [3 4] At the time, more than 5 million Americans were taking these drugs.

Did the consumers notice? Could they judge for themselves? They could not. Until it became very severe, the heart damage had no symptoms. Did their doctors notice? They did not. Pondimin was on the market for more than 20 years before two alert medical workers in North Dakota noticed something suspicious. What is the first lesson of the diet drug debacle?

Not only does it take systematic testing to discover whether drugs work, it also takes

systematic scientific study to discover serious adverse effects that are potentially harming millions of people. If we don't have the proper safety system in place, people will be harmed for years or decades. In their potential to harm millions of people there are few rivals for drug treatments–whether they are mainstream prescription drugs or alternative remedies. This is exactly why society has erected the safeguards now being examined in this hearing.

Another aspect of the issue today is more difficult. Should people with advanced cancer, or Parkinsons Disease or full-blown AIDS have the right to any treatment they choose? Some of these people might not live long enough for the kind of drug testing I believe so important to protecting the public. Should they not be entitled to take any risks they choose?

On the surface, the case for individual liberty seems compelling. However, another example will illustrate the dark problems underlying this seemingly straightforward idea.

Suppose you are dying of cancer, and I offer you this ghoulish shell game. In one of my hands, I have hidden a treatment that might save your life. In the other hand, is a quack medicine that will make you so sick you can hardly get out of bed, and will hasten your death. I can give you a free choice. But which hand holds the lifesaving drug? The left hand? Or the right hand?

This is not a meaningful choice. Without extensive drug testing we just can't tell which hand holds a dangerous poison, and which conceals the life saving drug. Without proper testing even a potentially life saving treatment may be harmful if given in the wrong dose, or to the wrong patients. I want people to have choices too. But they should be real choices, involving scientific data about how much harm and good various treatment alternatives can be expected to achieve.

We have a only one proven solution. We need public policies to promote more drug

testing, not still more new loopholes that could endanger the heath and safety of millions of people.

Some may ask, "But aren't people going denied a life-saving treatment for the several years it takes for human testing and drug evaluation?" My answer is that we don't know that it is a life-saving drug until it is tested. Even if proven life-saving, we can't truly hope to save lives until we have done enough testing to know how to use it properly. The history of modern drug treatment includes many cases of valuable drugs that proved ineffective or harmful because they were used in the wrong patients, or at the wrong time in the progression of a disease. [5] [6] Until it is tested, and we know how to use it, a drug cannot properly considered a life-saving treatment.

Alternative medicines pose special problems that deserve the attention of this committee. They are falling between the cracks of the system we have devised to search for new medicines. Large drug companies are expected to invest millions of dollars in the elaborate drug testing we wisely require. In return they are granted patents that are so lucrative that a single blockbuster drug can sustain an entire multinational pharmaceutical giant. This system has provided many beneficial medicines, but at a price. Only large firms can afford the extensive testing required by law. Large organizations tend to follow conventional thinking; daring innovators often work alone or in small firms. It is certainly possible there are neglected therapies that involve common molecules or natural ingredients that cannot be readily patented. Also there may be promising scientific avenues of advance that were ignored or abandoned by mainstream medical research and its partners in the pharmaceutical industry. The tiny office in the National Institutes of Health devoted to alternative therapies doesn't have even a fraction of the resources needed to investigate the most promising leads.

What is needed is money and a structure to target research and assign priorities. The funds could be come from general tax receipts–as do the funds for the National Institutes of Health. Or the research could be financed by a small tax paid by industry. I believe that consumers would be willing to pay an extra amount to insure they got a product that might benefit their health rather than harming it. The policy problem is to figure out how to get the necessary scientific testing done. The solution is not to expose more Americans to untested and possibly ineffective or harmful compounds.

Finally, I would like to address the issue of the FDA and experimental cancer treatments. My main concern is that there is already too much experimental treatment of cancer patients--rather than not enough.

A survey by the General Accounting Office showed that 23 percent of all cancer patients receive an experimental treatment; another GAO study estimated that about 56 percent of cancer patients receive a drug for off-label use–which can be considered quasi-experimental use of an approved drug.[7] [8] Despite the billions we spend on research and treatment, the mortality rate from cancer is higher today than it was in 1970, despite dramatic declines in most other major causes of death. [9] The use of so much experimental treatment may be one important reason we have had such disappointing results. Does the U.S Congress want to expose more patients to experimental cancer agents without the safeguards required for formal National Cancer Institute protocols or human drug testing studies under FDA supervision?

Finally, some people seem to believe that heartless FDA bureaucrats are somehow keeping valuable drugs away from people in life or death situations. I have published articles and books filled with criticism of the FDA, detailing many failings and numerous ways it could do a better

job. [10 11] But I also am here to testify that after 20 years in Washington I have not found a group of more capable public servants more sincerely dedicated to protecting the American public. By the large, they work at a thankless task under very difficult circumstances, and I for one, have great respect for their efforts.

In conclusion, I believe the central issue before the committee today is not access to treatment, but assuring that the proper and necessary drug testing is conducted to insure that both mainstream medical therapies and alternative medicines help rather than harm people. That is easier said than done. But with sound public policies, we can move towards this goal. However, if Congress abandons the essential safeguards of drug testing, there is no limit to the harm that may occur.

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Mr. BURTON. Thank you. Mr. Moore, there's going to be a half a million people die of cancer this year. That's statistics. It's accurate, I think, and you can see that year after year, and we had a graph to show that pretty clearly on the screen.

You held your hands up with two different alternatives. One was quack therapy and one was one that might work. Those half million people who are terminally ill and have been given no hope, shouldn't they have a right to choose or should they just die?

Mr. MOORE. I believe that the job of the Congress is to pursue policies that will create meaningful choices. A meaningful choice is not to embrace desperately a treatment about which little is known. Even if 5 years from now that treatment proves to be beneficial, but it was in the wrong news, that won't help anyone.

Mr. BURTON. Have you read the bill that we're talking about?

Mr. MOORE. Yes.

Mr. BURTON. Have you?

Mr. MOORE. Yes.

Mr. BURTON. And you take issue with that bill?

Mr. MOORE. The bill, as I read it, would basically make FDA approval essentially optional as long as the patient was notified, and second, the only bar on unapproved treatments would be advertising and marketing, but in a media era one national TV show would quickly bring these treatments to expose thousands of people to them.

Mr. BURTON. I want to tell you something you may not have heard before. You may not have been here; I'm not sure; I didn't see you in the audience.

But there was a fellow who was, when I was a State legislator, who was a leading medical authority who later became a leading medical authority in the whole United States of America, and he fought a number of pieces of legislation in the Indiana General Assembly and vetoed them. He was overridden, incidentally. And he later, after he fought anything that was not approved by the AMA and the conventional treatment that was approved by FDA, his wife developed cancer, and he used drugs that were not approved or legal, and it was because—and I don't criticize him for it because it was his loved one who was dying—because there was no hope, and he wanted to try to save her life. Do you think that was wrong?

Mr. MOORE. What I think would be wrong would be to legally authorize practitioners and organizations to prey on the desperate hopes of dying people by holding forth some treatment with a plausible hope on the surface, but that hadn't been tested. What if that person had 10 treatments to try and each one cost $10,000? That's the kind of world this bill might create.

Mr. BURTON. No, I understand what you're saying, but when there's no hope, and the FDA shuts off every avenue to people—and we've had some witnesses here today who have had those avenues shut off—it's pretty inhumane.

I want to ask you another question, though, because you and I don't need to get into a long dialog and debate. I think you know how I feel, and I know how you feel.

In your book, "Deadly Medicine," you talk about the National Institutes of Health and the FDA as bureaucratic institutions that

are infested with vicious politics and are greatly influenced by big money medical lobbies. In fact, your book talks about a drug released by FDA because they were pressured by physician groups, and this drug killed a lot of innocent people. This is your book. Yet, today, you're here to tell us that the FDA should be trusted to oversee the testing of all pharmaceuticals and that they have the best interests of the American people at heart. I don't understand that difference. Can you reconcile that for me?

Mr. MOORE. Well, certainly, and I would expand it, because I'm publishing a new book that's filled with even more criticism of the FDA.

Mr. BURTON. Oh, really?

Mr. MOORE. Yes.

Mr. BURTON. I'll look at that with interest.

Mr. MOORE. But, in fact, I have probably written more pages of criticism of the FDA—now that may not be true—than anybody in this room, but I am a prominent critic of the FDA and its shortcomings. That does not mean, however, for a minute that I don't find that its personnel are well-trained, sincere, and dedicated; that I don't believe that FDA—it also means that I believe they work very hard and conscientiously to do a very difficult job making torturous choices. Mistakes get made——

Mr. BURTON. I understand.

Mr. MOORE [continuing]. And very serious ones get made.

Mr. BURTON. My wife, you may have heard, had breast cancer, and she was in a program that I read about in a national publication, and there are 70-some women in that, and many had been adjudged terminally ill, and they went into the program and their lives have been extended, at least in their minds, for a long period of time. It's an immune-stimulating therapy. The FDA shut that down arbitrarily and left these women without hope. We were able to get it reopened through some discussions, some information being given to the FDA.

But it was a program that was proven to be somewhat effective, and it gave them hope. And, yet, arbitrarily, they shut it down because we had asked them to expand it to include other people, and they said, oh, my gosh, there's several things they have not yet complied with, and so we're going to shut the program down.

What do you think about that?

Mr. MOORE. Well, not only do I think it could have happened, I have chronicled many other mistakes that have occurred, many which involve thousands of lives. You felt one was at stake. So I'm not here to say that we have an agency that is shining perfection. What we have done is to create a set of guidelines, and most of those guidelines and laws make quite good sense, and we need to find better ways to make them work better.

Mr. BURTON. Well, let me finish my time. Have you ever held a position at the National Cancer Institute?

Mr. MOORE. No.

Mr. BURTON. The Food and Drug Administration?

Mr. MOORE. No.

Mr. BURTON. Have you ever taken part in conducting any clinical trial?

Mr. MOORE. No.

Mr. BURTON. OK. Mr. Souder.

Mr. SOUDER. I know it's a little difficult; Mr. Moore's representing kind of one side, and all the other witnesses have been the other. Having been in the minority as a staff person, not as a Member of Congress, and us used to having one or two witnesses under that pressure, I appreciate the difficulty, and I think it adds to the debate to have two different viewpoints with this. It's one that I find intriguing.

I wanted to ask some followup questions, because to me there seem to be some differences in how serious an illness a patient has, obviously, and second, whether there's been any preliminary research on the drug, and whether it's a controlled experiment, where there is some knowledge of the patient, versus something that we have no idea what's going to happen with it.

In that process, one of my—because I've talked to a number of drug companies who have said that they have dropped research on certain AIDS drugs because of the prolonged cost versus the smallness of the market, and that part of the problem here is that, while I see some merit to the research, in some of these highest-risk diseases that cost of the research may be prohibitive from even getting an extended experiment.

Maybe I could start with Dr. Simone, with your background, and then anybody else who wants to comment on it, too. How much of a problem is this, and how much of it could be solved by expanding the eligibility of those in controlled tests versus just going mass market?

I, too, believe that there's probably not a parent or a husband or a wife of a patient who, if they had to fund whatever cost it was to have the hope of extending the life of their child or spouse, wouldn't do it, even if that was a false promise and bankrupted and affected the rest of the family for the rest of their lives. So we do have, I believe, some responsibility to work through this. On the other hand, it seems kind of perverse to say, "but you don't have that option," if you want to take it. Is there some room in between here to maneuver?

Dr. SIMONE. Yes, I think there is a lot of room in between. One of the things that Dr. Moss mentioned was the POMES convention that we had a few months ago, and I think we can work through that. Scientific guidelines need to be met, not anecdotes. Anecdotes are fine to give us the springboard to scientific guidelines, but more appropriate, when a pharmaceutical company decides what area to look at for drug development and research, they look at areas that there are lots of people involved per year. There's 1.2 million people per year of cancer, a little more for cardiac disease, a little less for pulmonary disease. So they look at the places where there are lots of numbers for people to have drugs, because the investment varies anywhere from $100 million to $500 million per drug to come out on the market, depending on what you read and what sources you look at. So they want to make sure they are going to get that investment back. So that do that.

For instance, very few drug companies ever look at any tropical medicine illnesses. It doesn't involve us, but there's also not a big market in the tropical areas for them to do any issues about.

So I think that's what they do. They look at the issue of numbers of people—it's a numbers game—how many people are out there that are going to have this illness, and how can we recoup our R&D investments.

Mr. SOUDER. Are the drugs involved in most of these cases very expensive or is it the research that's expensive?

Dr. SIMONE. I think it's a combination of the research, but also the process of getting the drug ushered and shepherded through the FDA issues.

I got an IND, to give you my own personal experience, I got an IND, which is an investigational new drug approval, permission from the FDA. It took some help from some key people in the Senate to do that. Without that help, it would have cost at least 2 years and many, many, many dollars. Essentially, I got mine through in about 7 or 8 months.

So if you have help, people who are looking after you, I think it can help. But, otherwise, if you have to hire the attorneys, hire the people within as agents for you at the FDA, not part of the FDA but as agents to work through the FDA, if you have to hire all those people and work through all those groups, it costs a great deal of money to do that.

Mr. SOUDER. Dr. Eggers, you've talked about your particular case. I wondered if you could talk a little bit about the question and some followup to Dr. Simone, and also you've stated that they had cited you for incorrect patient consent forms. I assume there were some things that were more substantive that, too. Could you give me some idea of other types of things?

Dr. EGGERS. Well, there have been endless, endless discussions of technical questions. You see, from my point of view, the problem with dealing with the individual bureaucrats is that no one looks at the individual case. From the point of view of most of the people who wrote the letters in my case that I dealt with, they have been people who were either nurses or people with pharmacy training, although I had some access to M.D.'s and Ph.D's. Basically, it's the job of the individual bureaucrat to document your deficiencies. That's the job of that person, and that's how they do a good job. Likewise, when the enforcement people get involved, it's their job to try to send people to jail. That's how that bureaucrat advances his or her career.

So I think it's inherent in human nature that bureaucrats are really going to do things which delay the whole process, and I don't see that tinkering with the system is going to change it.

Also, the process, obviously, is very difficult for an individual at a university without the money of a drug company to deal with the FDA. And if you're talking about an alternative medicine which is nonpatentable, no one in the world is ever going to bring that before the FDA to ever be tested.

Mr. MOORE. Could I just speak to the drug testing issue, because I think he makes a very important point? Things that cannot be patented under our present system are unlikely to ever be tested, and therefore, may not become available. It includes treatments today that literally millions of people are probably taking. I would urge that the committee consider some alternative methods for funding the kind of drug testing that needs to be done. It's not only

alternative therapies. Long-term testing of mainstream drugs is usually not done because it is not found profitable or is too costly for drug companies to undertake. So we have very important unanswered questions about drugs, approved drugs that millions of people are taking.

But a very small charge on every prescription and nutritional supplement and natural remedy, on the order of 1 to 4 percent, would probably pay for most of the testing that needs to be done. I believe that trying to arrange a method to do the testing is a much better solution for the public over the long run than just saying, because it's difficult or too expensive, let's just forget about it.

Mr. SOUDER. In Mr. Shays' subcommittee of this committee, one of the more fascinating hearings I've ever sat through, which I didn't think was going to be fascinating when we started, was on the second use of drugs. It's clear that one of the primary funding mechanisms that any drug company is going to calculate in anything they develop is what are the second and third uses that physicians are going to do, individuals are going to pass through, particularly in children's medicine. It's an area that's just floating around out there, that nobody really knows quite what to do with, because the danger is that, if we do too much of that testing, we then drive out the second use of drugs or make it prohibitive to get at the first use.

Dr. MOSS. Not all of the things that we're discussing here are drugs, and some of them are mind/body techniques; some of them are electromagnetic techniques; or herbs that are traditionally in use for millennia. I think that the Congress set up the Office of Alternative Medicine at NIH in order to carry out this sort of testing. Having served for almost 5 years as an advisor to the OAM, I'm deeply disappointed in the failure of OAM to carry out these tests. I think Dr. Simone has worked with the OAM and worked with me on many of these things, and he can testify to this.

The problem, as I see it, does not lie with the OAM. I think there is an entrenched cancer establishment in this country that does not want to see a fair evaluation of a lot of these alternative treatments, and I think there is an economic motivation because the Food and Drug Administration, the National Cancer Institute, the NIH are economically linked to the pharmaceutical companies that have no interest, and to put it mildly, they have no interest in an herbal treatment that would be extremely inexpensive.

I would fault the manufacturer of the herbs as well, and I have said this many times in public in their presence. You take an herbal mixture like Essiac tea, which is composed of four different herbs, they probably make about somewhere between $8 and $15 million a year, in various formulations selling this tea. There's never yet been a single study done on Essiac tea. One very small, flawed study in 1977 at Sloan-Kettering Institute, but aside from that, they haven't put one nickel into the testing of these treatments. I would like to see some mechanism brought to bear on the purveyors of alternative treatments that, either with moral suasion or through law, requires them to kick back some of the profits that they make to doing these kinds of studies.

But I think Congress has to intervene at this point into this situation over there at NIH and OAM, and look into this, and make

sure that they get this process rolling. I agree with Tom Moore that, in the end, it's only good science that's going to answer the pressing, burning question, which is: What works and what doesn't work? I mean, I differ from Mr. Moore in that I think side by side with this we have to liberalize and open up the process by which patients have access to nonconventional treatment. I think that's only humane, because when it's you and it's your family, you want and you need, and you have to have, choices. If you illegalize those, those avenues, people are going to go ahead and do it anyway, but they're going to have to flee the country in order to do it.

But the essence of it is, let's get that testing process going over there at NIH. Let's break this logjam, so that we really can get the kind of data that we need to make informed choices.

Dr. SIMONE. I have a real quick comment, if I may. Chairman Burton mentioned this morning helicobacter pylori, a bacteria that causes stomach ulcers. The only way to treat it properly is to use a few dollars' worth of antibiotics and bismuth. However, the drug companies, because they've invested lots of R&D dollars and lots of money upfront with these other antacid medications, continue to persuade the public that on advertising, television advertising, that that's what you need if you have stomach indigestion. You see these advertisements all the time. I think there might be a role for that.

We know that it's a very inexpensive treatment to cure ulcer disease, and by the way, this same bacteria causes stomach cancer. So we have another prevention there. Most doctors don't know about it, even today after about 15 years in the literature, they simply continue prescribing these antacids and these very expensive antacid pills. That's one point.

The second point is, apropos the herbal medicines, we know that saw palmetto, a very inexpensive herb, went head-to-head with certain studies in the urological literature for prostate problems with the existing medications used for prostate problems, and it came out equal to, if not more effective than, the prescribed medicines. So in their own literature, in the urological literature, they talk about saw palmetto as being efficacious and equal to the prescribed medications, but very few of the urological doctors prescribe it or recommend it. So we have those issues as well.

Mr. BURTON. Do you think, Mr. Moss—Dr. Moss, is it?

Dr. MOSS. I have a Ph.D. So either one is fine.

Mr. BURTON. We'll call you "Dr. Moss"; a Ph.D. is hard to get.

Do you think this POMES study or issue would solve a lot of the problems, if we could get that on track?

Dr. MOSS. Well, you know, we've had a history of many, many years of contention between proponents of alternative treatments and the cancer establishment over the efficacy and safety of different treatments. We had the Krebiozen controversy in the fifties and the sixties. We had the Laetrile controversy, hydrozine sulfate, vitamin C, Burzynski—these are the main ones. In each case, initially, the National Cancer Institute refused to carry out any testing on these things, saying they were beneath contempt, and it was generally the Congress that forced them to seriously start to look at these things. But in every case that they carried them out—and we could go into the details on this—there was at the end of the

dispute more acrimony, more questions, more disbelief in the system than there was when they went into it. People have very strong feelings one way or the other who's fault this was.

What POMES has proposed is the establishment of an oversight board, and the oversight board would contain people like chairmen of departments at Memorial Sloan-Kettering, like heads of comprehensive cancer centers, as well as patients and informed consumers, journalists, whatever. I mean there would be a broad spectrum of people on that panel, all of whom were distinguished by their knowledge of the disease and by their fairness and their noncommercial involvement, if you will, with the treatments that are being discussed.

This would be a kind of arbitration panel and oversight committee, so that as the protocols are being set up for the testing of a new treatment, and as the clinical trials unwind, if either side has a complaint with the behavior of the other side, then they can bring that problem to the oversight board.

So, for instance—and I'll give you a very quick instance—in the course of trying to do a clinical trial on Burzynski's medicines with brain cancer, the people at the NCI and the FDA and Memorial Sloan-Kettering decided that not enough people were being admitted into the trial. So they changed the protocol midstream. They felt that they were doing this because they could recruit more people into the trials. So it actually was for the benefit of the trial. But you could see how, from Dr. Burzynski's point of view, to change the protocol is to change the terms under which he had agreed to participate. He felt that the patients would no longer respond because they were using people with lower performance scores and more complicated and difficult tumors. He said, to me at least, that he could devise a protocol that could treat these more advanced patients, but the protocol he had agreed upon was not it. NCI went ahead with this, and he objected, and then they canceled the test, saying he was uncooperative.

Now that situation would not have occurred if an impartial, broadly based oversight board existed, because NCI would have had to come to the oversight board and say, here's our problem: not enough people. We want to change the protocol. And Burzynski could come to the oversight board and it could be hashed out in that way, instead of the arbitrariness of the way in which it's done now.

Many times it looks as of NCI wants to be the judge, jury, and executioner of the nonconventional treatments, and that's the situation that has to stop. So that's the core——

Mr. BURTON. We will ask the people at FDA and NIH about the POMES program——

Dr. MOSS. Thank you.

Mr. BURTON [continuing]. And see if we can't get that on track.

One more thing, Mr. Moss, and if you'd keep your comments as brief as possible——

Dr. MOSS. OK, sorry.

Mr. BURTON. You had a story about Laetrile. My colleague, Mr. Waxman, made some comments about that, and a lot of people have said that that was just a crazy product that really had no effect, although in Indiana when we had hearings on that years ago,

we had hundreds and hundreds of cancer patients that came forward and said it did have some helpful effects.

Dr. MOSS. My involvement with this field started in 1974. I was the science writer, hired as a science writer, and then later assistant director of public affairs at Memorial Sloan-Kettering Cancer Center, and we were carrying out very extensive studies on Laetrile in animals, and those studies at Memorial Sloan-Kettering largely were positive in nature; that is to say, Laetrile quite dramatically stopped the spread of cancer in experimental animals.

I, as the assistant director of the public affairs department, was told to tell the public the opposite, that Laetrile was totally ineffective. This was the party line, and I was told to put that out. To make a long story short, after almost 4 years of this, I got up at a press conference and said that, "I cannot do this in good conscious," and was fired on the next day for, as they put it in the New York Times, "failing to carry out his most basic job responsibility," which was to lie on behalf of your boss, if your boss tells you to lie. So——

Mr. BURTON. So the tests at Sloan-Kettering on this particular substance——

Dr. MOSS. Yes.

Mr. BURTON [continuing]. In laboratory animals proved that Laetrile did have a positive impact on some cancers?

Dr. MOSS. Well, it was an animal model, in that in three different animal systems, especially in one breast cancer model, there was a dramatic reduction in the——

Mr. BURTON. Why was that the case? Why did Sloan-Kettering, a leading cancer institution, why would they condone misleading the public like that?

Dr. MOSS. Well, up until 1975—1974–1975—they were very, very excited and enthusiastic about Laetrile, and the top leaders of the center came to Washington in 1974, made a very strong presentation on behalf of Laetrile, and basically, between that time and the time they came back in 1975, a lot of pressure was brought to bear on them to change their mind about this.

Mr. BURTON. Was it pharmaceutical companies or——

Dr. MOSS. Not directly. The pressure came from the FDA, the American Cancer Society at that time, the National Cancer Institute, although there was ambivalence at National Cancer Institute, and Dr. Dean Burk, who was one of the founders of NCI, shared the views of the Sloan-Kettering scientists. But I saw them change their minds as they realized that this was getting so hot on a personal level. As one of the officials said, "I don't want to die on the barracks for Laetrile. It's not a cure for cancer. It's only a palliative." And he didn't want to give up his career, which is what it would have meant, essentially. I've covered this, by the way, very extensively in my book, "The Cancer Industry," talked about this a lot.

Mr. BURTON. How much is it? I might buy a copy. [Laughter.]

Dr. MOSS. For you, nothing. [Laughter.]

Mr. BURTON. No. Can we accept books? I don't know whether we can or not. We can't accept some books.

Do you have any more questions?

Mr. SOUDER. I want to make one additional comment for the record, because—and I think it's an important warning for everybody with this as to how Members of Congress react to this issue, and that is that, many of us want to be as receptive as possible to giving people options, but we also want to be cautious in some areas.

I remember a number of years ago when I worked as Republican Staff Director of the Children and Family Committee, and we were holding some hearings with some Indian tribes, and one of the leaders of the Utes was arguing that our health funds that go to their tribe should be able to be used for Indian medicine men, because he said their cure rate is as effective as hospitals because so much is psychosomatic, because a lot of people catch other disease in hospitals. There it became extra complicated, because once you give it to an Indian tribe, the people in some of those Indian tribes didn't want to go to the medicine man; they wanted to go to a hospital. But these questions come up in many parts of our health policies, and there's not a lot of basic public support at this point for necessarily giving somebody's tax money to an Indian medicine man as opposed to a hospital.

A second element with this is that we're having a huge national debate, which I believe is inappropriate, about the so-called medicinal use of marijuana, where you have a component in marijuana that can have some impact, but that, in fact, it has other public policy implications, and in fact, can be used by people who want to get in the public policy debate as a backdoor way to change drug laws in this country. That is a huge issue that's going to be developing and get hot in this debate.

Like I say, many of us who are receptive to trying to broaden this and to make sure there's a scientific base also want to make sure that we're not just opening the door toward every type of experiment funded by the taxpayers of somebody who says they have some kind of instant solution. It's got to be a scientific-oriented-type thing, as much as possible controlled, because we already have enough budget problems without chasing everything that comes up, and getting into other public policy areas that inevitably cross this issue.

Mr. EMORD. May I speak to the safety point?

Mr. SOUDER. Yes.

Mr. EMORD. I think it's an extraordinarily naive assumption that the Food and Drug Administration is the source of protection for the health of the American people, as if it is exclusively the only source for that protection. The protection, in fact, is extraordinary at the State level. The real situation here is a patient who is terminally ill, seriously ill, going to their physician, receiving conventional treatments that, unfortunately, fail in the case of cancer, and then looking for an alternative. In the first instance, the person who has the disease is extremely self-interested. They want to do what's best for themselves, and they would take a drug if it would cure them, but it won't. So they're cautious.

Now the argument that they throw caution to the wind has been made, and I think in some instances some people may do that if they're desperate. But the point is they can't act alone. What do they do? They go see the physician. The physician's under the State

medical board. He has to meet his standard-of-care requirement in order to be licensed and to function. He pursues his best interest. If he can't protect himself from being charged with not meeting the standard of care, he'll be out of business, and the medical boards are very effective in doing that, all too effective in certain instances.

Now that doctor protects the patient and provides a safety net for the patient. Then if we're talking about an experimental drug, the physician is going to the manufacturer or to the clinical investigator and asking that person to evaluate the patient's chart and determine whether or not they should be a candidate, either for an emergency exception or what have you. They, then, conduct an evaluation. What pressure is there? Well, they have to worry about being sued if they were to give it in a circumstance that would be grossly negligent. They have to worry about the Food and Drug Administration second-guessing their judgment and questioning them about the propriety of their judgment somewhere along the line. They also have to worry about the results of their clinical trial and whether or not this will prejudice those results.

Now there are disincentives in the current system that are profound and that prevent these drugs from reaching people who seriously need it. The clinical investigator or the sponsor of the drug has to worry about the fact that the FDA will take any information from an emergency patient who gets the drug outside of the protocol and use that in determining efficacy. And what does that do? That creates a huge disincentive for the company or the clinical sponsor, because they don't want to lose control. They want to have the precise patients who meet the criteria of the protocol being the only ones, because all the millions that they've spent on drug development will rest in the FDA's determination in the end.

So, to make a long story short, the safeguards are there. You've got doctors who are protecting patients, and they do this on a day-to-day basis, not with experimental drugs; 80 percent, 70 to 80 percent of approved drugs are used for off-label indications—experimental indications. We have that happening right now.

So the question is, why are people not dying right and left from doctors giving out drugs? The reason is that the primary safety is not the FDA, which very rarely interferes with the doctor's practice; it is the doctor. When you have a desperate situation, when you have a person who's terminally or seriously ill, and they need an alternative, they need an alternative today; they can't wait until the completion of clinical trials. They need an alternative. If that alternative is comparatively safe, in the judgment of the professional who is caring for that patients, then if the Federal Government stands in the way of that patient getting that treatment, it is an outrage. You're denying that person. If tomorrow the cure is substantiated, but today it's not, and in the professional judgment of that physician, who knows the clinical history of that patient, knows it better than anybody else, that that patient should be given that alternative, then why should we have the FDA serve as a super-M.D.?

And the FDA, by the way, in making these judgments, has maybe two or three pages of information about the patient. They make that judgment in a couple of hours. They might hand it off

for professional review, but within 24 hours usually they're making this judgment. They're oncologists, some of them; they're not specifically trained in that cancer. They're not scientists who understand the specific drug interaction, the specific cancer that's in issue. They're not experts. They're doctors. They're not scientific experts, and we are letting them make the judgment about life-or-death issues.

Better to leave it with the doctor who's studying that for year after year after year, struggling with the patient's life, trying to make a determination. We shouldn't let someone in Washington second-guess the professional judgment of oncologists, hematologists in the field, trying desperately to make a judgment to keep someone alive. It seems to me to be an outrageous scenario when we allow that to happen.

Mr. BURTON. I yield to Mr. Mica, but before that, Mr. Moss, you can make a brief response.

Dr. MOSS. If I could, I'd like to make a brief comment about quackery.

Mr. BURTON. Any of you on the panel that want to respond is fine. There's not that many of us here, and Mr. Mica will be with us.

Dr. MOSS. A couple of the Congresspeople brought up the issue of quackery, and whether the Access to Medical Treatment Act would encourage quackery. I do think that quackery exists in the cancer field, and it is a problem, although I think oftentimes it's exaggerated as a problem, but I do see treatments for which there is virtually no substantiation and little rationale, and that are marketed, heavily marketed, in unethical ways that oftentimes cost a lot of money for people. They're probably throwing out their money. So it is a problem.

But my feeling is that the intransigence of the medical establishment toward alternative treatments is what creates a fertile climate for quackery; that the more treatments that are brought into the mainstream, the more options that people have, the less likelihood it is that people are going to turn to these so-called quack treatments, and that is because they will have a chance to have choices when they've been given a diagnosis of terminal cancer, for instance, that otherwise they would have to go to Mexico or some other country to get.

I think also the intransigence of the medical establishment, the cancer establishment, gives credence to the idea that there is a single suppressed cure for cancer that you can only get down in the Dominican Republic or some other place, and that, therefore, it's all a conspiracy against the patient.

If we can break through and allow some of the treatments that are used in countries like Germany or Japan to be done in a clinical setting here in the United States at good medical centers, as experimental treatments, then I think we cut the ground out from under the whole phenomenon of cancer quackery.

Mr. BURTON. Mr. Mica.

Mr. MICA. Thank you, Mr. Chairman. I had a couple of questions for Mr. Emord.

I guess you're an attorney who has specialized in representing patients who are trying to get access to investigational drugs, and

you've had experience, specific experiences, in that regard. You're familiar with the FDA Modernization Act and how it approaches resolving some of the problems you've seen. Where are its strengths and weaknesses, or how would you approach this as an attorney recommending us in drafting legislation to deal with the problems you've seen?

Mr. EMORD. Well, thank you for that question. The FDA Modernization Act, section 561, is the provision that purports to expand access to alternative therapies—drugs and medical devices. There is, in fact, no significant, substantive change from existing law and this codification. Why is that?

The present disincentives for a drug company or clinical investigator to supply a drug on an experimental basis are unchanged by this law. What are those? The clinical investigator or the sponsor of a drug puts together the patient group for the protocol and the protocol design to maximize the chance that the FDA will approve the drug. The FDA takes into account all information on treatment, including that which occurs as a result of an emergency use. So long as that emergency use information is made a part of the evaluation of efficacy, drug companies will fear supplying the information because they have patterned—they have done their studies and research on specific patients who meet certain design criteria and not the other ones.

In addition, they're regulatees, and they fear offending the FDA. They fear offending the scientists on the staff of the FDA. They don't want to do anything that can jeopardize the millions invested. This doesn't affect that at all.

What would affect it is if we said, in every instance where an experimental use of a drug is to take place, that the sponsor of the drug or the investigator need only receive, for example, from the patient, or the patient's physician actually, an informed consent that says, look, I as a doctor, I've informed the patient of the available conventional alternatives; the patient has exhausted those alternatives or has refused to take them, for whatever reason; an experimental alternative seems appropriate to me as a professional; I recommend your drug.

This informed consent, with known risks and benefits of the conventional treatment that would have to be explained, could go to the clinical investigator sponsor; they could then immediately send the drug and notify the FDA that they had done so, but that information on the treatment used would not go into the efficacy determination. Now that would ensure more availability to drugs.

The second disincentive: economic disincentive. Currently, a company that's invested massive sums of money can't recoup that investment until after the drug is approved, basically. Nevertheless, if there are large numbers of people who physicians think should have access to this experimental drug, they can't afford to make these drugs available over a long period of time because of the extraordinary cost. They've got to be able to recoup some of that money back.

So if we allowed payments to go to the companies for those drugs, it would ensure that they could be made available. If we would allow them to recoup some of the profit potential, that would help.

The third thing is the FDA's final say-so here. The FDA, in the section 561, maintains the final say-so, just as it does currently. They can say no. If a physician seeks to use an IND or an emergency exemption or a treatment IND, the Secretary of HHS, and by delegation the FDA Commissioner, will second-guess that judgment, has the right to second-guess that judgment. We've got to get the FDA out of the business of second-guessing clinical judgments.

We all fear fraud. I just as much as anybody else do not want to see patients taken advantage of. But we have a Department of Justice that prosecutes fraud and that uses specific criteria to determine the presence of fraud. We have States' attorneys general that do the same thing. In this area where we don't have scientific certainty, this is all experimental.

Dr. Simone has told you that approved drugs don't work for cancer. They're experimental. In this experimental area we have to allow people access, and if we have a charlatan out there who's selling something that does not work and is profiteering off of it, we have the Department of Justice and others who can prosecute.

Notice this one thing: The present system, I would argue, expands fraud, actually promotes it. Why? Because the FDA has so narrowly prevented physicians from getting access to experimental drugs; they tell the patient there's nothing I can do for you. What does the patient then do? The patient, who doesn't have any scientific background usually, will go and try anything they can get their hands on to see if it will work. So they're duped by frauds who prey upon them.

If we would, instead of doing this, allow the system where you go to the clinical investigator, you go to the sponsor of the drug, you have the physician say, "I think he needs this drug"; you let the drug be given to them, and you cut it out of the approval process. Then you're going to have a situation where fraud is dramatically decreased, because people will not be as vulnerable.

Mr. MICA. Well, you've taken some time and explained your response. So you're saying that section 561 basically is not any change from existing law, that you're recommending that we allow an informed consent process. I guess we do that to a degree. I think my wife had an operation, and I signed some papers, or she did, and they did this procedure dozens of other times—or thousands of other times—but they still tell you that there may be risk. So informed consent is missing from—or this level is missing and is needed, you think something of that nature.

Then you said payment to drug companies, and you had a third recommendation.

Mr. EMORD. The third one would be the disincentive on——

Mr. MICA. Right.

Mr. EMORD [continuing]. That the clinical trial data that would come from an emergency use or an experimental use would not go to the FDA.

Mr. MICA. Well, I have a couple of questions, if I may, Mr. Chairman. How's our time? Do I have time to proceed?

Mr. BURTON. Yes, go ahead.

Mr. MICA. Thank you.

I don't know if I've seen a specific legislative proposal that would alter this, and maybe you have that that you can provide the com-

mittee. I don't know that much about the process, but I'm concerned that we just allow anyone to have the ability to say sign an informed consent. I'm wondering if there is some level on which FDA does look at these drugs or these treatments. Right now the problem seems to be getting past all of the trial and approval time, where we've seen some initial results that seem to be promising and where you have someone in a cancer or terminal situation or ready to grasp it, and anything and everything else is excluded. Is it possible to have some level of approval by FDA to where this informed consent kicks in?

Mr. EMORD. Well, I think that so long as we are talking about existing clinical trials that the FDA is supervising, getting patients to have access to that is what we're talking about. The FDA, remember now, has basic control over the existence of the clinical trial. Now this doesn't solve the entire problem because the FDA does not allow some drugs to go into the clinical trial process, and that's a separate problem. But taking the universe of drugs in clinical trials, and most of the drugs we've heard from today are in those clinical trials, so that all we're talking about is giving one more person that drug on a doctor's recommendation with informed consent. There are people already taking it. If it was a hazard to health, the FDA would shut the thing down, and they have the power to do so. So there's your safeguard.

Mr. MICA. Well, then, the other question that I have is, in payment to drug companies, are you talking about the individual seeking the treatment paying the drug company?

Mr. EMORD. Well, yes.

Mr. MICA. The other problem you have is folks who are terminal, who want this experimental drug treatment, are willing to pay anything, and now we do force them into going to Mexico or some bizarre treatment that is off, totally off the charts. The most expensive time to develop a drug is probably in its experimental use versus when it's marketable, because then you have a much broader area. What's to prevent the drug companies from gouging folks to get into this experimental routine?

Mr. EMORD. Well, we should prohibit gouging, and we can require that they would publish or make publicly available the amount they're charging for it.

A drug company, recognize, has to rely upon several things: good will. They have to rely on the good will associated with their product. They have to prove their product works over the long run. In addition, if you're talking about access for terminally ill patients, any company that would charge an extraordinary sum of money to make a product available beyond that which was justified, based on the cost of investment in it, would be, if that information had to be public, publicly excoriated, and the FDA wouldn't take too kindly to them either. Remember, these are within clinical trials.

One other point: The access bill is actually the bill that would provide the greatest source of relief, and the details I'm talking about now could be either made a part of FDA regulations in implementing it, and should be, or could be discussed here in Congress and made a part of the legislation.

Mr. MOORE. May I speak just for 1 minute on this issue?

Mr. BURTON. Go ahead, Mr. Moore.

Mr. MOORE. Well, I just wanted to note for the record that the existing provisions of the FDA Modernization Act have some very sound logic behind them. You would certainly want to modify those with considerable care.

For example, one of the concerns he mentioned was that people do indeed have to report on how the treatment worked. If we have thousands of people who are getting, under compassionate use, a drug, that is valuable safety information that could teach all of us something about the safe use. And I would hate, on behalf of society, to lose that information.

We have also traditionally prohibited charges for the drug——

Mr. MICA. So would you require that they be reporting or——

Mr. MOORE. That's what the current law requires, and I think it's wise.

Mr. MICA. OK.

Mr. MOORE. Second——

Mr. BURTON. Let me just ask a question there on that subject. As I understood it from the counsel, though, that might prohibit or discourage the drug companies from providing that needed remedy or possible remedy to a patient who is terminally ill because they're afraid that that information would be put into the record, and it would long term hurt their investment. Is that not correct?

Mr. EMORD. That's true.

Mr. MOORE. Actually, there is evidence that drug companies are reluctant to provide investigative drugs, but I think I would state it a little differently than he did. He may have actual cases where he's seen this, but a normal efficacy trial may only include specifically enrolled patients who meet the enrollment criteria. So it would be very unusual—he may know of some cases—but it would be very unusual where it would affect the efficacy of the drug.

Where the concerns he expresses are more real, however, are this: You have a group of high-risk patients, many of them terminally ill. So the drug company would be reporting a significant number of patient deaths, especially if the drug treatment involved wasn't very effective and said, the study you've been hearing about, cancer drug treatments, most of them aren't terribly effective; we wish they could do better.

So there is some concern about that, but that issue, if we have information, can be isolated by analysis, and I know of many cases—in fact, heart drugs that I wrote about in my previous book, the FDA received many, many, many reports of patients who died with compassionate use. In fact, about 30 percent of the patients who received one drug by compassionate use died, and this did not interfere with the approval of the drug. It was part of the safety data, and the experts understood, or thought they understood, that it was because these were very seriously ill patients that they died. It turned out the drug had a role in it, too, but, by normal analysis, we do have protections from this.

So the provisions of the law were carefully thought out to balance society's interest against the patient's interest, and I think it represents quite an interesting and important compromise, and I would tinker with that balance with great care.

Mr. EMORD. The law does nothing substantively to change the status quo. The issue about patient safety, a physician who has a

patient who is using an experimental drug, experiencing adverse reactions, will stop that patient from taking it. That might not be true within the context of a clinical trial if devious minds are at work, attempting desperately to get those millions of dollars recouped in a drug approval.

Furthermore, once the drug is approved for a specific indication, it can be used for any other indication, and will be by physicians, based on the clinical data they gather during their experience in the use of the drug. The point is it is a bugaboo to suggest that only the FDA will protect the public. The people who protect the public every single day, and we trust to protect the public, are the physicians who care for them. The FDA is very rarely involved in these circumstances, and thus far, if the history of the testimony that's been presented to this committee and other committees is indicative, the FDA has done a damned poor job of protecting seriously ill patients because it has let them die rather than try an experimental alternative.

Some experimental alternatives will be risky. Some of those alternatives may not work, and the person will die. But compare that opportunity to succeed and a doctor's belief that there's a good chance that it might work with the FDA's position that, until we have established the clinical efficacy of this thing, you shouldn't be able to be given a chance to use it—and the problem with this in the end is that the FDA decides, second-guesses the judgment of the physician and decides, based on very limited information.

You wanted a specific example. I had a patient who had cancer. David Smith was his name. We went through the whole process. The FDA wouldn't allow him to have an experimental treatment, but he had Hodgkins disease. We had MRIs to show, before he took an experimental treatment, what his condition was; after he took the experimental treatment, what the condition was. We could not get the FDA to review the MRIs, and the answer was, "We have a backload of cases. We've got to go through these things rapidly. We have to do it in 24 hours. We can only look at a couple of pages of information. We don't have time to examine the MRIs. We don't have time to examine the entire clinical history of this patient. We don't have time to consider all the drugs that have been given to this patient or treatment that was given to this patient. You just summarize for us what happened, and we'll base our determination on that."

Now that's not science, and that's not sound medicine. And, yet, the super-M.D. here, the FDA, is basing its judgment upon it. This system does not work. It is not adequate, and it's hurting people. It's resulting in the loss of life, and it's causing enormous stress to the American people.

Mr. BURTON. Do you have any more questions?

Mr. MICA. Well, just to conclude, the problem we have, and I've dealt with this on a personal level with folks—I know one of my best friends just had his 30-year-old daughter diagnosed with terminal—well, inoperable brain cancer, and they are doing everything they can to try and find every treatment possible. Because of the restrictions you just described, and because now it looks like what's being proposed isn't going to really solve the problem, we forced these people—I mean, this man, these people will do any-

thing to save their daughter. So we force them into Mexico or these bizarre treatments, and give them no other alternative. It appears we've also created a career for you to do the alternative, which is sue to get access. It's not acceptable, and I'm saddened to hear that what's proposed isn't going to change this at this time.

Thank you, Mr. Chairman.

Mr. BURTON. Yes, sir.

Let me just end up by saying, first of all, this has been a very informative day for me. I am not a medical expert, as you could probably tell. But we on this committee I think will be committed to trying to help find solutions. I know that there's another committee that's charged with the responsibility of moving legislation in this area, and that's the Commerce Committee. Chairman Bliley and I are pretty good friends. So if we can come up with some recommendations, I will sit down with him and make those recommendations. And we will continue to have hearings on this to try to force the issue, because we have oversight over the entire Government, including the FDA and NIH and every place else.

Let me just ask you to do me a favor, though, all of you, and this includes the patients who are still here. If you have recommendations that you think we ought to consider as a Congress to make this system work better, in addition to what we've already heard today, I wish you would get those to me in writing as quickly as possible, because I'm going to be meeting with people from the FDA in the next week or two. Make them very simple, so that I can understand them, so that I can communicate those to the FDA. If you will do that, we'll see if we can't do something to help streamline this system and make things a little bit better, and maybe help a few people live a better quality of life, live a little bit longer.

With that, I want to thank you very much. Mr. Moore, Mr. Emord, Dr. Simone, Dr. Moss, and Dr. Eggers, thank you very much for being here. Thank all of you patients as well. [Applause.]

The committee stands in recess.

[Whereupon, at 2:17 p.m., the committee adjourned subject to the call of the Chair.]

[Additional information submitted for the hearing record follows:]

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February 2, 1998

VIA FEDERAL EXPRESS
Laurie S. Taylor, Esq.
Committee Counsel
House Committee on Government
Reform and Oversight
2157 Rayburn House Office Building
Washington, D.C. 20515-6143

Dear Laurie:

As we discussed, I have attached a letter for inclusion in the hearing record identifying the principal deficiencies of FDA's current treatment use protocol and IND process and recommending an alternative. I look forward to seeing you on February 4. When should I arrive?

Best regards,

Jonathan W. Emord

Attachment

EMORD & ASSOCIATES, P.C.
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February 2, 1998

The Hon. Dan Burton
Chairman
House Committee on Government
Reform and Oversight
2157 Rayburn House Office Building
Washington, D.C. 20515-6143

Re: Federal barriers to patient access to treatment

Dear Congressman Burton:

This letter supplements my written testimony before the House Committee on Government Reform and Oversight and is offered for the hearing record. It focuses on federal regulations now in place, explains how those regulations are interpreted by the Federal Food and Drug Administration (FDA), and recommends a legislative reform that would remove barriers that impede competent, fully informed patients (diagnosed as terminally or chronically ill) from exercising freedom of informed choice in the selection of promising experimental treatments.

I start with a bias in favor of the terminally and chronically ill. As a matter of principle, I believe a terminally and chronically ill patient's right to life and liberty encompasses the freedom to accept or reject any treatment recommended for disease. I believe each such patient rightfully the master of his or her own body, possessing a property right in that body, a right the state may not deprive without a compelling interest and due process of law. Actions that make premature death inevitable, such as FDA decisions to disallow a terminally ill patient access to an experimental drug when approved drugs fail, constitute arbitrary deprivations of life without a compelling interest and without due process of law.

In *Cruzan v. Director, Mo. Dept. of Health,* 497 U.S. 261, 278 (1990), the Supreme Court recognized a constitutional liberty interest in a patient's rejection of unwanted medical treatment, even if that decision contributed to the death of the patient.

In *Washington v. Glucksburg*, 117 S.Ct. 2258 (1997), the Supreme Court refused to recognize a constitutional interest in taking life-ending medication for the purpose of alleviating the pain and suffering of chronic or terminal illness. The Court found no historical recognition of a suicide right in Anglo-American jurisprudence. Several members of the Court did indicate, however, that were the case one of state denial of palliative care for the chronically or terminally ill, a constitutional liberty interest would be implicated.

Surely a liberty right that protects a patient's decision to reject treatment (and thereby hasten death) cannot logically be limited to a freedom to die through rejection of life support. That right must also protect a patient's decision to fight for life when FDA-approved drugs have failed (through the ingestion of experimental drugs). In the case of a terminally ill patient who fights for life, the right to liberty becomes inextricably intertwined with the right to life (the freedom to control one's biological destiny and fight for survival through promising, experimental therapies). A government violates the liberty and life rights of its terminally and chronically ill when it effectively compels them to rely on failed treatments by denying them access to experimental alternatives.

Under its statutory mandate, the FDA starts with a legal bias in favor of preventing the distribution of any unapproved drug until it has determined whether that drug is both safe and efficacious. The avowed purpose is to protect the public from harm by ensuring that no drug is marketed that is either unsafe or inefficacious. That purpose would appear served for illness that is curable with approved drugs, but it is disserved for illness that cannot be cured with such drugs. Indeed, rigid adherence to the position that only approved drugs can be used to fight illness is a death sentence for those patients whose illnesses are presently deemed incurable. In the case of the chronically and terminally ill, the aim of protecting against harm is best served by a frank recognition that in the absence of an approved cure, experimental drugs provide the only hope and should not be withheld. If our government truly values life, it will not stand in the way of a chronically or terminally ill patient's access to experimental alternatives but will craft its laws to make such access convenient, when based on fully informed consent.

In apparent recognition of the need to afford limited access to experimental alternatives, the FDA has adopted regulations that permit investigational drugs to be used under either a "treatment protocol" or a "treatment IND" (21 C.F.R. §§ 312.34; 312.35). The acronym IND stands for "investigational new drug" application. Under this system, the patient may only use drugs that are, in the first instance, made available by a corporate sponsor or clinical investigator and, in the second, approved for the use by the FDA. Through one route, a sponsoring company can file a treatment protocol with the FDA under an existing Investigational New Drug application. Implementation of the protocol must receive FDA approval. Through another route, a clinical investigator or licensed practitioner can submit his or her own Investigational New Drug application and receive a drug from a sponsoring company or clinical investigator for treatment use. Under what is frequently termed a "compassionate use exemption," FDA will authorize a treatment use outside IND protocols if certain criteria are met.

To start, only the corporate sponsor or the clinical investigator may file a treatment protocol, a treatment IND, or seek a compassionate use exemption. As a consequence, a terminally or chronically ill patient or his or her physician must first convince a corporate sponsor or clinical investigator to agree to make the drug available. There is a natural disincentive for the sponsor or investigator to do so. They seek ultimate approval of their experimental drugs from FDA and generally do not want to risk losing control over trials involving the drug. They have expended enormous resources in designing protocols that include patients who are the best candidates to establish the efficacy of the drugs. When patients outside protocol requirements are given access to the experimental drug, they increase the risk that data collected from their use of the drugs will be negative and will jeopardize overall drug approval, thus forfeiting all money expended for the drug's development. In addition, investigational drugs are expensive and sponsors and investigators cannot charge a market rate for the drugs distributed to patients. As a consequence, many corporate sponsors and clinical investigators routinely deny terminally and chronically ill patients access to their investigational drugs. While a licensed practitioner can file a treatment IND, that IND is of no use if a corporate sponsor of a drug refuses to make it available fearing the effect clinical data will have on drug approval.

In addition, even if a sponsor or investigator will file for FDA approval of the use, the FDA will, in the last analysis, decide whether that use is approved. In exercising its discretion FDA can, and frequently does, second guess the judgment of highly trained practitioners with specific expertise in the disease concerned as well as the judgment informed by years of clinical experience with the patient of the attending physician. If FDA is not satisfied that there are no comparable or satisfactory approved drugs or therapies, it will deny access to the drug. It will do so even if it finds, contrary to the expert's advice and counsel, that a chronically or terminally ill patient may benefit from continued treatment with an approved drug or therapy that has previously failed to cure the patient's illness. It will do so even if the patient is fully informed of known risks and benefits of the approved treatments and seeks an alternative treatment, aware of the potential risks of that treatment. It will do so even if the patient refuses the approved drug or therapy and indicates that he or she will choose death over continued use of the approved drug or therapy. In short, in the last analysis, FDA substitutes its judgment for that of the patient, attending physicians, and medical experts. It has the power to (and it has in fact) overruled the recommendations of attending physicians and medical experts and refused access to experimental drugs on three occasions in my experience.

Moreover, those who review treatment use requests, examine a comparatively small amount of data before making their decision and lack specific education, training, and experience in the precise kind of disease under review. It would be virtually impossible for FDA to hire and retain scientific experts conversant in the science and latest findings on every kind of terminal and chronic illness. They do not do so. Instead, they rely on a few skilled professionals to make life or death decisions on all manner of terminal and chronic illness, despite the fact that they frequently lack a thorough review of the scientific literature, clinical experience in the treatment of the disease in question, and any detailed scientific understanding of the active constituents in the investigational

drug in question. They lack the time to review the entire clinical history of the patient and, so, do not do so. They lack the time to review MRIs and other best evidence of the patient's condition, and, so, do not do so. They frequently only have sufficient time to examine a limited, paper submission filed by the sponsor or investigator and make a "go - no go" determination on that. Incredibly, the FDA makes life and death decisions based on this limited review and there is, effectively, no recourse for a patient denied access.

Based on my experience and that of my colleagues, I believe the process heavily influenced by political, rather than scientific, factors. Indeed, a patient who has connections in Congress, can afford to hire attorneys to wage a battle with the FDA, and can acquire media attention for his or her plight is more likely to receive approval from a reluctant FDA than one who has neither the contacts nor the money to pay for such a campaign. The process thus discriminates against those who are least able to defend themselves, the chronically and terminally ill who lack financial resources. For the poor, the system is a cruel one, affording no alternatives.

To correct the deficiencies of the present system, Congress should: (1) remove the disincentive from sponsors and investigators' supplying investigational drugs to attending physicians for use in the treatment of chronically and terminally ill patients who fall outside protocol requirements by prohibiting FDA from evaluating data obtained from such uses unless requested by the sponsor or investigator; (2) allow attending physicians to administer investigational drugs acquired from sponsors and investigators upon (a) establishing that the patient is chronically or terminally ill; (b) attesting to the fact that approved drugs and therapies have either been tried unsuccessfully or refused by the patient; and (c) obtaining a written, fully informed consent from the patient that specifies known risks and benefits of approved drugs and therapies, perceived risks of the investigational drug, and patient signed approval for administration of the investigational drug; (3) remove FDA from the business of approving treatment uses for chronically and terminally ill patients who fall outside protocol requirements by instead allowing sponsors and investigators to make that decision and merely report treatment use distributions of the drug to FDA; (4) deny FDA authority to halt such treatment uses to instances in which the Department of Justice through the United States Attorneys' offices has instituted criminal proceedings in federal court against a sponsor or investigator on grounds that the sponsor or investigator has engaged in fraud against the United States or against the treatment use patient or patients during the clinical investigation; and (5) permit sponsors and investigators to charge patients for drugs distributed for such treatment uses.

The new system described here is effectively brought about through the Access to Medical Treatment Act. The envisioned reform would dramatically decrease the ability of the FDA to block patient access to promising investigational drugs. All requisite safeguards would still be in place. In operation, the system described in the paragraph above would work as follows. A chronically or terminally ill patient, on the advice of his or her physician, would execute a consent form. The form would describe the approved drugs and therapies for the patient's disease and their known risks and benefits. It would also describe the known risks associated with the investigational drug, and it would

include the patient's consent to administration of the investigational drug. The physician would send a copy of the consent and other clinical information to the sponsor or investigator, as the case may be, and seek access to the investigational drug. The sponsor or investigator would then determine whether to make the drug available and would report to FDA any distribution of the investigational drug to a physician for administration based on a treatment use. The sponsor or investigator could charge the patient for the drugs distributed for treatment use outside approved protocols. The FDA would be barred from taking into account for drug approval purposes any treatment use by patients outside approved protocols unless the sponsor or investigator wanted FDA to do so. In those rare instances when a sponsor or investigator perpetrates a fraud against the government or the patient, FDA would not handle the matter in the first instance but by the Department of Justice through the United States Attorney's office. If a USA were to file suit alleging fraud against the government or a patient by a sponsor or investigator, the FDA could then order a halt to the treatment use.

This revised system provides appropriate safeguards against unlawful marketing of unapproved drugs at the same time that it disarms FDA from abusing its power by denying chronically or terminally ill patients access to experimental drugs when FDA-approved alternatives have failed. It also removes FDA from the business of second guessing the judgments of patients, attending physicians, and medical experts and, instead, commands FDA to honor their requests unless the United States institutes actions against the sponsors or investigators from whence the drugs come.

The Committee's attention to this matter is long overdue. A resolution that will cause the FDA to respect, instead of flout, the wishes of the chronically and terminally ill, their attending physicians, and medical experts is sorely needed.

Sincerely,

Jonathan W. Emord

ROMAN KNIER

BURZYNSKI
RESEARCH INSTITUTE, INC.

June 25, 1996

VIA FEDERAL EXPRESS

Paul Zimmerman, C.S.O.
FDA/CDER Oncology, HFD-150
1451 Rockville Pike
Rockville, MD 20852

RE: IND #43,742

Dear Mr. Zimmerman:

I am hereby submitting three copies of the application for Compassionate Exception to the Protocol in IND #43,742 for Roman Knier. In the attached documents you will find a short patient history and a rationale for the treatment with Antineoplastons and an explanation why the patient does not fit under the original protocols under the IND.

This patient's treatment will be conducted according to Protocol BT-21. The Informed Consent Form will be the same as required by Protocol BT-21.

The patient will receive treatment under my care.

Sincerely yours,

Stanislaw B. Burzynski, M.D., Ph.D

12000 RICHMOND AVENUE • HOUSTON, TEXAS 77082-2431 • (713) 597-0111 • FAX (713) 597-1166

BURZYNSKI
RESEARCH INSTITUTE, INC.

**APPLICATION FOR COMPASSIONATE EXCEPTION
TO THE PROTOCOL IN IND #43,742**

Patient's Name: KNIER, Roman

Diagnosis: Glioblastoma multiforme.

Sponsor: Burzynski Research Institute, Inc.

Principal Investigator: S. R. Burzynski, M.D., Ph.D.

Short Patient History

The patient is a 63 year old white male who has been diagnosed with left frontal lobe glioblastoma multiforme confirmed by biopsy. The diagnosis was confirmed by Dr. Lucy Rorke at Children's Hospital of Philadelphia. On May 3, 1996 a left frontotemporal craniotomy was performed. The patient has significant aphasia with no attempt to converse, impairment in reasoning and short-term memory loss. The patient has decided to first pursue conventional means of treatment with BCNU and radiotherapy which will be completed on July 5, 1996. The total amount of radiation at the time of completion will amount to 5940 cGy. An interim CT scan performed on June 6, 1996 halfway through the radiation therapy has indicated stable tumor size in spite of further clinical deterioration of the patient. It is proposed that the patient be admitted to take the treatment with Antineoplastons according to Protocol BT-21.

Rationale for Treatment with Antineoplaston A10 and AS2-1

The patient is suffering from a highly aggressive form of a neoplasm for which no established curative protocols are available. I have been informed that the patient's son, Mr. Steve J. Knier, has consulted with three employees at the FDA and he has been informed that Compassionate Exception will be granted to his father, Mr. Roman Knier as soon as the application is submitted. This application is being submitted based on the information obtained from the patient's son and at the patient's request.

June 25, 1996

S. R. Burzynski, M.D., Ph.D.

T:cm

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FOOD AND DRUG ADMINISTRATION
OFFICE OF DRUG EVALUATION I

DIVISION OF ONCOLOGIC DRUG PRODUCTS

CDER Oncology Group (HFD-150), Parklawn Building
5600 Fishers Lane, Rockville, MD 20857

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TO:	Dr. Burzynski	FROM:	Dianne Spillman (for Paul Zimmerman)
PHONE:	(713) 597-0111	PHONE:	(301) 594-5770
FAX:	(713) 597-1166	FAX:	(301) 594-0498

DATE: June 27, 1996

Total number of pages, including cover sheet: 1

COMMENTS: IND 43,742
Special exception treatment request
Burzynski Research Institute

Regarding your June 25, 1996 request (received by us on June 26) concerning Ramon Knier, we have the following comments:

It is not possible to distinguish clinical deterioration due to chemotherapy and/or radiotherapy side effects from that due to tumor progression until the patient has an opportunity to recover from the effects of the chemotherapy and/or radiotherapy. We do not have information at present to assess the patient's tumor status. If the patient is demonstrated to have progressive tumor, we will approve the application for compassionate exception.

RICHARD D KLATZCO

BURZYNSKI
RESEARCH INSTITUTE, INC.

November 18, 1997

FEDERAL EXPRESS AND FAX

Paul Zimmerman, CSO
FDA/CDER Oncology, HFD-150
1451 Rockville Pike
Rockville, MD 20852

RE: IND #43,742
Serial #868

Dear Mr. Zimmerman:

I am hereby submitting three copies of the application for Special Exception to the Protocol in IND #43,742 for Richard D. Klatzco. In the attached documents you will find a short patient history and a rationale for the treatment with Antineoplastons and an explanation why the patient does not fit under the original protocols under the IND.

This patient's treatment will be conducted according to Protocol HN-2. The Informed Consent Form will be the same as required by Protocol HN-2.

The patient will receive treatment under my care.

Sincerely,

Stanislaw R. Burzynski, M.D., Ph.D.

SRB/cf

12000 RICHMOND AVENUE • HOUSTON, TEXAS 77082-2431 • (281) 597-0111 • FAX (281) 597-1166

BURZYNSKI
RESEARCH INSTITUTE, INC.

APPLICATION FOR SPECIAL EXCEPTION
TO THE PROTOCOL IN IND #43,742
SERIAL #868

Patient's Name: KLATZCO, Richard D.

Diagnosis: Squamous cell carcinoma of the pyriform sinus, stage III

Sponsor: Burzynski Research Institute, Inc.

Principal Investigator: S. R. Burzynski, M.D., Ph.D.

Short Patient History

The patient is a 46 year old white male who noted a lump near the angle of the right jaw in April of 1997. Fine needle aspiration was performed on August 22, 1997 and the pathology was consistent with granulomatous inflammation. On August 29, 1997, he underwent excision biopsy of the right neck mass and the findings were showing metastatic poorly differentiated squamous cell carcinoma. The size of the mass was 6 x 3.5 x 2.5 cm. It was an encapsulated and irregularly shaped nodule. The ENT examination at this time was grossly normal. His staging was T1 N 2b MO, Stage IV. On September 4, 1997, he had an MRI scan of the neck showing right neck adenopathy with several enlarged up to 2.2 cm in length right neck lymph nodes in the spinal accessory and internal jugular nodal chains. On September 5, 1997, CT scan of the chest showed no evidence of chest mass or metastases. The possibility of chemotherapy and radiation therapy was discussed with the patient, but declined. On September 10, 1997, he underwent direct microlaryngoscopy with random biopsies of right pyriform and right base of the tonge and nasal endoscopy with biopsies of the nasopharynx. He tolerated the procedure well. The pathology report showed poorly differentiated predominantly non-keratinizing squamous cell carcinoma in the right pyriform sinus. No evidence of malignancy at the base of the tongue. No abnormality of the tongue. No evidence of malignancy in the nasopharynx. On October 3, 1997, MRI of the brain, face and neck showed pathologic lymphadenopathy with large right jugulodigastric node. No primary tumor mass was identified in the soft tissue of the neck. The size of the jugulodigastric node was 2.4 x 1.6 cm in the transaxial dimension and 3.3 cm in craniocaudal dimension. On October 22, 1997, the patient had CT of the head with normal ventricles. No abnormal intra or extra-axial masses or fluid collections were visualized. No abnormal enhancing lesions were present. CT of the neck revealed soft tissue fullness in the left fossa of Rosenmuller suspicious for neoplastic process and further workup was suggested. There was a large lymph node in the right carotid space which measured 1.8 cm x 1.0 cm. Bone scan of October 22, 1997, showed no scintigraphic evidence of metastatic disease. There were some benign left distal femoral lesions which were not compatible with metastatic disease. Chest x-ray on October 22, 1997 showed no evidence of intrathoracic metastases. Clear lungs and normal heart were identified. An MRI of the neck performed on November 5, 1997 showed an enhancing soft tissue mass in the right pyriform sinus which measured approximately 0.8 x 1.9 cm and right jugular adenopathy. The node measured approximately 2.0 x 2.7 cm. Otherwise, there was no significant adenopathy within the neck.

12000 RICHMOND AVENUE • HOUSTON, TEXAS 77082-2431 • (281) 597-0111 • FAX (281) 597-1166

APPLICATION FOR SPECIAL EXCEPTION
TO THE PROTOCOL IN IND #43,742, SERIAL #868
Patient's Name: KLATZCO, Richard D.
Page 2

Rationale for Treatment with Antineoplaston A10 and AS2-1

The patient has a pathology confirmed squamous cell carcinoma of the pyriform sinus. The enhancing mass in the right pyriform sinus measures less than 2cm in diameter and there is also one internal jugular lymph node which measures 2.0 cm x 2.7 cm. The patient was offered to receive radiation therapy, but he declined that option. The patient does not qualify for the admission to the study because his disease is not advanced enough to meet entrance criteria. Therefore, we are applying for Special Exception on behalf of the patient to be able to receive treatment according to Protocol HN-2.

S. R. Burzynski, M.D., Ph.D.

SRB/cf

DEPARTMENT OF HEALTH AND HUMAN SERVICES
PUBLIC HEALTH SERVICE
FOOD AND DRUG ADMINISTRATION
INVESTIGATIONAL NEW DRUG APPLICATION (IND)
(TITLE 21, CODE OF FEDERAL REGULATIONS (CFR) PART 312)

Form Approved: OMB No. 09100-0014.
Expiration Date: March 31, 1998.
See OMB Statement on Reverse.

NOTE: No drug may be shipped or clinical investigation begun until an IND for that investigation is in effect (21 CFR 312.40).

1. NAME AND ADDRESS OF INVESTIGATOR.
STANISLAW R. BURZYNSKI, M.D., Ph.D.

2. DATE OF SUBMISSION
11/18/97

3. ADDRESS (NUMBER, STREET, CITY, STATE, AND ZIP CODE)
12000 RICHMOND AVENUE, SUITE 260
HOUSTON, TEXAS 77082-2431

4. TELEPHONE NUMBER (Include Area Code)
(281) 597-0111

5. NAME(S) OF DRUG (Include all available names: Trade, generic, chemical, Code)
A10 INJECTIONS
AS2-1 INJECTIONS

6. IND NUMBER (If previously assigned)
#43,742

7. INDICATION(S) (Covered by this submission)

8. PHASE(S) OF CLINICAL INVESTIGATION TO BE CONDUCTED: ☐PHASE 1 ✓PHASE 2 ☐PHASE 3 ☐OTHER *Special Exception* (Specify)

9. LIST NUMBERS OF ALL INVESTIGATIONAL NEW DRUG APPLICATIONS (21 CFR Part 312), NEW DRUG OR ANTIBIOTIC APPLICATIONS (21 CFR Part 314), DRUG MASTER FILES (21 CFR 314.420), AND PRODUCT LICENSE APPLICATIONS (21 CFR Part 601) REFERRED TO IN THIS APPLICATION

IND # 43,742

10. IND submissions should be consecutively numbered. The initial IND should be numbered "Serial Number: 000." The next submission (e.g., amendment, report, or correspondence) should be numbered "Serial Number: 001." Subsequent submissions should be numbered consecutively in the order in which they are submitted.

SERIAL NUMBER:
#868

11. THIS SUBMISSION CONTAINS THE FOLLOWING: (Check all that apply)

☐ INITIAL INVESTIGATIONAL NEW DRUG APPLICATION (IND)

☐ RESPONSE TO CLINICAL HOLD

PROTOCOL AMENDMENT(S):
☐ NEW PROTOCOL
☐ CHANGE IN PROTOCOL
☐ NEW INVESTIGATOR

INFORMATION AMENDMENT(S):
☐ CHEMISTRY/MICROBIOLOGY
☐ PHARMACOLOGY/TOXICOLOGY
☐ CLINICAL

IND SAFETY REPORT(S):
☐ INITIAL WRITTEN REPORT
☐ FOLLOW-UP TO A WRITTEN REPORT

☐ RESPONSE TO FDA REQUEST FOR INFORMATION ☐ ANNUAL REPORT ☐ GENERAL CORRESPONDENCE

☐ REQUEST FOR REINSTATEMENT OF IND T HAT IS WITHDRAWN, INACTIVATED, TERMINATED OR DISCONTINUED

☒ SPECIAL EXCEPTION-RICHARD D. KLATZCO (Specify)

CHECK ONLY IF APPLICABLE

[redacted]

FOR FDA USE ONLY

CDR/DGD RECEIPT STAMP	DDR RECEIPT STAMP	IND NUMBER ASSIGNED
		DIVISION ASSIGNMENT:

FORM FDA 1571 (10/89) PREVIOUS EDITION IS OBSOLETE

TRANSMISSION REPORT

(TUE) NOV 18 1997 18:27
BURZYNSKI

DOCUMENT #	TIME STORED	TIME SENT	DURATION	PAGE(S)	MODE	RESULT
4260898-588	11. 18 18:24	11. 18 18:24	2' 10"	6	ECM	OK

DESTINATION	DST. TEL #
913018274590	913018274590

BURZYNSKI
RESEARCH INSTITUTE, INC.

Facsimile Cover Sheet

DATE: **TIME:**

To: Paul Zimmerman
Company: FDA Office of Drug Evaluation / Oncology Division
Phone: 301-594-5775
Fax: 301-827-4580

From: Sherry Ysais for Cheryl Owens, Clinical Trials Administrative Director
Company: SRB
Phone: (281) 597-0111, Ext. 113 / Private Line (281) 598-5301
Fax: (281) 493-5068 / (281) 597-1166

Pages including cover page:
Comments:

Dear Mr. Zimmerman:

The following are documents included in this fax:

Patient Name:	S/N	CIF / Add'l Info / Other
Klatzco, Richard D.		Serial #868

48 148

FOOD AND DRUG ADMINISTRATION
OFFICE OF DRUG EVALUATION I

DIVISION OF ONCOLOGY DRUG PRODUCTS

CDER Oncology Group (HFD-150), Parklawn Building
5600 Fishers Lane, Rockville, Maryland 20857

THIS DOCUMENT IS INTENDED ONLY FOR THE USE OF THE PARTY TO WHOM IT IS ADDRESSED AND MAY CONTAIN INFORMATION THAT IS PRIVILEGED, CONFIDENTIAL, AND PROTECTED FROM DISCLOSURE UNDER APPLICABLE LAW. If you are not the addressee, or a person authorized to deliver the document to the addressee, you are hereby notified that any review, disclosure, dissemination, copying, or other action based on the content of this communication is not authorized. If you have received this document in error, please immediately notify us by telephone and return it to us at the above address by mail. Thank you.

PHONE: (301) 594-5775

TO: Dr. Burzynski

(281) 597-1166 (or 493-5088)

FROM: Paul F. Zimmerman, CSO

Total number of pages, including cover sheet: 2

Date: November 25, 1997

COMMENTS: IND 43,742 (SN 874)
Special exception request
Burzynski Research Institute

Regarding your request dated November 24, 1997 (received by us on November 25), concerning Richard Klatzoo, we have the following comments.

The additional information provided in the report from the University of Chicago was evaluated. Our consultants, including and ENT surgeon and an oncologist, both with extensive experience in head and neck cancer, concur with the assessment provided, that this patient has a better than 50% chance for long term cure if therapy is initiated immediately. To delay for even a few weeks could lead to further tumor growth and a dramatic reduction in the possibility for a positive long term result. On that basis, it would neither be fair nor ethical to

permit this patient to enter onto an experimental study with and agent that has not been reported to ever result in a tumor response in a patient with head and neck cancer. The request therefore cannot be granted. The patient is most strongly encouraged to reconsider his opposition to the treatment being offered at the University of Chicago, and begin therapy at once. It should also be noted that the replies to this request were provided within 24 hours of receipt by the Division.

ZACHARY MCCONNEL

FOOD AND DRUG ADMINISTRATION
OFFICE OF DRUG EVALUATION I

DIVISION OF ONCOLOGY DRUG PRODUCTS

CDER Oncology Group (HFD-150), Parklawn Building
5600 Fishers Lane, Rockville, MD 20857

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PHONE: (301) 594-2473 FAX: (301) 594-0498

TO: Dr. S. Burzynski
Burzynski Research Institute
713 597-1166

FROM: Dotti Page for Paul Zimmerman

Total number of pages, including cover sheet: _____

Date: 6-14-96

COMMENTS: See attached

DEPARTMENT OF HEALTH & HUMAN SERVICES

Public Health Service

Food and Drug Administration
Rockville MD 20857

IND 43,742

CERTIFIED MAIL
RETURN RECEIPT REQUESTED

Burzynski Research Institute, Inc.
12000 Richmond Avenue, Suite 260
Houston, Texas 77082-2431

JUN 14 1996

Attention: S.R. Burzynski, M.D., Ph.D.

Dear Dr. Burzynski:

I am writing regarding Zachary McConnell, a patient you seek to enroll on Antineoplastons injections under your IND 43,742. This request was previously addressed in our letter to you dated May 23, 1996. In that letter, we advised you that based on the limited information you submitted, we could not conclude whether the administration of antineoplastons in this patient was appropriate. However, we permitted administration of the drugs to continue for 15 days, provided that you submitted to us specified additional information regarding the patient.

Those fifteen days expired on June 7, 1996; but we did not receive any additional information from you. We did receive limited information on the afternoon of June 7 from Mr. Jonathan Emord, an attorney representing the patient. We informed Mr. Emord that we must receive information directly from the IND sponsor seeking permission to administer the drug; and that the information we needed to make a final decision on this request was specified in our letter to you of May 23, 1996. We further informed Mr. Emord that we would extend the date by which we must receive the required additional information regarding this patient until June 24, 1996. We stated that "Administration of antineoplastons may not continue beyond that date unless we receive from the Burzynski Research Institute the information requested in our letter of May 23, 1996."

We acknowledge your June 13, 1996 communication to Catherine M. Cook, Esq., of the Food and Drug Administration, that was sent to her by facsimile on June 12, 1996. You identify this communication as your official response to our May 23, 1996 letter. Your June 13, 1996 communication contains the information that was previously provided by Jonathan W. Emord, Esq. in his June 7, 1996 facsimile communication to Dr. DeLap. We will include this communication in your IND 43,742. However, all future correspondence regarding IND matters must be sent by you to the IND, in accordance with regulations, as we have previously advised you.

IND 43,742
Page 2

The information provided in these submissions is not what we requested concerning Zachary McConnell, and is not adequate for us to conclude whether continued administration of Antineoplastons to this patient may be permitted. Our greatest concern is that if there is a standard therapy available that has a significant likelihood of curing this child, this standard therapy should not be abandoned in favor of administration of unproven investigational products. Your submission of the letter signed by Mr. Emord, and the affidavit of the patient's father regarding his understanding of his child's condition, has given us some further insight into this patient's illness but does not give us the information we need to address this concern.

Please be advised that if you wish to pursue your request to continue to administer antineoplastons to Zachary McConnell under your IND, you must provide us with additional information regarding this patient, no later than June 24, 1996. After considering the information you have provided to date, the additional information we require includes: (1) a statement from a physician with specialized training and experience in the treatment of pediatric brain tumors, indicating that failure to administer the radiation therapy to this patient as previously planned would not significantly affect his chances of being cured (considering the surgery and chemotherapy he has already received); and (2) copies of reports (signed by the attending radiologist) of the MRI scans that were performed on February 7, 1996 and April 26, 1996, and reports of any other MRI scans or CT scans that may have been performed between February 7 and April 26, 1996.

We appreciate the urgency of this situation, and we hope that you will promptly provide us with the information we need to ensure that this patient's care is not being compromised by the administration of Antineoplastons.

If you do not provide us with the information we need by June 24, then administration of antineoplastons to this patient must cease on that date.

Once again, we want to emphasize that this letter does not excuse any violations of law or failures to comply with the IND regulations that may have occurred.

Sincerely yours,

Robert J. DeLap

Robert J. DeLap, M.D., Ph.D.
Director, Division of Oncology Drug Products
Office of Drug Evaluation I
Center for Drug Evaluation and Research

IND 43,742
Page 2

The information provided in these submissions is not what we requested concerning Zachary McConnell, and is not adequate for us to conclude whether continued administration of Antineoplastons to this patient may be permitted. Our greatest concern is that if there is a standard therapy available that has a significant likelihood of curing this child, this standard therapy should not be abandoned in favor of administration of unproven investigational products. Your submission of the letter signed by Mr. Emord, and the affidavit of the patient's father regarding his understanding of his child's condition, has given us some further insight into this patient's illness but does not give us the information we need to address this concern.

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Once again, we want to emphasize that this letter does not excuse any violations of law or failures to comply with the IND regulations that may have occurred.

Sincerely yours, Robert J. DeLap

Robert J. DeLap, M.D., Ph.D.
Director, Division of Oncology Drug Products
Office of Drug Evaluation I
Center for Drug Evaluation and Research

DEPARTMENT OF HEALTH & HUMAN SERVICES

Public Health Service

Food and Drug Administration
Rockville MD 20857

IND 43,742

CERTIFIED MAIL
RETURN RECEIPT REQUESTED

Burzynski Research Institute, Inc.
12000 Richmond Avenue, Suite 260
Houston, Texas 77082-2431

JUN 24 1996

Attention: S.R. Burzynski, M.D., Ph.D.

RE: Special exception request for patient Zachary McConnell

Dear Dr. Burzynski:

We have received your June 20, 1996 letter (sent to us by facsimile transmission on June 21, 1996), which responds to our June 14, 1996 letter concerning Zachary McConnell. Your letter states that it will not be possible for you to provide the additional information we have requested by June 24; but you expect to be able to address our requests by July 10, 1996. You further state that it may not be possible to obtain an opinion from a physician specialized in the treatment of pediatric brain tumors, but you may be able to get the opinion of a neuro-oncologist who has reviewed the literature on PNET and has reviewed Zachary's case.

Again, our primary concern is that this child should receive the best possible care, to minimize the chances of recurrence of his malignancy.

We understand that PNET involving the brain is a rare malignancy, and it may take some additional time for you to obtain an authoritative opinion regarding the importance of radiation therapy in the treatment of this child. However, we are also concerned that if radiation therapy is needed to control Zachary's cancer, then withholding this treatment may not be in his best interest. In other forms of cancer, radiation therapy works best to eradicate cancer cells when the cancer is very small or microscopic. The worst possibility is that if Zachary's tumor has not been cured by the treatment he has already received, then withholding radiation therapy until there is evidence of tumor progression could substantially reduce his chances for cure. It is unclear why the opinion of an experienced radiotherapist was not obtained by you prior to beginning antineoplastons.

Since you are actively attempting to obtain the information we have requested, we will agree to the extension you have requested (to July 10, 1996). As you propose, we will accept a statement from a physician with specialized training and experience in the treatment of brain tumors who has reviewed the latest literature on treatment of PNET of the brain in children and the details of Zachary's case (indicating that failure to administer the radiation therapy to this child as previously planned would not significantly affect his chances of being cured, considering the surgery and chemotherapy he has already received). We will also look forward to receiving the radiologist's report you have promised, interpreting all of the brain scans performed between February 7 and June 21, 1996.

If you do not provide us with the information we need by the new date of July 10, 1996, then administration of antineoplastons to this patient must cease on that date.

Sincerely yours,

Robert J. DeLap

Robert J. DeLap, M.D., Ph.D.
Director
Division of Oncology Drug Products
Office of Drug Evaluation I
Center for Drug Evaluation and Research

JULIA MC MILLAN

BURZYNSKI
RESEARCH INSTITUTE, INC.

IND # 43,742
Serial # 304

VIA FEDERAL EXPRESS

October 25, 1996

Paul Zimmerman, C.S.O.
FDA/CDER Oncology, HFD-150
1451 Rockville Pike
Rockville, MD 20852

RE: MCMILLAN, Julia

Dear Mr. Zimmerman:

I am, hereby, submitting three copies of the application of compassionate exception to the protocol in IND # 43,742 for Julia McMillan. In the attached documents you will find a short patient history and rationale for the treatment with Antineoplastons and an explanation of why the patient does not qualify for the original protocol under the IND. This patient's treatment will be conducted according to Protocol BT-9. The informed consent form will be the same as provided by Protocol BT-9.

The patient will receive treatment under my care.

Sincerely,

S.R. Burzynski, M.D., Ph.D.

SRB/tdc
Enclosure

12000 RICHMOND AVENUE • HOUSTON, TEXAS 77082-2431 • (713) [redacted]

BURZYNSKI
RESEARCH INSTITUTE, INC.

APPLICATION FOR COMPASSIONATE EXCEPTION
TO THE PROTOCOL IN IND # 43,742
Serial # 304

Patient's Name:	MCMILLAN, Julia
Diagnoses:	1. Left frontal lobe glioma. 2. Diabetes mellitus. 3. Porphyria. 4. Glaucoma
Sponsor:	Burzynski Research Institute, Inc.
Principal Investigator:	S.R. Burzynski, M.D., Ph.D.

Short Patient History

Julia is a 71-year-old Caucasian female with multiple medical problems including brain tumor, diabetes mellitus, porphyria and glaucoma.

The patient fell in December of 1990 having a closed head injury. There was no loss of consciousness but she developed prolonged sleepiness since that day. She was eventually evaluated and subsequently discovered to have both adult onset diabetes mellitus eventually requiring insulin therapy for management. Her concurrent visual disturbances led to a recommendation for MRI of the brain which revealed a left frontal lobe lesion in March 1993. Her oncologist did not recommend chemotherapy or radiation at this time and instead she went to Holland to use homeopathic cellular balancing therapy consisting of ascorbic acid, amino acids, nicotinic acid, and Conium. She did well and repeated the therapy eight months later in April 1994. Her most recent MRI of the brain from September 16, 1996, revealed a large complex mass with both solid and cystic components in the left frontal lobe which appears to arise from the floor of the anterior cranial fossa near the midline. There is considerable edema and mass effect with this lesion and marked deformity of the ventricular system and anterior aspect of the corpus collosum and significant subfalcine herniation to the right. She has had no surgery, biopsy or chemotherapy.

It is proposed that the patient will receive treatment according to Protocol BT-9.

Rationale for Treatment with Antineoplaston A10 and AS2-1

Ms. McMillan has multiple medical problems including insulin dependent diabetes mellitus, porphyria and glaucoma. The protocol is exclusive of accepting patients with chronic disorders and for this reason request is made for Compassionate Exception.

S.R. Burzynski, M.D., Ph.D.

October 11, 1996

T:tdc

12000 RICHMOND AVENUE • HOUSTON, TEXAS 77082-2431 • (713) 597-0111 • FAX (713) 597-1166

DEPARTMENT OF HEALTH AND HUMAN SERVICES
PUBLIC HEALTH SERVICE
FOOD AND DRUG ADMINISTRATION
INVESTIGATIONAL NEW DRUG APPLICATION (IND)
(TITLE 21, CODE OF FEDERAL REGULATIONS (CFR) PART 312)

Form Approved: OMB No. 0910-0014.
Expiration Date: March 31, 1997.
See OMB Statement on Reverse.

NOTE: No drug may be shipped or clinical investigation begun until an IND for that investigation is in effect (21 CFR 312.40).

1. NAME AND ADDRESS OF INVESTIGATOR.
BURZYNSKI RESEARCH INSTITUTE

2. DATE OF SUBMISSION
October 25, 1996

3. ADDRESS (NUMBER, STREET, CITY, STATE, AND ZIP CODE)
12000 RICHMOND AVENUE, SUITE 260
HOUSTON, TEXAS 77082-2431

4. TELEPHONE NUMBER (Include Area Code)
(713) 597-0111

5. NAME(S) OF DRUG (Include all available names: Trade, generic, chemical, Code)
A10 INJECTIONS
AS2-1 INJECTIONS

6. IND NUMBER (If previously assigned)
#43,742

7. INDICATION(S) (Covered by this submission)

8. PHASE(S) OF CLINICAL INVESTIGATION TO BE CONDUCTED: ☐PHASE 1 ✓PHASE 2 ☐PHASE 3 ☐OTHER Compassionate Exception (Specify)

9. LIST NUMBERS OF ALL INVESTIGATIONAL NEW DRUG APPLICATIONS (21 CFR Part 312), NEW DRUG OR ANTIBIOTIC APPLICATIONS (21 CFR Part 314), DRUG MASTER FILES (21 CFR 314.420), AND PRODUCT LICENSE APPLICATIONS (21 CFR Part 601) REFERRED TO IN THIS APPLICATION

IND # 43,742

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SERIAL NUMBER:
304

11. THIS SUBMISSION CONTAINS THE FOLLOWING: (Check all that apply)

☐ INITIAL INVESTIGATIONAL NEW DRUG APPLICATION (IND)

☐ RESPONSE TO CLINICAL HOLD

PROTOCOL AMENDMENT(S):
☐ NEW PROTOCOL
☐ CHANGE IN PROTOCOL
☐ NEW INVESTIGATOR

INFORMATION AMENDMENT(S):
☐ CHEMISTRY/MICROBIOLOGY
☐ PHARMACOLOGY/TOXICOLOGY
☐ CLINICAL

IND SAFETY REPORT(S):
☐ INITIAL WRITTEN REPORT
☐ FOLLOW-UP TO A WRITTEN REPORT

☐ RESPONSE TO FDA REQUEST FOR INFORMATION ☐ ANNUAL REPORT ☐ GENERAL CORRESPONDENCE

☐ REQUEST FOR REINSTATEMENT OF IND T HAT IS WITHDRAWN, INACTIVATED, TERMINATED OR DISCONTINUED

☒ Compassionate Exception (Specify)

CHECK ONLY IF APPLICABLE

[redacted]

FOR FDA USE ONLY

CDR/DGD RECEIPT STAMP	DDR RECEIPT STAMP	IND NUMBER ASSIGNED
		DIVISION ASSIGNMENT:

FORM FDA 1571 (10/89) PREVIOUS EDITION IS OBSOLETE

November 15, 1996

Dr Burzynski:

I spoke with Julia McMillan this morning. She said she doesn't remember anyone telling her she needed to do anything to follow-up on her compassionate exception. However, I told her what the FDA requested. She asked me to relay to you that

1. She has reports in her charts from 4 neurosurgeons as well as the MRI's concerning her diagnosis of brain tumor.
2. She has Porphyria which restricts her from having anesthesia in order to have a biopsy. She has been told it would be deadly for her to have anesthesia. She is extremely sensitive regarding chemicals in her system.
3. She knows her brain tumor is malignant and she doesn't want to have surgery or biopsy as it may metastasize.

I told her I would give this information to you. She still wants to have treatment. These reasons are why she came here for your treatment.

Cheryl

Please refer to your October 25, 1996 requests (received by us on October 28, 1996) concerning Edward J. Flowers (303) and Julia McMillan (304). See attached notes.

Edward J. Flowers (303)
We request independent consultation supporting your assertion in your facsimile transmission dated October 25, 1996, that the 1996 lesion is consistent with malignancy (i.e. from a neurologist or neurosurgeon).

Julia McMillan (304)
1) Why has the lesion not been biopsied?
2) We require a biopsy or independent consultation from a neurologist or neurosurgeon that confirms the current lesion represents a malignant brain tumor.

Please remember that patients approved by the FDA to receive Antineoplastons as special exceptions to your study protocols are still under your IND(s). As the sponsor and the clinical investigator, you must insure that they sign informed consent (21 CFR Part 50); each special exception patient must be approved, prior to treatment, by the IRB that is responsible for review of these clinical investigations (21 CFR Part 56); and all other IND regulations must be followed. Also, these patients cannot be charged for the Antineoplastons without prior written FDA approval.

1) B/C it is Mal. + doesn't want it cut into + possibly hetastasizing -
2) Says she has this

6679706.

Went to 4 neurosurgeons as well as MRI's. Married to Neuro. Prof. @ UVA -

we have reports

Porphyria

Porferia - Missing enzyme in liver + is ultra sensitive to chemicals Anesthesia is dangerous for her.

FOOD AND DRUG ADMINISTRATION
OFFICE OF DRUG EVALUATION I

DIVISION OF ONCOLOGY DRUG PRODUCTS

CDER Oncology Group (HFD-150), 5600 Fishers Lane
Rockville, Maryland 20857

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PHONE: (301) 594-2473 FAX: (301) 594-0499

TO Dr. Burzynski

713 597-1166

FROM: Patrick Quinn, CSO/Project Manager

Total number of pages, including cover sheet: 2

Date: November 21, 1986

COMMENTS: IND 43,742
Special exception treatment request (335)
Burzynski Research Institute

Please refer to your November 18, 1996 request (received by us on November 20, 1996) concerning Julia McMillan (335). See attached notes.

1. What type of porphyria?

2. As stated in our facsimile transmission dated October 28, 1996, we require independent consultation from a neurologist or neurosurgeon that confirms that the current lesion represents a malignant brain tumor.

Please remember that patients approved by the FDA to receive Antineoplastons as special exceptions to your study protocols are still under your IND(s). As the sponsor and the clinical investigator, you must insure that they sign informed consent (21 CFR Part 50); each special exception patient must be approved, prior to treatment, by the IRB that is responsible for review of these clinical investigations (21 CFR Part 56); and all other IND regulations must be followed. Also, these patients cannot be charged for the Antineoplastons without prior written FDA approval.

JANICE MILLER
DAVID SMITH
ZACHARY MCCONNEL

DEPARTMENT OF HEALTH & HUMAN SERVICES

Public Health Service

Food and Drug Administration
Rockville MD 20857

IND 43,742

CERTIFIED MAIL
RETURN RECEIPT REQUESTED

MAY 23 1996

Burzynski Research Institute, Inc.
12000 Richmond, Suite 260
Houston, Texas 77082-2431

Attention: S. R. Burzynski, M.D., Ph.D.

Dear Dr. Burzynski:

Please refer to your Investigational New Drug Application (IND) submitted pursuant to section 505(i) of the Federal Food, Drug, and Cosmetic Act for Antineoplastons A10 & AS2-1 Injections.

Please also refer to your April 22, 26, 30, May 2, 7, 8, 10, 13, 14, 15, and 17 1996 submissions and our April 16 and 26, 1996 letters.

We are concerned about the large number of new patients that you have now revealed were started on Antineoplastons between February 24, 1996 and April 15, 1996 outside your INDs and outside of the many new protocols you had filed to your INDs. At that time, while we were doing our best to work with you on your many new IND protocols, you did not advise us that you were also continuing to start new patients on Antineoplastons outside of your INDs.

This letter responds to your requests regarding some of these patients. We want to emphasize that this letter does not excuse any violations of law or failures to comply with the IND regulations that may have occurred.

1. Regarding Susan Duban:

   Antineoplastons may be continued in this patient with Stage IV neuroendocrine carcinoma of the lung provided the infusions are tolerated and the patient is monitored carefully for toxicity.

2. Regarding Albert Frolander:

   Antineoplastons may be continued in this patient with carcinoma of the prostate provided the infusions are tolerated and the patient is monitored carefully for toxicity.

3. Regarding Zachary McConnell:

According to the information submitted, this patient with a PNET involving the right cerebral hemisphere of the brain received chemotherapy followed by only one day of radiation treatment. The parents of the patient decided not to continue radiation because the patient became "very sick" from the radiation therapy and there were "symptoms of increasing intracranial pressure". Antineoplastons were begun on March 19, 1996, only 14 days following the radiation therapy. No information is provided regarding the status of the brain tumor following surgery, following chemotherapy, or following radiation therapy.

Based on the limited information submitted, we cannot conclude whether the administration of Antineoplastons in this patient is appropriate. However, Antineoplastons may be continued in this patient provided you submit the following information within 15 days of this notification: the age of the patient, a copy of CT scan and/or MRI report providing evidence that the patient has a residual malignant brain tumor and a copy of a report from the patient's radiation oncologist indicating the tumor is not potentially curable with radiation therapy.

4. Regarding Patricia McPherson:

This is a woman with Stage IV breast cancer who had previously received CMF chemotherapy and is currently receiving tamoxifen which was started on January 4, 1996. The status of this patient's breast cancer prior to beginning Antineoplastons on April 2, 1996 is not adequately described in the submission.

Based on the submitted information, we cannot conclude whether the administration of Antineoplastons in this patient is appropriate. However, Antineoplastons may be continued provided you submit the following information within 15 days of this notification: reports of appropriate diagnostic studies, including radiological studies, providing objective evidence that the cancer in this patient was progressing on tamoxifen prior to beginning Antineoplastons.

5. Regarding Janice Miller:

This patient has squamous cell carcinoma of the head and neck region with involvement of the cervical lymph nodes and oral mucosa. Resection of the primary lesion at the base of the tongue was performed on April 3, 1996, "followed by the development of metastatic nodule on the right buccal mucosa". Antineoplastons were begun on April 15, 1996, only 12 days following surgery. The patient evidently had not received chemotherapy or radiation therapy. It is unclear from the scanty amount of submitted information whether or not this patient is potentially curable with standard therapy including radiation therapy.

Continued administration of Antineoplastons in this patient is not acceptable. However, we would reconsider a request to continue Antineoplastons if you provide the following information: a detailed description of the tumor and metastases prior to beginning Antineoplastons, including measurements and location of each lesion; and a written report by a certified radiation oncologist indicating that this patient is not potentially curable with radiation therapy.

6. Regarding Patricia Patroski:

This is a patient with breast cancer who underwent a lumpectomy and axillary node dissection in February 1996. Twenty-two axillary nodes were "inspected and reported for cancer"; the number of lymph nodes that were positive is not provided. The patient has been on tamoxifen since April 1, 1996 and Antineoplastons were administered orally beginning on March 19, 1996.

The administration of Antineoplastons as adjuvant therapy in patients with breast cancer is not acceptable. The administration of Antineoplastons in this patient should not be continued.

7. Regarding David Smith:

This is a patient with Hodgkin's disease who previously received "curative" radiotherapy but now has recurrent disease with involvement of bone and bone marrow. He has not received chemotherapy.

The administration of Antineoplastons to patients with Hodgkin's disease who have not received potentially curative chemotherapy is not acceptable. The administration of Antineoplastons in this patient should not be continued.

8. Regarding Devin Frable:

Administration of Antineoplastons may continue, if the patient had documented residual, recurrent or metastatic disease at the time administration of Antineoplastons was initiated.

9. Regarding Jason Homer:

Administration of Antineoplastons may continue, if the patient had documented residual, recurrent or metastatic disease at the time administration of Antineoplastons was initiated.

10. Regarding Patrick McLoughlin:

Administration of Antineoplastons may continue, if the patient had documented residual, recurrent or metastatic disease at the time administration of Antineoplastons was initiated.

11. Regarding Adela H. Koffman:

Administration of Antineoplastons may continue.

As you are aware, you must obtain written informed consent from all patients receiving Antineoplastons; and you must obtain IRB approval, prior to treatment, for each patient who receives Antineoplastons as a single-patient protocol exception (including the patients listed above). As the IND sponsor, you must notify each of the subinvestigators of our recommendations regarding the patients listed above.

As sponsor of this IND, you are responsible for compliance with the Federal Food, Drug, and Cosmetic Act and the regulations promulgated thereunder. Those responsibilities include reporting any unexpected fatal or life-threatening experiences by telephone to this Agency no later than three

working days after receipt of the information and the submission of annual progress reports.

If you have any questions concerning IND 43,742 please contact:

Paul Zimmerman
Project Manager
(301) 594-5775

Sincerely yours,

Robert J. DeLap

Robert J. DeLap, M.D., Ph.D.
Acting Director
Division of Oncology Drug Products
Office of Drug Evaluation I
Center for Drug Evaluation and Research

cc:
Carlton Hazlewood, Ph.D.
Chairman
Institutional Review Board
Burzynski Research Institute, Inc.
12000 Richmond, Suite 260
Houston, Texas 77082-2431

PATRICIA C. WALTER
3183 WESTON STREET
PHILADELPHIA, PA 19136-1811
215-338-4374
215-332-8849 fax

March 30, 1998

Congressman Dan Burton
US House of Representatives
Washington, DC 20510

Dear Congressman Burton:

I am following up on a letter I sent to you on January 30, 1998 regarding my husband who has had stage IV renal cell cancer for 9 months. Thanks to Dr. Stanislaw Burzynski of Houston, Texas:

- My husband is alive and well, when he was given 6 months to live in June 1997.
- As of November 17, he no longer is taking 60 mg of morphine three times a day.
- He has few side effects.
- The tumor is reduced by 50%.
- He has had no occurrence of metastases elsewhere.
- He has not been ill or hospitalized once in all these months.

I have been a medical editor for years, and therefore, I **know** no cure exists for stage IV metastatic kidney cancer. Yet, it seems my husband is being cured. **Therefore, I believe these results are profound.** Furthermore, my husband has not had chemotherapy or radiation therapy. The only treatment he has had is Dr. Burzynski's antineoplaston therapy. All of these things have been documented in an FDA-approved clinical trial in which my husband is enrolled.

I am writing to ask that you support Dr. Burzynski's drugs for a fast track approval by the FDA.

Antineoplastons have far exceeded the 10% response rate required for fast tract approval. However, the FDA is still stonewalling Dr. Burzynski. I am very concerned that antineoplastons will never be available to the public if someone such as yourself, who truly can make a difference, does not step forward. Please encourage the FDA to put these drugs on a fast track for approval.

As you can see by the enclosed information, Dr. Burzynski has 18 years of clinical proof of the effectiveness and safety of antineoplastons. He is currently conducting 71 clinical trials using antineoplastons to treat only terminally ill cancer patients. It is common practice for the FDA to approve drugs despite clinical trials being incomplete, especially drugs for cancer and AIDS. One recent example is xeloda for breast cancer. This is the purpose of the fast tract approval process.

Approval of these drugs will also remove the financial burden from dying patients and their families. My husband and I have been fighting our insurance company since September. We have spent over $75,000 for treatment and travel expenses.

March 30, 1998
Congressman Dan Burton
Page 2 of 2

Personal Choice is willing to pay for chemotherapy, **which does not work**, and for radiation therapy, **which does not work**. However, it will not pay for a therapy that is working. Dr. Burzynski's treatment is one-third the cost of chemotherapy and radiotherapy.

Furthermore, despite the fact that renal cell carcinoma is refractory to chemotherapy and radiation therapy, doctors offer both, without regard to the serious medical consequences for and debilitating effects on the patient. Although all the textbooks state that these treatment modalities **do not work**, doctors disregard the advice of the most reputable men in the field.

No one is questioning the needless and harmful use of these therapies, not to mention the astronomical costs incurred by insurance companies and the families of dying patients. It is time to stop torturing cancer patients and look in a new direction for an alternative treatment for cancer.

My husband deserves better than this from his government, that is, the FDA. He is a decorated Vietnam combat veteran. He fought in an unpopular war and has suffered the consequences for his entire life. He served on the Philadelphia Fire Department for 20 years and experienced a heart attack while fighting a fire, thus ending his career. During his career, he saved lives and millions of dollars in personal and commercial property. Now, no one will help him.

Please support Dr. Burzynski's efforts to cure patients the medical establishment has given up on. Thank you.

Sincerely,

Patricia C. Walter

Patricia C. Walter

enclosures

1970s-1990s Over 100 papers published on antineoplastons by Dr. Burzynski (Dr. B) in peer-reviewed journals. Hundreds more published in peer-reviewed journals by independent investigators.

1977 Dr. B published the results of his first group of terminally ill patients treated with injections of the original broad-spectrum peptide, Antineoplaston A, in the peer-reviewed journal *Physiological Chemistry and Physics.* Most of the 22 patients achieved either significant tumor reduction or stabilization; 4 had complete remissions; 2 had reductions of 50% or more; and in 4 patients the cancer progressed.

1984 The FDA met with Dr. B in their Rockville, MD, headquarters and brought in 10 doctors to evaluate slides and laboratory analyses in regard to how well the drug worked in the test tube and in mice. They were shown the patient case histories. All 10 were amazed.

1980s, 1990s Successful tests of antineoplastons were performed at the University of Kurume in Japan, where the drug has been used for years to treat liver and kidney cancers, including a cure of liver cancer in that university's president.

1991 **Dr. Nicholas Patronas, chief of the neuroradiology section at the NIH, a specialist in reading scans and radiographs of cranial tumors, was sent as part of a panel from the NCI to evaluate some of Dr. B's patient case histories. Of the cases he examined, Patronas testified, "It's amazing, the fact that they are living and doing so well.... These particular individuals not only survived, but they didn't have major side effects. I think it is impressive and unbelievable." Patronas also said that he had never seen any treatment work so well against brain cancers.**

1997 **Dr. Robert E. Burdick, a Seattle oncologist and faculty member of the highly respected University of Washington Medical School,** evaluated both conventional treatments and 17 of Dr. B's patients who were undergoing treatment for brain tumors. He said, "It is rare, currently, to ever get a complete remission or cure in... malignant brain tumors, using our standard modalities of surgery, radiation, and chemotherapy.... As a rough estimate, neurosurgeons do well to cure one in every 1000 patients.... Radiation therapy slows the growth in tumors in adults, gaining perhaps 1 month of life, and again may result in a cure in only one in 500-1000 patients, those cures being in the pediatric age group. Despite 30 years of clinical trials, chemotherapy has not resulted in the development of a single drug or drug combination that elicits more than an occasional transient response in primary brain tumors."
Against this dismal background Burdick evaluated Dr. B's cases. Of the 17, there were 7 complete remissions. There were 9 partial remissions of 50% or more, and one case of stable disease. Burdick summarized that **"The responses here also are far in excess of any prior series of patients published in the medical literature...the response rate here is an astounding 81%, with an equally astounding 35% complete remission rate. Such remission rates are far in excess of anything I or anyone else has seen since research on brain tumors began. It is very clear the responses here are due to antinoeplaston therapy and are not due to surgery, radiation, or standard chemotherapy."**

1996 Among brain tumor patients in Dr. B's CAN-1 trial, of 45 patients 19 achieved reductions of 50% or more in their tumors, 19 displayed smaller tumor reductions or stable disease, and only 7 had tumor growth. Most of these patients had already failed conventional therapy and had been told that there was no treatment available that could stop their tumors from growing. That is, they were terminally ill.

1996 Dr. B sent scans and X-rays from 28 patients with brain tumors for independent evaluation by Dr. Bruce Dean, a partner in the widely respected radiology firm, Southwest NeruoImaging in Phoenix, Arizona. Dean confirmed a complete response in 13 of 28, found a partial response of 50% or more tumor reduction in another 3 patients, and initial improvement but later recurrence in 5 patients. Eight patients suffered continued, progressive disease, with no apparent benefit.

Tax ID #76-0301750
Medicare CI 32

STANISLAW R. BURZYNSKI, M.D., PhD
12000 RICHMOND SUITE #280
HOUSTON TX 77082

PHONE (281) 597-0111
FAX (281) 597-1166

DATE _____ TIME _____ PROTOCOL _____ DATE OF LAST SCAN _____

PATIENT NAME Walter, C CONFERENCE WITH _____

TYPE RADIOLOGY VIEWS _____

7/18/98 49 RCCA PROFESSIONAL SERVICES RECORD

1	1.9 x 1.5 = 2.85 → prob single vs non mult			
2	3.7 x 1.7 = 6.29			
≠ (r) adrenal gland mass, d/ – px proven ⊖				
	9.14			
7/28/97	9/29/97	11/4/97	12/19/97	2/16
2 x 1.7 = 3.4	1.6 x 1.3 = 2.05	1.3 x 1 = 1.3	1.1 x 1 = 1.1	1² = 1
10 – 3.4 = 7.6	5.4 – 2.05 = 3.6	8 – 1.3 = 6.8	7.6 – 1.1 = 6.5	6.29 – 1 = 5.29

BL 30%

Prior 19%

CASE MANAGEMENT AND PROLONGED SERVICES
(WITHOUT DIRECT PATIENT CONTACT)

99354	PROLONGED EVALUATION AND MANAGEMENT SERVICE BEFORE OR AFTER DIRECT PATIENT CARE (eg. REVIEW OF EXTENSIVE RECORDS AND TESTS, COMPARISON OF PREVIOUS AND CURRENT MR'S AND SCANS, etc.)	99361	MEDICAL CONFERENCE WITH OTHER HEALTH PROFESSIONALS - APPROX 30 MIN
99355	EACH ADDITIONAL 30 MINUTES	99362	MEDICAL CONFERENCE WITH HEALTH PROFESSIONALS-PROFESSIONAL SERVICES, REVIEWING RECORDS AND REPORTS - APPROX 60 MINUTES,
99212	FOLLOW-UP VISIT- LOW COMPLEXITY TO DISCUSS TESTS		

PROVIDER OF SERVICE _____ DATE _____

WHITE ORIGINAL- CHART YELLOW COPY- BILLING PINK COPY- NURSING

Michael Nussbaum, M.D., F.A.C.S.
General Surgery • Surgical Oncology

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Philadelphia, Penna. 19152

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January 14, 1998

MEDICAL DIRECTOR
PERSONAL CHOICE
BLUE CROSS BLUE SHIELD
CAMP HILL, PA

RE: CHARLES R. WALTER

DEAR DOCTOR:

I INITIALLY SAW MR. WALTER IN JUNE OF 1997 AT HOLY REDEEMER HOSPITAL AND A RETROPERITONEAL EXPLORATION WAS PERFORMED ON JULY 1, 1997. THE DIAGNOSIS ON DISCHARGE WAS RENAL CELL CARCINOMA.

SINCE CONVENTIONAL TREATMENT DID NOT OFFER THE PATIENT MUCH PROMISE HE CHOSE TO SUBMIT TO AN ALTERNATE TREATMENT.

FROM HIS TEST RESULTS IT WOULD APPEAR THAT HE HAS HAD A POSITIVE RESPONSE FROM THIS MODE OF TREATMENT. I WOULD RECOMMEND THAT MR. WALTER CONTINUE THIS TREATMENT.

SINCERELY,

MICHAEL NUSSBAUM, M.D.

***Cancer: Principles and Practice of Oncology*, 5th edition**
Edited by Vincent T. deVita, Jr., Samuel Hellman, and *Steven A. Rosenberg*
Philadelphia: Lippincott-Raven, 1997

This is the definitive book on cancer therapy in traditional medicine. Steven A. Rosenberg is a prominent scientist at the National Cancer Institute who is treating patients with renal cell carcinoma, and with malignant melanoma, using interleukin-2 immunotherapy. There is only a 3% response rate with IL-2. More patients die from the treatment than are cured by it. My husband, who has stage IV renal cell carcinoma, is ineligible for the trial for two reasons. First, he has an abnormal stress cardiac examination owing to a heart attack he suffered fighting a fire while employed as a firefighter by the City of Philadelphia. Second, he has tumor involvement of the central nervous system.

In Chapter 65 entitled "Kidney and Ureter" by Simons and Marshall of Johns Hopkins University Hospital, the authors state the following concerning renal cell carcinoma:

"The very best approach to the fully informed patient with metastatic disease is to recommend an investigational treatment protocol whenever possible."

"...the **poorly differentiated** and sarcomatoid tumors have a particularly unfavorable prognosis...and **large, high grade tumors** and particularly sarcomatoid variant tumors have an exceptionally poor prognosis."

These features describe my husband's tumor type.

From *Current Practice of Medicine* published by Current Medicine in Philadelphia:

"Various systemic treatments have been used for this disease [renal cell carcinoma], including standard chemotherapy and biologic response modifiers. Clinical benefits are uncommon, and the impact on disease control rates and survival is minimal at best. This tumor is usually relatively radioresistant, and radiation therapy is mostly used for palliative purposes (pain control, brain metastasis)."

TABLE 1.
Cost-to-Benefit Analysis of Conventional Therapy vs FDA-Approved Clinical Trial of Antineoplaston Therapy in Renal Cell Cancer

COSTS	CHEMOTHERAPY	ANTINEOPLASTON THERAPY
Administration of drugs	$2000+/day	$450/day
Hospitalizations during drug administration	$2000+/day	0 (Not necessary)
Hospitalizations for life-threatening treatment-related side effects, along with testing, medications, consultations, and so on	$2000+++/day	0 (None)
Cost of drugs to counteract the many side effects of chemotherapy and radiation therapy	$$$	Minimal (for water pills, drug therapy for high cholesterol, and mineral supplements)
Cost of long-term side-effects	$$$	0 (There are none)
Costs of nursing care at home	$$$	0 (We provide care for infusions and Hickman catheter)
Costs of hospice care	$$$	0

TABLE 2.
Cost-to-Benefit Analysis of Side Effects of Traditional Therapy and FDA-Approved Clinical Trial of Antineoplaston Therapy in Renal Cell Cancer

Vinblastine is the recommended chemotherapeutic agent. Combination therapy is not recommended outside of a new clinical trial.

CHEMOTHERAPY

Minor Side Effects:

Nausea
Vomiting
Pain at the IV site
Hair loss
Headache
Mouth sores
Loss of appetite
Stomach pain
Skin rash
Mental depression
Deep ache in jaw or throat, resulting from facial nerve being affected

Serious Side Effects:

Neurological damage*:
Constipation
Tingling and numbness of fingers and toes
Inability to complete sentences
Inability to do simple arithmetic
Inability to recall words
Hearing loss
Loss of deep tendon reflexes and increased motor weakness causing foot or wrist drop, difficulty walking, difficulty arising from chairs, clumsiness, and loss of coordination

ANTINEOPLASTON THERAPY

Side Effects:

Fatigue
Water retention
High cholesterol levels

RADIATION THERAPY

Minor Side Effects:

Fatigue
Skin burns
Loss of appetite
Wasting
Depression
Nausea
Diarrhea

Serious Side Effects:

Low leukocyte and low platelet counts, resulting in susceptibility to life-threatening infections

Radiotherapy can actually stimulate tumor growth

---

*Signs of neural drug toxicity, a potentially serious and nonreversible condition when allowed to progress.

NOTES:

Because current radiosensitizers face the same inherent drug-resistant phenotypes as do chemotherapeutic agents, radiation is offered for palliation of specific symptoms, for example, bone pain and brain metastases.

Chemotherapy with vinblastine can last up to 2 years, if the patient survives. In some patients, the neurological defects are permanent.

TABLE 3.
Traditional Chemotherapy and Radiotherapy vs Antineoplaston Therapy

Over the course of the disease (cancer) **CHEMOTHERAPY and RADIATION THERAPY**, including repeated hospitalizations and medications to counteract their detrimental and often life-threatening effects, can cost ***over $1 million.***	**ANTINEOPLASTON THERAPY** costs $470/day. Over 2 years, the cost is ***under $350,000***.
Millions of deaths have occurred from short- and long-term effects of chemotherapy and radiotherapy.	**Not one person has been injured or killed** by antineoplaston therapy in 18 years of use in humans.
As a medical editor, I know studies exist to show these therapies have **no permanent effect on solid tumors in most types of cancer and, in fact, do not prolong life. Moreover, these therapies have been shown to destroy the quality of life.** However, doctors continue to offer both without making patients aware of these facts.	Antineoplaston therapy improves the quality of life for patients with terminal cancer, reduces pain, and extends life. Yet doctors do not tell their patients of this alternative therapy.
Not only are doctors recommending these therapies that do not work, that make patients weaker and sicker, and that are proved not to extend life, but insurance companies pay for these therapies. **Not only do they pay, but they pay without question, despite the *published evidence* that these therapies do not work.**	Insurance companies will not pay for so-called investigational trials involving antineoplaston therapy, although Dr. Burzynski has been treating patients with antineoplastons for over 18 years. **The US government has admitted that these drugs are safe and effective, the two criteria that are used to judge drugs for FDA approval. All insurance companies in the United States and the FDA are well aware of these facts.**
Medical journals are financed by drug companies, as are the societies that publish the journals. These prestigious journals routinely publish articles touting the minor improvements in various regiments of chemotherapy and radiotherapy. These peer-reviewed journals **cannot** be impartial because they are **directly** funded by large pharmaceutical companies.	Dr. Burzynski has no funding and never has had funding outside of his position at Baylor College of Medicine and being offered a position, which he turned down, at MD Anderson Cancer Center. **The costs of antineoplaston therapy are borne by his sick and dying patients and their families.**
Drug-company and federal funding is available for FDA-approved clinical trials into new harmful chemotherapy drugs (based on the theory of destroying cells), or new combinations of old, harmful drugs.	Dr. Burzynski has no funding for his FDA-approved clinical trials because FDA tactics, such as raiding dying patients' homes, have frightened away investors. Investors know of the FDA's retaliatory tactics.

Table 3, continued

The result of chemotherapy and radiotherapy is death.	**The result of antineoplaston therapy is good-quality life.**
Lawsuits are routinely filed against doctors and hospitals for malpractice on behalf of cancer patients.	In the over 18 years that Dr. Burzynski has been treating dying patients with antineoplastons, **he has never been sued by a patient.**
Traditional research into new chemotherapeutic agents has lower standards for approval than do antineoplastons.	The FDA is forcing some patients who show improvement off of treatment in order to skew the results against antineoplastons.

PATIENT ACCESS TO ALTERNATIVE TREATMENTS: BEYOND THE FDA

THURSDAY, FEBRUARY 12, 1998

COMMITTEE ON GOVERNMENT REFORM AND OVERSIGHT,
HOUSE OF REPRESENTATIVES,
Washington, DC.

The committee met, pursuant to notice, at 1:18 p.m., in room 2154, Rayburn House Office Building, Dan Burton (chairman of the committee) presiding.

Present: Representatives Burton, Mica, Davis of Virginia, LaTourette, Sessions, Snowbarger, Waxman, Condit, Sanders, Maloney, Norton, Kucinich, and Tierney.

Also present: Representative DeFazio.

Staff present: Kevin Binger, staff director; Daniel Moll, deputy staff director; William Moschella, deputy counsel and parliamentarian; Judith McCoy, chief clerk; Teresa Austin, assistant clerk/calender clerk; Laurie Taylor and Carolyn Hicks, professional staff members; Will Dwyer, director of communications; Ashley Williams, deputy director of communications; Robin Butler, office manager; Ashley Godwin, staff assistant; Phil Schiliro, minority staff director; Phil Barnett, minority chief counsel; Cherri Branson, minority counsel; Karen Lightfoot, minority professional staff member; and Ellen Rayner, minority chief clerk.

Mr. BURTON. Representative Waxman is recognized.

Mr. WAXMAN. Mr. Chairman, I know you're going to proceed to opening statements, and I have an opening statement which I'd like to have inserted in the record by unanimous consent.

Let me apologize to those who are going to be testifying at the hearing today. I, unfortunately, have a conflict on a similar and related matter. The Health and Environment Subcommittee, which I once chaired, of the Commerce Committee is holding a hearing today on the question of human cloning, what legislation would be appropriate and whether such legislation might stop very promising health research that could lead to new therapies. So, I will certainly examine the testimony of all the Members, and I look forward to learning about the discussion. This is an important hearing that you're holding and I appreciate your willingness to let me make my statement now.

Mr. BURTON. Thank you, Mr. Waxman.

[The prepared statement of Hon. Henry A. Waxman and the prepared letter of Hon. Eleanor Holmes Norton follow:]

Statement of Rep. Henry A. Waxman
February 12, 1998

Mr. Chairman, today we are going to hear from a number of witnesses, some of whom have fought hard battles against cancer. I know my colleague Jim Moran has experienced the devastating impact of this disease on his family when his youngest child was struck ill with cancer, and I know how hard they have fought for her recovery. I have the greatest sympathy for his family and for all families who have fought against cancer. I appreciate their willingness to share their stories and I hope that they will succeed in defeating the disease.

I understand that cancer patients need a variety of medical treatments and that they desire not only conventional treatment but also experimental drugs. But cancer patients need more than simply access to experimental drugs. They need to have those drugs property tested and to learn whether or not they work. No one wants to waste money on treatments that don't work. No one wants to forego treatments that might be more effective. And if something does work, it should be made available to as many patients as possible.

The issue of access to unapproved drugs has far reaching implications -- not only for those who are ill today but also for those who become ill in the future. And that is why this issue was taken so seriously and studied so extensively by another committee of Congress, the Commerce Committee, which has primary jurisdiction over the Food and Drug Administration. The issue of access to unapproved treatments was considered by the Commerce Committee as they worked over the last three years to craft bipartisan legislation to reform the FDA -- legislation that was signed two months ago. As part of that process, some of the witnesses here today testified before the Commerce Committee.

I am disappointed that this Committee is now holding a second hearing on FDA issues without giving FDA the opportunity to testify. I would like to hear how they would respond to many of the issues that will be raised by witnesses today. I think it would have been appropriate to have had an opportunity to hear from the agency and get a full perspective of the issues.

I would like to welcome the witnesses and apologize for not being able to be present for the whole hearing. Unfortunately there is a concurrent hearing at the Commerce Committee which I must also attend.

ELEANOR HOLMES NORTON
DISTRICT OF COLUMBIA

COMMITTEE ON
TRANSPORTATION AND
INFRASTRUCTURE

SUBCOMMITTEES
SURFACE TRANSPORTATION
PUBLIC BUILDINGS AND
ECONOMIC DEVELOPMENT

Congress of the United States
House of Representatives
Washington, D.C. 20515

COMMITTEE ON
GOVERNMENT REFORM AND
OVERSIGHT

SUBCOMMITTEE
RANKING MINORITY MEMBER,
DISTRICT OF COLUMBIA

CIVIL SERVICE

CO-CHAIR
CONGRESSIONAL CAUCUS FOR
WOMEN'S ISSUES

February 9, 1998

Honorable Dan Burton
Chairman
Committee on Government Reform and Oversight
United States House of Representatives
2185 Rayburn House Office Building
Washington, D.C. 20515

Dear Dan:

On February 12, 1998, I will be unavoidably detained outside of Washington. I understand that on the same day, the Committee will be holding its second day of hearings on "Patient Access to Alternative Treatments: Beyond the FDA." I will therefore be unable to attend the hearing and would appreciate your making this explanation a part of the official committee record.

Sincerely,

Eleanor

Eleanor Holmes Norton

cc: Honorable Henry Waxman

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Recycled Paper

Mr. BURTON. A quorum being present, the Committee on Government Reform and Oversight will come to order.

Our first panel will be Representative Peter DeFazio, Representative Jim Moran and Georgia State Senator Ed Gochenour, I hope I pronounced that correctly.

As I understand it, the first panel is on its way so we'll proceed with opening statements and then we'll get to the panel as they arrive.

Today is the second in a series of hearings examining issues and problems related to alternative medical treatment for millions of desperately ill Americans. Last week we heard from panels of patients and experts who discussed what they saw as the failures of the Food and Drug Administration and the National Cancer Institute. Today, we will hear potential solutions to the problems that have been brought before us, one in the form of legislation that would give all Americans the freedom to choose their own medicine.

As I stated last week, the purpose in holding these hearings is to lay the issues out on the table and deal with them in the most reasonable and balanced way. I want to stress that in no way is this a partisan issue. Dedicated Members from both parties have raised concerns about patient access. We owe it to the millions of patients, their families and loved ones who are not satisfied with conventional treatments to give our time, our energy and attention to this important issue.

It was mentioned last week by members of this committee that these hearings are not necessary, that these issues have been heard before and that the FDA Modernization Act has been passed to fix these problems. However, we heard last week also that serious problems still exist, that these issues are still tremendously important to Americans and that the FDA Modernization Act fails to properly address patient access to medicine.

America is perhaps the world leader in allowing its citizen individual liberties, but sadly we continue to deny our citizens the guardianship of their own health. Without health, all other liberties become meaningless.

Medicine is often a matter of individual choice. We know from modern science that what works for some does not work for others. Last week we heard testimony that some of our country's top Government scientists are not promoting the progress of medicine but, instead, are holding it back. Today, we will hear from a highly esteemed doctor, researcher and advisor to the German Congress on the medical advances that have taken place in Germany in the past 20 years that further illustrate how American medicine is, in some ways, behind the times.

The meager advances by conventional medicine in the treatment of most chronic and deadly diseases has made alternative and complimentary therapies overwhelmingly popular. But we learned last week that our system needs significant change so that these therapies are given the opportunity to be tested in scientific but rational ways. Not too many dying cancer patients want to be a part of a test where they will end up with a placebo and no chance for survival. Today, we will hear testimony about different but reliable means of testing alternative and complimentary therapies.

Defending good science and weeding out fraud are the reasons bureaucrats use to closely monitor independent researchers and doctors who use alternative therapies. However, we heard from patients and doctors last week who felt that the FDA interference is often unnecessary and unwanted. Americans do not want the Government, in this case the FDA, telling them how to treat their illness, especially when State level protections are already in place to safeguard the public from those who might do harm to patients. Today, we will hear from a doctor who sits on the Michigan State Medical Licensing Board who will tell us about those State level protections.

The FDA process for patient access to unapproved treatments in a good example of the nature of the Federal Government to micromanage the lives of individual Americans often unnecessarily.

Access to a treatment in the development process that is not approved by the FDA generally requires participation in a clinical trial. But if a patient does not qualify under the strict guidelines of a trial, the FDA then makes a life or death decision as to whether a patient can have the treatment under a special exception. If the answer is no, their access is shut off with no appeal, and many times they're just told go home and die.

Today, we will hear from a young girl with little chance of living because of her illness. She has undergone the most painful rituals of conventional medicine; chemotherapy, massive radiation and a full bone marrow transplant that nearly killed her. Now she wants to take a nontoxic treatment to keep her cancer from coming back, but the FDA will not let her. This little girl if a perfect example of why Americans want change: compassion.

These hearings will explore ways to help those hundreds of thousands who get left out of the FDA sponsored experimental treatments and, therefore, are left out in the cold. We have some highly esteemed Members of Congress who will continue the discussion of last week about the legislation entitled, The Access to Medical Treatment Act, and how that legislation can assist American citizens in accessing the treatment they and their physician believes is best for them.

Seriously ill patients want options. I have stated before that the terminally ill should have access to any potential cure available. But chronic illnesses can be just as bad. If conventional medicine does not offer a cure, the chronically ill should have the right to try an alternative without the headache of bureaucratic red tape and Government officials who think they know better. The Government ought to be helping them find new alternatives, not throwing up roadblocks. It's time for the Congress to show true leadership in providing greater access to new and promising treatments. Good health and medicine require it.

And I'll just add as a final note that I believe anybody who is terminally ill, who is suffering chronic problems, ought to have the right to try any therapy that they think is necessary. And one of the things that was so startling to me last week was a chart that showed in the case of cancer, I believe it was breast cancer or was it just all forms of cancer, that all forms of cancer since the 30's there has not been any real improvement in the survival rate with all the so-called new technologies and treatments we have. And if

that's the case, if there's no more hope than there was 40 or 50 or 60 years ago, then why are we denying these people alternative methods of treatment when their lives depend upon it?

And with that, I yield to the gentleman from Connecticut.

Mr. SANDERS. Close.

Mr. BURTON. Massachusetts.

Mr. SANDERS. A little northeast.

Mr. BURTON. Vermont. Vermont. Forgive me, I'm sorry. I know it's Mr. Sanders from Vermont.

Mr. SANDERS. All right, you're halfway there. OK. Thank you, Mr. Chairman.

I find these hearings much more positive than many of the other meetings that we hold on this committee, and I agree with much of what you have said.

I think there is no question but that in the United States of America there has been an explosion of interest in alternative and complimentary medicine in the last several decades, and frankly no matter what the U.S. Congress does, that explosion is going to continue. We are not leading the effort, we are as they say, the tail—well, I don't know what they say, but we are following the country. We're not leading, we're following. The American people want alternatives, they want change and we've got to listen to those requests.

In the State of Vermont, we held a hearing I remember a couple of years ago, 500 people came out to discuss alternative health. The U.S. Congress established several years ago the Office of Alternative Health, which is being swamped with requests. And frankly, Mr. Chairman, I would hope that we would take a look at whether or not the Office of Alternative Health is getting the kind of attention and respect from the NIH that is deserves. I think it was established, I think they are trying to do a good job and I don't know that they're getting the support from the NIH that they require, and I would hope that we would take a look at that and I would support your efforts to do that.

I think everybody knows that many of the ideas that were in fashion 20 or 30 years ago about health care problems and cures no longer stand the weight of time. We had doctors on television advertising cigarettes 30 years ago, we had people laughing that nutrition was an important aspect of health care, chiropractic approach was thought to be fraudulent, herbs and vitamins just quacks were talking about these issues and now all of those ideas are part of mainstream thinking.

So I think that I want to congratulate Representative DeFazio for introducing his important legislation. I think, as you said Mr. Chairman, there are a lot of problems out there which establishment medicine has not yet come up with appropriate answers and I think this Congress should be looking at all of the alternatives that are out there, many of them which are less expensive, many of which have far less, if any, severe side effects than some of the cures that are being brought forth by the establishment medicine.

So, I thank you very much for holding these hearings and look forward to hearing from our witnesses.

Mr. BURTON. I thank the gentleman from Vermont for that very elegant statement.

Does any other Member have an opening statement they'd like to make? If not, then Mr. DeFazio and Senator Gochenour, would you please stand.

[Witnesses sworn.]

Mr. BURTON. Mr. DeFazio, do you want to start with an opening statement?

STATEMENT OF HON. PETER A. DeFAZIO, A REPRESENTATIVE IN CONGRESS FROM THE STATE OF OREGON

Mr. DEFAZIO. Thank you, Mr. Chairman. I've provided copies of written opening statement to the committee. I'll depart from that, but I would draw people's attention to it that it's been entered in the record.

Mr. Chairman, in the opening remarks both you and Mr. Sanders have made points that I'd like to amplify upon.

I think the question before the Congress; some people have said to me, "Well, why is the hearing in Mr. Burton's committee and not over in Commerce," and quite frankly because we've run into the same stonewall over there that we've run into many other times when advocating for alternative treatments and for people's right to in an orderly and safe way access alternative treatments. So, I'm very pleased that this committee has chosen to show the leadership and begin to open this subject to discussion.

This subject has not been discussed in any comprehensive manner before any other committee of the U.S. Congress, and it is far past time.

The New England Journal of Medicine a few years ago said more than about a third of the people in America had accessed alternative health care. A recent survey that was done by a group called Interactive Solutions, a polling firm, showed that over 42 percent of Americans have used some form of alternative care, and 67 percent think it should be a part of every health insurance plan. And the minority document from last week, which I read, said that at some point in their lives 80 percent of the people in America had accessed alternative care.

So I think the question before us is: Are we going to go forward in a reasonable manner of building in the protections people need but allowing them to make the health care choices that they have a right to make as individuals, in protecting them from fraud and abuse and adulterated substances, or are we going to continue to stonewall and, in effect, protect the pharmaceutical giants and the status quo? Because I think part of the bias at the FDA is you can't err by not approving something, and that's unfortunate. I think it grows in great part out of the thalidomide tragedy and other things where something was kept out of the country, rightly so, and did save lives and prevent tragedy. But we've moved well past that, and I would particularly direct people to the German model.

Just a few points I'd like to make here. The Germans have set up a two-track process. They have an allopathic base approval process for pharmaceuticals, which is akin to ours; it's expensive, it's elaborate, it takes years and drugs do go through that process. But they also have set up a separate listing for natural based and alternative products which are approved on the basis of recent literature and clinical practice and not through 8 years of double-

blind clinical studies with a $500 million expenditure, and there's a reason for that. The products in question are natural products. They're not under German law or United States law or international law patentable. They can't be patented so nobody is going to take one of those substances, no matter how effective, even if it's the cure for cancer or something else, through the approval process because at the end anybody can manufacture it and sell it. They don't get the same exclusive rights that an allopathic pharmaceutical drug company gets after it goes through a $500 million process. We recognize the expense, we allow them to recapture their investment.

We need a two-track system minimally to deal with this and then also the catastrophic and compassionate use things that the chairman raised, which I'll get to at the end of my testimony. And I'd just like to mention a few substances, because I think they're very relevant.

In Germany, Prozac is prescribed, but only for really severe cases of depression and mental illness. But St. Johns Wort is prescribed 7 times as often at a fraction of the cost with a fraction or none of the side effects that Prozac has. Yet St. Johns Wort in this country if you were to say take this as a physician or a health care provider, the FDA could prosecute you because that's not an approved substance. It's a natural substance. Nobody can patent it, we know it works, it works in Europe, it would work on Americans but it'll never be on the shelf here accompanied by data and literature that says this is an effective treatment for your depression or your seasonal affective disorder.

Since most of my colleagues are male, think of prostate problems, something that effects basically 1 out of 10 American men particularly as they age, benign prostate enlargement and the problems that come with that. Now, there's a nifty drug out there, Proscar, very expensive, has a few potential side effects; impotence, liver damage, and a few other things, but it works to some extent or you can take a natural substance, saw palmetto particularly in combination with Pygeum Africanum and pumpkin seed and some other things. They've found in Europe that actually it's as effective or more effective in terms of dealing with the benign prostate enlargement, and it doesn't have any side effects.

But can that happen here in the United States of America? Well, it can happen if you or someone who goes out and does your own research and then goes to the health food store and buys it without any claims attached to it, you can buy those substances, yes, despite some past efforts on the part of the FDA to keep people from buying those things. They are available. But you can't go to your physician and have them prescribe it or your health care provider and have them prescribe it. In my State, you can go to a naturopath and they'll tell you that. But, again, they're at risk; they are exposed to the FDA process at some point because those products were not solely manufactured within my State and they are subject, theoretically, to Federal regulation and FDA overview.

One other simple example would be sleep disorders. When I first came to Congress and went on a few trips a physician would say, "Here, take these things, they're really nifty." And I said what's that, he says Halcyon. I said OK, so take some Halcyon for your

jet lag when you go to Europe or somewhere else. Well, it turns there's some problems with Halcyon; among them there seem to be some problems with agitation, even people who use it very frequently with some pretty severe psychological problems. It can cause problems of nausea, it's been implicated perhaps in the infamous incident of President Bush and the Prime Minister of Japan in terms of the problems President Bush had. But that was the standard treatment.

Now it turns out if you were in Europe you can go into any drug store without a prescription and say, "I'm having trouble sleeping," and they would say, "Well, here's some Valerian," and I've done that in France and in Portugal on my own.

Now in America no one can claim that Valerian will help you with a sleep disorder because the FDA hasn't approved it. But in Europe it's widely recognized, widely used and effective. There are other substances which do similar things.

And then I would go to something that has finally received some acceptance in this country, acupuncture. Many of us remember when the first people went to China and said, "Oh, this can't work, can't possibly work. You can't operate on that person and use acupuncture for anesthesia. You've got to give them these substances that essentially suspend their life and put them on life support while you operate on them because that's the way we do it and that's what science says works and what—you're sticking needles in somebody. That can't work." Well, it turned out they watched, it worked and then it turned out there was wider applicability. And finally after decades of being denounced by the so-called quack busters and others, acupuncture has been accepted by a panel under the auspices of the NIH to treat a number of conditions.

So, we don't know everything we need to know, and we do need a more open process and a process that recognizes the fact that there are things out there that don't fit into our medical model. In fact we've actually got a quote from Dr. Michael Friedman, a Commissioner of the FDA, he essentially said this. He said, "Collectively we need to address how to promote research on possible effective remedies where market incentives may not work." Yes, he's right, our process not only doesn't work, it locks them out and leads FDA bureaucrats to attack what we know are effective remedies elsewhere. That's my spiel on naturopathy.

The other issue that's been raised by the Chair is very much on point. There was a panel, which I believe the chairman discussed in the last hearing about the practice, outcomes, monitoring and evaluation system, POMES, where the OAM and NCI convened a group of people to come together and begin to look at unconventional treatments for cancer. Because, as the chairman said, we have made scant progress in terms of life expectancy, even quality of life, for people suffering the horrible scourge of cancer, despite our commitment as a Nation of billions of dollars. Maybe we should look outside the box a little bit. But I don't believe that there is real commitment on the part of OAM, FDA or NCI to really follow through on that process. I would just say that I think the chairman's very much on the right track there.

And also the Reform bill that passed last year in terms of compassionate use exemptions and that was not adequate and needs to be further addressed.

Mr. BURTON. Mr. DeFazio, I think your time has expired.

Mr. DEFAZIO. Yes. Thank you.

Mr. BURTON. Let me just say that we intend to work with you to try to make sure that we get some results from the FDA and try to get your bill passed. And I will be talking to Chairman Bliley about your bill. I intend to be a co-sponsor of your bill, if I'm not already, so we're going to be working on that.

What we need to do today and in the future hearings is get as many facts as possible so we can lay all that before the chairmen of the relevant committees in both the House and the Senate and then push like the dickens to get the FDA to move as well.

Mr. DEFAZIO. Thank you, Mr. Chairman.

Mr. BURTON. Yes, sir. And thank you for that very comprehensive statement.

[The prepared statement of Hon. Peter A. DeFazio follows:]

Thank you, Mr. Chairman, Mr. Waxman and members of the committee for giving me the opportunity to testify before you this morning. I would like to state for the record that I am not here to discredit or undermine the mandate of the FDA, NIH or the NCI. Nor am I here to advocate for a particular medical modality or interest group.

To me, the issue is very clear. Patients all over this country are being denied access to beneficial health care treatments by our government despite significant consumer demand. These treatments are readily available in Europe and elsewhere. In 1993, the New England Journal of Medicine found that one out of every three Americans are using some form of alternative medicine.

A recent nation-wide study conducted last November by Interactive Solutions on behalf of Landmark Healthcare Inc, an HMO found that over 42 percent of American consumers use some form of alternative medicine. 67 percent surveyed believe that the availability of alternative care is an important factor when choosing a health plan. The background memo prepared by the minority staff of this committee stated that over 80 percent of American consumers have used some form of alternative medicine at least once in their life-time.

A wide range of treatments are being used by millions of American consumers but will not be researched or certified in an official manner. Most of these products are not patentable in the U.S. because they are made from essential nutrients and other natural substances.

Sadly, none of these products are compatible with the current FDA approval process because the system is only designed to give approval to pharmaceuticals. Natural medicinal products should be offered a separate approval track similar to the one used in Germany, where manufacturers submit their product for approval and undergo a different set of scientific reviews for safety and efficacy. American consumers in growing numbers are using these products regardless of whether they are approved or not. Its time for the FDA to develop a two track approval system — one for natural medicine and one for commercial pharmaceutical products and devices to meet consumer demand.

THE GERMAN MODEL FOR APPROVAL OF HERBS AND NATURAL SUBSTANCES.

* Commission E in Germany uses an approval process for medicinal herbs and phytomedicines that is separate from the conventional medical process. It is used to approve medicinal herbs and phytomedicines. Manufacturers and medical researchers are able to get approval for their plant-based medical products without having to submit to the extraordinary rigors, requirements, conditions and huge expenses needed for the approval process for pharmaceuticals.

* The Commission is composed of an expert panel of physicians, pharmacists, pharmacologists and biostatisticians. It is the German government's equivalent of the FDA and is charged with reviewing plant-based products based on bibliographic literature that is actively gathered. This bibliography contains information on chemical data, pharmacology, toxicology, clinical studies, traditional and historical use, epidemiological studies and patient case records. Members of the Commission assess this data on a doctrine of absolute certainty for safety but reasonable certainty for efficacy.

* This bibliography is used as a guide to consumers, doctors, pharmacists, professors and practitioners. Over 70 to 80 percent of general practitioners prescribe herbal medicine approved by the German government.

* Statutory language contained in the Dietary Supplement Health and Supplement Education Act (DSHEA) authorized the creation of a Presidential commission on dietary supplements. This past November, the commission recommended that the FDA review herbs for possible approval as over the counter drugs (OTC), which alluded to the Commission E model in Germany.

THE BENEFITS GARLIC IN TREATING HIGH CHOLESTEROL LEVELS.

* A recent study published in the American Journal of Clinical Nutrition demonstrated that cholesterol levels may be reduced when taking both fish oil and garlic. This basic nutritional combination contains significant health benefits to individuals suffering from high blood cholesterol levels. For centuries garlic has been used as a natural antibiotic and a powerful immunostimulant. This simple and low cost combination has the potential for preventing curable conditions and could be used as an alternative to commercial products. Unfortunately, it will never be approved under the current process.

TREATMENT FOR BPH: THE COST AND SIDE EFFECTS OF SAW PALMETTO VS. PROSCAR.

* There is a significant amount of evidence indicating that saw palmetto, pumpkin seed extract and an herb called Pygeum Africanum are far safer, less costly and more effective that the current, FDA-approved allopathic treatment for an enlarged prostate due to benign prostatic hyperplasia (BPH).

* This condition afflicts one in every 11 American men per year. The current FDA approved treatments for BPH are Proscar or finasteride. Both treatments costs around $75 per month and can cause dangerous side effects such as impotence, insomnia, urinary track complications and urogenital birth defects.

* According to Lanh Green, Diane Wysowsi and Jean Fourcroy of the FDA, Proscar also causes gynecomastia, which is excessive development of the male breast. This condition is also known to cause breast cancer in men. Merck, the manufacturer of Proscar grosses more than $1 billion in sales annually for the product.

* Numerous medical studies conducted throughout Europe demonstrate that the extract of saw palmetto (serena repens) is far safer and more effective in reducing pain and swelling in the prostate than both allopathic treatments approved by the FDA. There are no known side-effects associated with saw palmetto and it is widely used around the world. It was rejected by the FDA even after its efficacy was proven through numerous clinical trails. The FDA reported that these results were *statistically significant* but denied the health claim because the results were not *medically significant*.

THE BENEFITS OF TREATING DEPRESSION WITH ST. JOHNS WORT OVER PROZAC.

* St. Johns Wort is the preferred therapy over pharmacological medication for mild depression in Germany. It is reported to enhance the immure system, increase antiviral activity and relieve seasonal affective disorder. The British Medical Journal published an overview of 28 clinical trials in 1996 conducted by

Ludwig-Maximilian University in Munich which confirmed that in 1,757 patients St. Johns Wort was as effective as commercial antidepressants. The studies also noted that the herb produced minimal side effects.

* Prozac, the preferred alternative to St. Johns Wort in the U.S., is known to cause nausea, anxiety, insomnia, diarrhea, dizziness, headaches, sexual dysfunction, and difficulty with concentration. St. Johns Wort also costs about $10 per month whereas Prozac costs, on average, $80 per month. In Germany, the leading St. Johns Wort product outsells Prozac by seven to one.

* NIMH in conjunction with OAM will be conducting a four-month double-blind study of 330 patients on the effectiveness of St. Johns Wort in treating clinical depression compared to Zoloft and a placebo. The coordinating research site will be Duke university. The study will cost $4.5 million and it will come out of the OAM budget.

THE BENEFITS OF USING VALERIAN ROOT OVER HALCYON.

* Valerian root is commonly used all over the world as a sleep aid. It is also used to treat fatigue, jet lag, nervousness and is an approved therapy in France, Germany and the U.K. Valerian has also been listed in the European pharmacopeia since 1973 and has been proven safe and effective in numerous pharmacological studies. Several double-blind clinical studies have proven the benefits of Valerian root in improving sleep quality.

* In 1994, the European American Phytomedicine Coalition submitted a citizens petition to the FDA for approval of Valerian as a night time sleep aid. To date, their petition is still pending and they haven't received a response from the FDA regarding the status.

* Halcyon, the FDA approved, pharmaceutical counterpart to Valerian, contains benzodiazepine which is known to cause nausea, dizziness, insomnia, panic, depression, headaches, anxiety, and deprives patients of REM sleep which disrupts normal sleep patterns.

* When I came to Congress in 1986, Halcyon was commonly prescribed to Members of Congress for jet lag and sleep disorders. Halcyon was also the substance that was implicated in the incident where former President George Bush vomited at an official dinner hosted by the Japanese Prime Minister.

ACUPUNCTURE – ONCE CONSIDERED "QUACKERY" NOW A LEGITIMATE MODALITY ENDORSED BY A GROUP OF PHYSICIANS SPONSORED BY AN NIH COMMITTEE AND CHAIRED BY DR. DAVID RAMSEY, PRESIDENT OF THE U. OF MARYLAND MEDICAL SCHOOL.

* The history of acupuncture in the U.S. demonstrates the inflexible bias of the mainstream medical community even after it was widely accepted by American consumers. Acupuncture has been used by one fourth of the world's population for the last 2500 years. However, in the U.S. it was considered "quackery" by a majority of physicians and surgeons until recently.

* In a recent NIH consensus development meeting, a 12-member committee of independent doctors and scientists determined that acupuncture was a satisfactory treatment for chronic pain, vomiting and nausea

related chemotherapy, nausea from pregnancy and postoperative dental pain, back pain and post stroke care.

* A report published by the NIH committee on acupuncture stated that "while conventional medical practices are often thought to be utilized because there is substantial research evidence to support them, this is frequently not the case. But this does not mean that these treatments are not effective. The data in support of acupuncture are as strong as those for many accepted Western medical therapies."

* According to the American Cancer Society information database, acupuncture is described as "simple and often works. It has few side effects or complications and the cost is low. For these reasons, it can be a good choice for some problems that have no underlying cause which can be treated.

A CALL FOR ACTION ON THE PRACTICE OUTCOMES MONITORING AND EVALUATION SYSTEM (POMES).

* This summer, the OAM and the NCI convened a meeting called the Practice Outcomes Monitoring and Evaluation System (POMES) to consider how to better evaluate the practices of doctors who use unconventional methods to treat cancer. However, most of the participants of the meeting were puzzled and somewhat discouraged by the lack of attendance among the top FDA and NCI officials, especially since the meeting was sponsored by NCI. Dr. Klausner, Director of NCI and his Deputy, Dr. Wittes were both absent from the meeting.

* A consensus was developed at the meeting that called for the creation of an Oversight Board led by a body of experienced medical professionals that would help guarantee a level playing field for research in the area of alternative practices. Unfortunately, since the meeting, NCI and NIH has done nothing to facilitate the consensus decisions that were made. I urge the committee to put pressure on the NCI to follow through with the POMES meeting.

GENERAL FACTS ABOUT CANCER.

The NCI has spent over $10 billion in research over the past five years, yet cancer patients are still left with the same limited treatment options such as radiation, chemotherapy or surgery -- all very costly and physically debilitating, and there has been little increase in life expectancy during that time period. Very little has changed in the last 20 years. Let's take a look at some well-known facts:

* Roughly 1.5 million Americans are diagnosed with cancer each year.
* By the year 2000, two out of every five Americans will be diagnosed with some form of cancer.

* I ask my colleagues, why are we spending so much taxpayer money exclusively on conventional medical research when we have so little to show for it. I would urge my colleagues to request the NCI to begin to conduct studies on alternatives that are being used by practitioners and doctors in the U.S. and around the world. It seems to me that we aren't being fiscally responsible in our fight against these deadly cancers.

BREAST CANCER

* In 1962, over 63,000 women were diagnosed with breast cancer

* In 1971 when President Nixon launched the "war on cancer," this number reached 69,000, and a woman's lifetime risk of contracting breast cancer was one in fourteen.

* Since 1960, nearly 2 million American women died from breast cancer. This is an outrageous figure and yet the NCI and the NIH still spend billions of taxpayer dollars. This level of mortality is not acceptable and the NCI and the NIH should spend a portion of their research funding on researching promising alternative therapies

WHAT IS WRONG WITH THE CURRENT FDA DRUG APPROVAL PROCESS.

* According to a report by the Office of Technology Assessment (OTA), the FDA approval process costs an applicant over $500 million dollars and about 8 to 12 years before a new drug receives approval.

* I ask my colleagues, does it make sense for a medical researcher who is not affiliated with a multinational pharmaceutical company to seek FDA approval for a product if they cannot obtain a patent for it? If their product is non-patentable they are unable to recoup the financial losses involved with seeking approval. How could they ever expect to obtain FDA approval if they cannot obtain the amount of capital involved? The answer is that many manufacturers and medical researchers do not seek approval due to these cost barriers.

* In his testimony before the House Commerce Committee on September 23, 1997, Dr. Michael Friedman, the Lead Deputy Commissioner of the FDA expressed the same concerns. He said that "collectively we need to address how to promote research on possible effective remedies where market incentives may not work."

* Our current system still forces many Americans to seek treatments outside the U.S. Patients are also forced to obtain medications without any official monitoring or content review. This leaves chronically-ill patients with no other choice but to circumvent the law by obtaining treatments which are unapproved.

WHAT IS WRONG WITH THE FDA REFORM BILL THAT PASSED LAST YEAR?

* Despite claims to the contrary, the FDA modernization act provides no new mechanisms for expanded patient access to investigational drugs and therapies. Instead, the Act continues to let the FDA make the final word on whether a seriously-ill patient receives access to an investigational drug despite the pleas of doctors, scientists and other medical professionals. Specifically, Section 561 of the Act:

1) requires FDA to conduct a detailed and time-consuming administrative review for chronically ill patients seeking alternative treatments in emergency situations.

2) permits the FDA to second-guess the judgement of attending physicians and scientific experts regarding the safety and efficacy of an unapproved treatment, thus enabling the government the last word in denying patient access.

After reviewing the provisions in this Act, I am amazed that my colleagues remain convinced that this bill provides all the access necessary to critically ill patients and their families. I choose to stand with patients and families who are fighting for fairness and low cost, effective treatment options that are currently absent from our health care delivery system. It is time for our government to defend the rights of American

patients and consumers. The discussion of a consumer bill of rights, which is not within this committees jurisdiction, should correct the flaws of the FDA modernization bill.

I urge you all to remember the words of one of our great leaders, a crusader for fairness and freedom, Abraham Lincoln when he said "the legitimate object of government, is to do for a community of people whatever they need to have done, but cannot do for themselves in their separate individual capacities. In all that the people can individually do as well for themselves, government ought not to interfere." (Collected Works of Abraham Lincoln July 1, 1854)

I look forward to working with this committee and the FDA in making patient access to medical breakthroughs a reality for millions of Americans.

Mr. BURTON. Mr. Moran.

STATEMENT OF HON. JAMES P. MORAN, A REPRESENTATIVE IN CONGRESS FROM THE STATE OF VIRGINIA

Mr. MORAN. Thank you, Mr. Chairman.

This is an important issue and we are very appreciative that you're using this committee to focus attention on the need to reexamine our policies with regard to alternative medical treatments.

I support access to alternative medical treatments, and the reason is attributable to my own experience with our daughter. She had diagnosed when she was 3 years old with medulloblastoma, which is a very aggressive form of brain cancer. She had a 20 percent chance of surviving until her 5th birthday and that was assuming that she used all of the conventional treatments, the radiation, the chemotherapy and, of course, as much surgery as could be performed.

Like all parents who face that kind of a situation, we reacted out of desperation. We spent thousands of hours, particularly my wife, learning about all the alternative things that we could do and what the impact of chemotherapy and radiation would be upon our daughter. My wife must have bought several hundred books, every book that she could find and she seemed to read them all while I was trying to go through the functions we have to perform here at work. And she put together a vitamin and supplement program that was designed to strengthened her while our daughter underwent chemotherapy. That did buy us time. The doctors had recommended that we go under radiation immediately just almost simultaneously with her taking the chemotherapy. But having met a lot of children who had gone through that, while the cancer may have been killed, the child's personality was dead as well.

Dorothy is now 6 years old. She has been cancer-free for many months. We don't know what worked. We don't know why she is alive and virtually all of the children that we met in similar situations at Children's Hospital are now dead. We know that she would not be alive if he she hadn't had radiation and chemotherapy as well as the surgery, obviously. But we also think that one of the factors that contributed to her health today is the vitamin and mineral supplement that we put her on, and that's why we think that every family facing a situation like this needs to be able to make that choice.

We're not proposing that they do what we did, but we certainly think that they ought to have the choice. Obviously, we don't know what the future holds. The likelihood is that our daughter is still alive because we were such aggressive health care consumers, if you will. The basic premise that we went on was that while you can cut or burn, or poison the cancer within the body, it's going to come back again unless you deal with the underlying cause, which is a compromised immune system; an immune system that is not strong enough to ward off the cancer in the first place. And I think that the way you buildup that immune system is through these, what we're calling alternative complimentary medicine. It's really nontoxic vitamins and minerals and related things that strengthen the immune system. That's the purpose. Build up our own natural defenses.

I don't think our story is unique. I know it's not because we've heard from thousands of people around the country who have read about our situation, but there are some States that where families have not been as fortunate as we have been. And I read about California, for example, where it's illegal for a doctor to treat cancer with anything but chemotherapy, radiation and surgery and I don't think they're supposed to talk about any alternatives, so that the person is really on their own. Fortunately, we had doctors who when we asked were willing to tell us everything they knew, and that was critical in our approach to it. But there are a lot of families who don't get that information. And they wind up essentially being pushed to the sidelines watching their family member suffer and really not being able to use their emotional energy in a positive way, in a constructive way. They need to be able to do that, and sometimes our laws preclude them from being able to do that.

I think State governments really do a disservice to their citizens when they restrict access to alternative treatments, particularly complimentary alternatives. I think the Federal Government may be disserving individuals that face life threatening illnesses when they really don't provide a choice of treatment even when it is experimental or hasn't been approved by the FDA. We really have an obligation to speed up that process of approval. We can't let people die because of any kind of bureaucratic itinerary or unwillingness to, I guess, be aggressive. I don't want to say the desire to avoid all risk, because I think that they assume some risk, the professionals do, but we need to make the information available to the public just as soon as we can and in as full a manner as possible. And I'm not sure that that is being done now, although I do have great respect for the FDA and the professionals within the FDA, and we want them to continue to use their professional expertise and continue to be the credible source that they are, but it seems as though they can perhaps speed up the process without a commensurate loss of their credibility.

I know my time is up, let me just try to sum this up.

People are now doing it anyway. People are finding alternative treatments. About half of all cancer patients use an alternative cancer therapy in treating their illness, so it's going to happen anyway. The Government really should be helping them in making that kind of a decision. I think that's what we're looking for. And I would rather, and I know you would as well, have the FDA and professionals at NIH and so on giving information than people getting their information from whatever they see at the grocery store checkout line. And that's where many people are getting their information. While some of it may be true, I think we take a real risk in that being the principal source of information. So I support Congressman DeFazio's bill, the Access to Medical Treatment Act, and I appreciate your promoting it, Peter.

I very much appreciate, Mr. Chairman, your holding the hearing on it. Thank you.

[The prepared statement of Hon. James P. Moran follows:]

Testimony of Congressman James P. Moran
Committee on Government Reform and Oversight
Oversight Hearing on Patient Access to Alternative Treatments
February 12, 1998

Mr. Chairman:

Thank you for holding this hearing today on patient access to alternative medical treatments. This is an important issue, and I hope that this hearing will help focus attention on the need to reexamine our policies with regard to alternative medical treatments.

Like many of the people who are involved with this issue, I support access to alternative medical treatments. My views on this have been supported by the experience that my wife and I have had with our daughter Dorothy, who suffered from medulloblastoma, a very aggressive form of brain cancer. When she was first diagnosed, she was 3 years old and was given a 20 percent chance of surviving until her 5th birthday. Dorothy's 20 percent chance of survival was contingent upon undergoing all available conventional treatments, including chemotherapy and radiation.

Like all parents who face this unbelievable, horrific news, my wife and I spent every moment that we could learning about the effects of chemotherapy and radiation. We examined what other treatments we might be able to pursue for Dorothy that could help her beat the terrible odds she was facing. My wife, after literally hundreds of hours of research and assistance from medical professionals, developed a vitamin and nutritional supplement program for Dorothy that not only helped her withstand chemotherapy, but also bought us some time to strengthen Dorothy and wait until she was a little older before undergoing radiation treatments, which can have very detrimental impacts on young children.

Dorothy is now 6 years old. She is cancer-free and has been for many months now. I can't say for sure what part of Dorothy's treatment worked and helped her beat those initial odds. I don't think she would be alive today without radiation and chemotherapy, and I strongly believe that no patient should ever forego conventional treatment. But, I do think that the vitamin and nutritional supplement program helped Dorothy, and I think every parent of a child facing a serious illness should be able to make the kinds of choices we made about pursuing alternative and complimentary therapies.

I don't know what the future holds. But I do know that we've gotten this far by being vigilant and aggressive health care consumers, by pursuing alternative therapies to help strengthen our daughter, improve her health, and help her endure the battle against cancer.

I am here today, because I know that our story is not unique. I have heard from thousands of people across the country who face similar situations, but who are not fortunate enough to

have access to physicians who can help them seek alternative treatments, or who live in states, like California, where it is illegal for a doctor to treat cancer with anything except chemotherapy, radiation or surgery. These people are desperate because they are fighting a terminal illness and conventional treatments are failing them, and so is the medical establishment.

State governments that restrict access to treatment fail their citizens. The federal government also fails to help these individuals by refusing to allow those facing life-threatening illnesses to choose a treatment that is experimental or has not yet been approved by the FDA. We fail them by restricting the ability of physicians to discuss alternative treatments with their patients.

I appreciate the incredibly difficult job that the FDA has in approving drugs and medical devices. The time that is taken to review these products helps to ensure that, once approved, they are completely safe for human use and live up to their promises. The length of the approval process, however, also has negative consequences. Individual practitioners, scientists, smaller companies and others who do not have the financial resources or the expertise to complete the arduous FDA approval process are prevented from gaining access to the market.

Does that mean that individuals facing a life-threatening illness, who learn of a potential effective treatment, should be denied access to this treatment because it has not yet been approved by the FDA? I don't think so. I think people facing life-threatening illnesses should be able to consult with their physicians and make an informed choice about alternative treatments.

Several surveys show that individuals facing life-threatening illnesses already do this, despite roadblocks and barriers. A recent survey showed that about half of all cancer patients use an alternative cancer therapy for the treatment of their illness. Finding and using these options is difficult and risky. A child or adult with advanced cancer will often seek out advice from popular magazines, friends, health food stores, and go to foreign countries in a haphazard and expensive manner in order to seek effective treatment.

Despite the fact that the United States leads the world in exceptional medical care, the current system excludes the development and utilization of non-harmful alternative medical treatments that may help patients and generate new approaches to treating illnesses. I support Congressman DeFazio's bill, the Access to Medical Treatment Act, because it will help to open up the system to the utilization of new alternative treatments and allow physicians to discuss these treatments openly with their patients.

As a parent and a legislator, I believe that this is the appropriate direction to take to help benefit individuals who face life-threatening illnesses and who desire access to all possible treatments and potentially lifesaving cures.

Thank you again for holding this hearing and giving me the opportunity to participate.

Mr. BURTON. Thank you, Mr. Moran. And I'm very happy to hear that your daughter's doing well. I know that you went through some tough times and I don't think any parent or any family can appreciate what you went through unless they've gone through it themselves. They may say they understand, but they don't. So I'm real happy she's doing well.

Mr. MORAN. Thank you.

Mr. BURTON. And I want to tell you one more thing. We are committed, I and I think a majority of this committee, to doing everything we can to open up the process and we're going to work with both your gentlemen to try to get that done.

They have Representative on there, you know he's a Senator. Do you know why they call a senator the upper house? It's because they had no room for them on the first floor.

Senator.

STATEMENT OF ED GOCHENOUR, STATE SENATOR FROM GEORGIA

Mr. GOCHENOUR. Thank you, Mr. Chairman.

I'm honored to be here before you today and even more honored to be sitting here with these two Congressmen, who are obviously very knowledgeable from what they've already said.

I am a State Senator from Georgia, and in my third term. In November 1996, I was diagnosed with a brain tumor myself. Obviously it was shocking to me, and went to three of the best neurosurgeons in Georgia trying to make a determination how to approach this. Obviously, not being familiar with the situation and cancer, but we went to the neurosurgeons. And basically they had all contradicted themselves after I got through talking to them all, and they obviously didn't know exactly what in their opinion was the best approach because they all said different things and they all said the other one wasn't saying the exact same thing. So I had to make a decision on what I was going to do; what neurosurgeon I was going to go to or exactly what was going to happen.

So after talking with my wife, even though everybody was trying to push us real hard, we backed off a little bit and started looking at alternative treatments and talked with a number of people. And after a significant amount of time we decided to go with Dr. Burzynski out in Houston, TX, which I know you've had some other patients from Texas here. I chose Dr. Burzynski, started treatment December 1996. After 3 weeks of treatment, 50 percent of the tumor was gone and after months the complete tumor was gone.

He has two types of treatments; an IV initial treatment and I was on an IV treatment. Had to wear 2 IV bags and a pump around nearly 24 hours a day during my General Assembly session in Georgia for a whole year. And then this past December, I was able to get rid of that stuff and start taking capsules now for the next 3 or 4 months.

But basically in a nutshell that's my situation, my personal situation, how I became involved with alternative treatments and alternative medicine. And because of that and finding out that alternative doctors, and specifically Dr. Burzynski, was having tremendous trouble not only with the FDA but the local State medical examiners and State doctors and stuff, I actually got a copy of Con-

gressman DeFazio's Alternative Access to Medical Treatment Act and revised it for the State of Georgia. Georgia became on April 22nd the 12th State to have that bill signed into law. And so in Georgia if a doctor wants to practice alternative medicine, if he meets just two simple requirements that he actually discusses orally with the patient the potential side effects and what the outcome is, and he gets a written statement from that patient that he can practice alternative medicine without the fear of the State medical examiners harassing him.

And I'm here to testify and hope that you at the Federal level can do the same thing so that doctors like Dr. Burzynski and other doctors can practice also without the harassment of the FDA.

My bill passed in a short time of 3 weeks, in Georgia that's a pretty quick time of passing a bill, with nearly no dissension at all on the bill. Once I was able to discuss with the people, the General Assembly, what was going on and gave them my situation, they overwhelmingly supported the bill and saw that there was need to open up this access to doctors and alternative treatment.

In the past year now, since this bill has been signed, there are doctors now just beginning to practice alternative treatments. And my hope is that Dr. Burzynski, who has a tremendous treatment for cancers of all kind, will be able to get his treatments approved or at some point where they can be accessed easily. Because what's happening is, is that people can't get to his treatments and because of the low number of people that are accessing his treatment, his price is high on his treatment. He's not a large pharmaceutical company and doesn't have the money himself and so very few people can actually access his treatment because insurance doesn't cover it. My insurance didn't cover it and I was fortunate, one of the people that was able to raise the money, nearly $100,000 to this point to take his treatment and still have to continue to raise the money to finish out his treatment, although we are fighting with our insurance company and hopefully that they will at some point in time see the wisdom in covering this type of treatment.

You know, the politics of this issue is not on a partisan basis, obviously. All you've got to do is look at the situation and I think you got to follow the dollar. There isn't a large constituency of people out there that are going to be here supporting the bill, but there will probably be people here opposing it. And I think that once again that the dollar is driving that, and I think that the large pharmaceutical companies don't want access where people have access to alternative treatments. And Dr. Burzynski's is one of those treatments, his patent, and he can pursue his treatment and people can pursue it.

I just want to thank you for this time before your committee and I will be glad to answer any questions.

[The prepared statement of Mr. Gochenour follows:]

** Proprietary **

Testimony of State Senator Ed Gochenour of Macon, Georgia.

Feb. 12, 1998

Thank you Mr. Chairman and committee members.

My name is Ed Gochenour and I am a State Senator from Georgia. I have a wife, Ginger, and we have been married 21 years. We have two boys ages eight and eleven.

I have to run every two years just as you do and on the same cycle. One week before this past election I had an incident that caused me to believe something was wrong with my health. My initial reaction was that it was stress related because it was in the final days of the campaign. I called a doctor friend of mine and she got me into see a neurologist the next day. The neurologist thought I had a mini stroke based on his examination. He sent me for some test including an MRI just to see if I had had any previous stokes. Well, the MRI came back showing a 2 inch brain tumor in the right front side of the brain.

I went to three of the best neurosurgeons in Atlanta and they seemed to contradict themselves in what the best options for treatment were for me. The last doctor was one of the best neurosurgeons at Emory University in Atlanta. This doctor suggested chemotherapy and radiation and said this might control the growth of the tumor for a while but eventually it would come back and be a lot worse and at that time we would have to see what options were available. Obviously this was not a very good option in my opinion. I decided against chemotherapy and radiation because of the toxic side affects and the increase chance of other cancers they themselves caused. A person that takes chemo and radiation is 25 times more likely to have another form of cancer than the average person.

I talked it over with my wife and we decided to look at an alternative type of treatment. I looked and studied the options for several weeks and decided that Dr. Burzynski had the cure for brain tumors.

I began treatment in the middle of December and three weeks later 50% of the tumor was gone. After five months the cancerous part of the tumor was completely gone. I have to remain on the IV part of the treatment until the end of this year and then will take the antineoplastons for several years by capsule.

After learning of alternative treatments and the problems they were having with the FDA, this past January during the Georgia General Assembly I introduced and was successful in getting passed an Access to Medical Treatment Act. The citizens of Georgia believe that patients ought to have the access to the treatment of their choice when their lives are threatened.

Because I am a State Senator my name has been in many stories nationwide associated with Dr. Burzynski. This has led many potential patients to call and ask me about the treatment and for help getting into a protocol.

The most disheartening thing about the whole ordeal with the FDA is that while the FDA is allowing the antineoplastons to go through clinical trials to test their efficacy, they are making patients take treatments they do not want to take before they can become a part of a clinical trial. One reason we choose Dr. Burzynski is that his medicine is nontoxic. For the FDA to make a patient take radiation before they can become part of a clinical trial for antineoplastons is unreal.

The FDA will not allow patients that don't fit the protocols to take the antineoplastons without a fight. One gentleman from Texas had high blood pressure and because the medicine is a sodium based medicine taking the normal dose the way the protocol requires would have caused him more problems. This gentleman needed a special treatment unique to him. It took six weeks of fighting with the FDA

and getting his Congressman involved before he could take the treatment.

When the FDA was created it was with good intent. The citizens of this country needed help with determining whether drugs were safe or not. But if I allowed my two boys to grow up without supervision they would become something different than they are now. They would be arrogant, belligerent, undisciplined and uncaring much like the FDA has become. I believe it is time that Congress steps in and brings some discipline to this department and restore some integrity.

Thank you for your time.

Mr. BURTON. Thank you, Senator.

You said that your treatment has cost roughly $100,000?

Mr. GOCHENOUR. That's correct.

Mr. BURTON. And did Dr. Burzynski indicate to you if there were a large volume of people taking these treatments and how that would effect the cost? Have you ever talked with him?

Mr. GOCHENOUR. Well, economics tells you that if he's got 200 patients now and all of them are not paying—are not able to pay as I have paid. If he's able to sell this medicine and give it to other doctors that could use it, obviously the price of that medicine would come down.

Mr. BURTON. So some of the patients don't have the ability to pay and they treat them anyhow?

Mr. GOCHENOUR. That's correct.

Mr. BURTON. That is correct. OK.

How much money did you have to spend, Representative Moran, in addition to your normal treatment? Was it a substantial amount of money for the other treatment for your doctor?

Mr. MORAN. Several thousands of dollars. We didn't keep an actual count, but it was several thousand. It wasn't close to a $100,000, it was some fraction of that, but it was certainly in the thousands.

Mr. BURTON. Any of my colleagues have any questions?

Mr. KUCINICH. I just want to say thank you, Mr. Chairman for having this hearing. And also, I understand, as I'm sure many of the people do in the Congress from a personal standpoint, how important the message which you are bringing here today is, how it's affected you personally, how it's affected your family. And this bill that Mr. DeFazio is bringing forward is an important first step in bringing recognition to those millions of Americans who are already aware of how alternative therapies can improve their chance not only for living but their quality of life.

So I wanted to say, and I know we're going for a vote in a minute, but I thank each of you for coming to this committee and also for sharing with us something that is to you sacred. Because it is sacred when someone comes in and talks about how something affects their family, how it's affected their own lives, and so I take that sanctity of a message with the importance of consideration it deserves and pledge to you my wholehearted support for the legislation.

Mr. BURTON. Mr. Sanders, do you have any questions?

Let me ask one more question of Representative DeFazio and then we'll let you folks go and we'll go to vote.

A lot of people have said that this bill is going to open the door to charlatans and people who are going to take advantage of innocent citizens who don't understand. How do you respond to that?

Mr. DEFAZIO. Well, Mr. Chairman, that certainly is a problem that's been ongoing in medical treatment for centuries. My bill actually builds in new protections against fraud that aren't currently available.

Well, first off, this would have to be a practitioner practicing within their scope of licensure within a State. States license health care practitioners. So you couldn't have a chiropractor doing something that was outside of that, like surgery or whatever. So it

would be within the scope of their practice. That's the first protection.

Second, they would have to disclose that it was unapproved. They would have to get a written consent form that was signed by the patient. They would have to immediately suspend treatment and report any unanticipated and undue side effects, counter-indications, problems with the treatment that imperiled the patient.

It provides that they could not have a financial interest, they could not advertise the treatment nor could the manufacturers advertise the treatment.

And then people would say, "Well, then what are you doing?" What we're doing is allowing practitioners who have inquisitive minds who are capable of just reading what's going on in Europe and elsewhere to inform their patients about those treatment or alternative treatments that are available here without worrying that someone might break down their door or try and lift their license, which is an actual fact to fear.

Mr. BURTON. And there have been prosecutions for this?

Mr. DEFAZIO. Yes.

Mr. BURTON. Let me just say to all three of you, I really appreciate your being here. This committee is committed to trying to make sure that everybody has access to alternative treatments, whether it's through the bill Mr. DeFazio is sponsoring or some other mechanism, and we're going to be having the FDA before this committee on a regular occasion until we get some answers.

And with that, thank you for being here. And Representative DeFazio and Moran, if you choose to come back and want to sit on the panel, we'd love to have you.

The Chair stands in recess until this vote is concluded.

[Recess.]

Mr. BURTON. The committee will reconvene our second panel and I hope my colleagues or I hope those in the room will not be discouraged by the lack of attendance at the hearings. This happens sometimes when there's a lot of things going on but we make the record available to all Members and our pursuit of the FDA in trying to bring about fairness will continue and will be resolute, so don't be discouraged because there aren't a lot of Members here at this point.

So we'd like to have Dr. Lyle E. Cheadle. Am I pronouncing that correctly?

Mr. CHEADLE. Yes, sir. You are.

Mr. BURTON. Come forward and bring your guest with you. Have a seat. You have an opening statement, sir?

Mr. CHEADLE. Pardon me?

Mr. BURTON. Do you have an opening statement you'd like to make?

Mr. CHEADLE. Yes.

Mr. BURTON. Would you pull the microphone real close to you so we can hear you?

Mr. CHEADLE. I guess my opening statement Congressman Burton is that we are enraged at what's going on.

Mr. BURTON. Pull it closer, please.

STATEMENT OF LYLE E. CHEADLE

Mr. CHEADLE. We are enraged at what's going on with the FDA, and I think the testimony that I'm about ready to give kind of shows that. We've been trying to get the FDA to do something now since September 15th. They haven't even responded officially to my correspondence and the testimony that I have here will kind of outline what transpired since September 15.

Mr. BURTON. OK, sir.

Mr. CHEADLE. And I'll just go ahead with my testimony, if you don't mind.

Mr. BURTON. Sure.

Mr. CHEADLE. Mr. Chairman and members of the committee, my name is Dr. Lyle E. Cheadle, Ph.D.

This is my daughter, Janet Isabella Cheadle. She is 7 years-old and has been to hell and back. She has been diagnosed as a Stage IV Neuroblastoma cancer patient. Over a period of 17 months, beginning on February 28, 1996, she has suffered through 10 months of very aggressive chemotherapy, 6 days of ablation chemotherapy, 3 days of total body radiation therapy, twice each day and then a very gruesome bone marrow transplant resulting in the doctors telling us on February 17th and 18th that our daughter had only hours to live. With God's will, many prayers and tears, the good Lord brought her back from the brink of death.

She was discharged in remission, only to incur three life-threatening secondary infections. She has been in relatively good health since August 1997. That may sound like good news, but this disease has a 93 percent rate of recurrence which results in death. Most medical journals do not give survival rates after 2 years. They know what happens.

After seeing what the damage standard protocols have wreaked on our daughter, Janet, we began research for an alternative treatment. We came across the Burzynski Clinic in Houston, TX. We contacted the clinic and we were told the Oncology Division of the Food and Drug Administration had to approve the treatment. On September 15, 1997, I wrote a letter to Mr. Paul Zimmerman, the Consumer Safety Officer, requesting that my daughter be allowed treatment at the Burzynski Clinic. While awaiting an answer, we gathered all the medical records on Janet and sent them to the Burzynski Clinic for evaluation.

After waiting approximately 3 weeks for an answer from Mr. Zimmerman, I called his number and via voice mail asked if he had any intentions of responding to my letter. As a result of my phone call, JoAnn Minor called me and said we'd have to wait for Dr. Burzynski's treatment protocol before we can do anything. The protocol package was sent to the FDA via fax. It was returned to the doctor's office disapproved in less than 5 duty hours. The doctor's office called and said the FDA had disapproved the protocol and would not allow the doctor to treat Janet prophylactically.

I was shocked by this response from the FDA.

The same evening I tracked down Mr. Zimmerman at his home phone and called him. He could not recall reading the letter. It amazes me that I wrote the letter and sent it express mail directly to him, "personal for." He could not recall reading the letter. He seemed confused. Several days before my call I had Dr. Reginald

Moore, MD, call Mr. Zimmerman. He also seemed to be confused when Dr. Moore called.

The morning after I called Mr. Zimmerman at his home, I received a call from Mr. David Banks, also from the FDA. I cried and begged both of these men to at least give my daughter a fighting chance. My pleas fell on deaf ears. Mr. Banks sent me a write-up, apparently from the "Journal of the American Medical Association" by a Dr. Green. It was dated 1992, as I recall. It was a smear of the treatment I was seeking and the doctor who invented it.

I called Mr. Banks back. He told me to call a Dr. Blaney at the Texas Children's Cancer Center. I was referred to a Dr. Stacey Berg. We discussed Janet's cancer. Dr. Berg stated that a patient in remission was not eligible to participate in this clinical trial for obvious reasons—nothing to measure. I spoke of the Burzynski Clinic. Dr. Berg stated she was familiar with the clinic. She agreed Janet had nothing to lose and everything to gain. She could not understand the logic behind the FDA's decision to refuse Janet treatment in view of the very, very poor prognosis of Janet by three highly qualified oncologists.

I wrote four letters to the President, a letter to the First Lady, every member of the Texas Delegation to Congress. Unfortunately, only Senators Phil Gramm, Kay Bailey Hutchison and Representative Chet Edwards responded. The FDA responded to Phil Gramm. They have not responded to Senator Hutchison because she has sent me three letters apologizing for the delay. I have received nothing back from Representative Chet Edward except a letter telling me that he cannot change a policy, procedure or regulation of a Government agency. The FDA tells me their hands are tied by laws that Congress has passed. I just wonder, who is in charge?

The FDA's reasons for not approving the treatment I seek for Janet are that it is inherently dangerous or there is no evidence the treatment would work. How in God's name do they know whether it works, if they don't try? When I asked the FDA why the treatment is dangerous, they tell me to reveal that information would violate the doctor's proprietary rights. I wrote a letter to the doctor, asking him to refute the FDA's statement, which he did in a letter dated December 12th, which is attached to this testimony.

Mr. Chairman, it appears to me that the only patients allowed to be treated by this doctor are terminally ill. What chance does any treatment process have which has only basket cases to work with? This gives the FDA ample opportunity to say, "See, we told you it wouldn't work."

Mr. Chairman, my family feels like trapped rats. We do not know from one day to the next if this cancer is going to return with a vengeance and kill our child but we know that there is a 93 percent chance that it will do so. We feel like screaming and lashing out at those who would sit and pass a death sentence on our daughter. Mr. Chairman, what the FDA is doing is tantamount to murder. That is the only word that I can come up with to describe their actions.

The FDA is the most arrogant agency of Government I have ever encountered. They are drunk with power over the life or death of cancer patients. They are vindictive and spiteful of anyone that does not adhere to their perceived norms. Mr. Chairman, we have

had a long war on cancer. The FDA needs to get on board to help conquer this terrible disease that kills 600,000 Americans every year. They apparently despise individual researchers when they should be acting in concert with them. They are a thorn in the side of finding a cure for cancer. Why should I have to fight the FDA to save my child? They should help me, not hinder me.

We wonder why our Government is locking themselves behind secure doors, metal detectors, armed guards and et cetera. We have forgotten that our government is of the people, for the people. We do not need bureaucrats setting up their little kingdoms in our Government agencies.

I also strongly feel that the GAO needs to conduct an exhaustive audit of the FDA to find out if they're working for the American taxpayers and not the big pharmaceutical companies.

That concludes my testimony, Mr. Chairman.

[The letter referred to follows:]

STANISLAW R. BURZYNSKI, M.D., Ph.D.
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December 12, 1997

Lyle Cheadle, M.D.
826 Cliffside Drive
Harker Heights, TX 76548

Dear Dr. Cheadle,

Thank you for your fax of December 8, 1997 which I received today. I see only two comments in the letter from the FDA which needs to be addressed:

1) Effectiveness of antineoplastons in neuroblastoma

2) FDA suggestion that "these drugs can be harmful."

The following is my response to these two issues:

1) In our Phase II study of Antineoplastons A10 and AS2-1 in Patients with Neuroblastoma, we treated only one patient who obtained partial response (more than 50% decrease in tumor size).

2) The FDA requires us to file safety reports on all first occurrences of any toxicity and on adverse reactions that are unexpected and serious, or life threatening during administration of antineoplastons, regardless if antineoplastons caused or contributed to the adverse experience. We are in the process of preparation of 1998 Annual Report to the FDA, which will include all such adverse reactions reported to the FDA since the previous Annual Report of 1997. There are five experiences of this type reported to the FDA and all of them consist of hypernatremia (increased concentration of sodium in serum). Based on the available information, it is our conclusion that four cases of hypernatremia were caused by the brain tumor. In an additional case, high concentration of sodium in serum was most likely caused by low fluid intake. In three of these cases, hypernatremia was completely or partially resolved. One patient died from bleeding from the tumor and another from extensive cancer and refusing the treatment for hypernatremia.

I hope this information will be useful to you. Please do not hesitate to contact us again if you require more information.

Sincerely,

S. R. Burzynski, M.D., Ph.D.

SRB/cf

Mr. BURTON. You have a lovely daughter, there, doctor, and I intend to talk to the gentlemen you talked about today.

Mr. CHEADLE. OK, sir.

Mr. BURTON. I'll call him today and if you want to stick around after the conclusion of the hearing, maybe we can talk to him together and see what we can do about that.

Mr. CHEADLE. We have to get a flight at 5:22.

Mr. BURTON. Well, we'll be talking to him, probably about 4. How's that?

Mr. CHEADLE. OK. That's fair. Thank you very much.

Mr. BURTON. As I understand it, your daughter can't survive any more chemotherapy. Is that correct?

Mr. CHEADLE. No. The doctors said she would never get through a second bone marrow transplant.

Mr. BURTON. Now, this cancer that she has, is it likely to return any time soon?

Mr. CHEADLE. Well, they say it normally comes back between the first and second year.

Mr. BURTON. And how long has she been free?

Mr. CHEADLE. The last time we checked was about 3 months ago. She was still in remission and we just returned from the hospital in San Antonio before I came up here, where they had done tests on her, but we haven't got the results of them yet.

Mr. BURTON. OK. How much money do you anticipate, if you can go to Dr. Burzynski's clinic, it would cost to help her?

Mr. CHEADLE. Would cost me personally, sir?

Mr. BURTON. Yes.

Mr. CHEADLE. Well, I think the startup cost and everything is about $17,000. And then, depending on which type of medication they give her, between $2,000 and $6,000 a month.

Mr. BURTON. Has he indicated to you what kind of success rate he's had with this kind of cancer?

Mr. CHEADLE. Sir, he has had one neuroblastoma cancer patient and they have gotten to 50 percent reduction in the tumor. However, this child, as I am told, had been through two bone marrow transplants and was essentially what I said, a basket case, when the child got there.

Mr. BURTON. So he's only had one case.

Mr. CHEADLE. He died of a secondary infection.

Mr. BURTON. But he thinks that there is a possibility it might help her?

Mr. CHEADLE. Yes, sir.

Mr. BURTON. OK. Well, we'll look into it at the conclusion of the hearing today and see what we can do to help. OK?

Mr. CHEADLE. Thank you very much, sir.

Mr. BURTON. Do you have any comments, professor?

Our third panel is Dr. Tammy Geurkink. Is that correct?

Dr. GEURKINK BORN. Yes.

Mr. BURTON. Geurkink, is that right?

Dr. GEURKINK BORN. Yes.

Mr. BURTON. Dr. Peter Matthiessen. Is that correct? And Dr. James Gordon. I only mentioned three. Did I have a fourth one?

Do you have opening statements? Why don't we start with you, Dr. Matthiessen? They have the name tags in the wrong place. All

set now? Why don't we start with the young lady. Dr. Born, would you care to go first?

Dr. GEURKINK BORN. Thank you very much, Mr. Chairman.

Mr. BURTON. If you can try to keep your comments to 5 minutes, I'll submit the rest for the record.

Dr. GEURKINK BORN. OK.

Mr. BURTON. We'll allow you a little latitude if you need to go longer.

Dr. GEURKINK BORN. OK.

STATEMENT OF TAMMY GEURKINK BORN, M.D.

Dr. GUERNKINK BORN. I'm here today to give you a perspective of the Access to Medical Treatment Act, as a board member because in spite of all the work done already on FDA reform, the job remains unfinished. And it remains unfinished in a vitally important area, as we've been talking about today, is patient access.

The FDA Reform bill passed last year did nothing to change the accessibility to alternative or complimentary medicine. In spite of what we've been told, it did nothing to increase patient access to unapproved therapies and it only codified existing law as it pertains to access to drugs already in the IND pipeline. I hope to give you a perspective from a practicing physician and as a member of a licensing board.

I am the vice-president of the Michigan Osteopathic Board of Licensing and Regulation. I have recently been appointed as a voting delegate to the National Federation of State Medical Boards. I have been practicing a scientifically based complementary medicine for 10 years in Grand Rapids, MI, and I have included for the testimony, a few of our more dramatic testimonials that have been written by our patients over the past years on the successes that they have experienced by combining the best of the alternative and traditional therapies.

My main objective today, however, is to give you a perspective that you may not have heard. A perspective that may explain to you why true patient access does not exist in this country currently. Two years ago I attended a National Federation of State Medical Board conference in Chicago, IL. The focus of the very first presentation was how to stop the practice of alternative and complementary medicine. As one Assistant Attorney General of California said at the meeting, "It's difficult for us to get patients to complain about these doctors, so we'll have to find a way to get them ourselves." He asked for a show of hands for how many States had prosecuted alternative medicine doctors. Many of the State reps raised their hands. Then he asked for a special conference of those to compare how they had been successful in prosecuting alternative physicians, simply because they practiced complementary medicine, not because they practiced bad medicine. Doctors may have been prosecuted for recommending nutritional or dietary therapies, not even necessarily controversial treatments. In fact, out of the 4 years that I have been on the board in Michigan, the number of alternative doctors that have been prosecuted for incompetence is extremely low. Many physicians practicing complementary therapies combine them with traditional therapies, allow their patients the best in each tradition. Many States have adopted policies that

have forced patients to seek medical care in other countries, or other States that may have more progressive policies. But if competent and well intentioned doctors are forced out of practice because they cannot bring a type of care that their patients need or want, patients will be forced to seek their health care from unregulated and unlicensed practitioners. Many good doctors with advanced degrees are unwilling to provide complementary therapy because of FDA and local medical society pressures. Despite overwhelming evidence that many complementary therapies are more effective and less costly, many doctors are unwilling to incorporate them into their own practices. Every doctor practicing good medicine should be able to incorporate complementary therapies into his or her own practice without fear of retribution from the FDA.

My job description as a State medical board member has been to protect the people of Michigan. I have been amazed at the swift and decisive actions which can take place in an effective medical board while policing its member physicians. And if you'd like me to tell you more about the process in which a physician can be disciplined, I'll be glad to go into that later. Physicians who harm their patients or in some way endanger the lives of patients are dealt with swiftly and effectively by State medical boards. The Access to Medical Treatment Act actually complements the medical boards and allows them to do their work much more effectively. As the bill demands that practitioners have solid reasons for believing that a therapy will work before providing it to anyone. No good doctor would want to jeopardize his license or livelihood by providing unethical treatments. The Access to Medical Treatment Act provides that the practitioner must know that a therapy will not cause harm. This provision can only help a State medical board while encouraging patient education and patient autonomy. Ensuring that a physician provides the best treatment for patients and the opportunity for the much needed research on complementary medicine are the AMTA's most notable provisions.

Many of my patients can afford the best of any medical care and they have chosen nontraditional therapies. While the FDA has done a great job at protecting patients from harmful drugs, it is unclear who they are protecting while prohibiting the further research and practice of complementary therapies. The patients who seek complementary therapies are not vulnerable, or likely to be taken in by doctors offering "quack cures." These patients are well informed and educated and the same therapy should be afforded patients who are unfortunate and uneducated. Patients need to be informed of all their options and make a decision in conjunction with their family, their doctor, and their spiritual guide as to the right treatment for them.

It has been my goal to offer the best and most affordable health care to the patients that I can. I love my job. I have been able to help hundreds and hundreds of patients live more full and enjoyable lives. I could tell you stories of many patients who have had their legs amputated and couldn't walk without getting chest pain. Many of them have been outraged that they were not given all of the available information about options either concerning their amputations or surgeries. I have many patients who travel to other countries to receive treatments for their cancer because no physi-

cian in the United States was able to offer treatment for them. The Access to Medical Treatment Act will allow patients who cannot afford to travel to Czechoslovakia for a cancer treatment or to Italy for an AIDS treatment a glimmer of hope that these treatments may one day be studied more thoroughly in the United States.

I believe I have a moral obligation to offer my patients information concerning all therapies available to them. I did not enter medical school knowing I would become interested in and practice complementary medicine. In fact, I had planned on being a surgeon. However, my own experience and insight has led me on a path which can only lead me forward. Forward into the future, and a future in which all patients are afforded the best medical care that we as physicians can offer.

Do you want me to stop?

Mr. BURTON. If you'd like, we can ask you questions and you can proceed later. We'll submit the rest of it for the record.

Dr. GEURKINK BORN. That's great.

Mr. BURTON. Thank you.

[The prepared statement of Dr. Geurkink Born follows:]

I am here today to give you a perspective from a Licensing Board member, because in spite of all of the work already done on FDA reform the job remains unfinished. It remains unfinished in a vitally important area - True Patient Access. Unfortunately the FDA Reform bill passed last year did little to change the accessibility to alternative or complementary medicine. In spite of what we have been told it did nothing to increase patient access to unapproved therapies and only codified existing law as it pertains to access to drugs already in the IND pipeline. I hope to give you a perspective from a practicing physician and as a member of a licensing board.

I am the Vice-President of the Michigan Osteopathic Board of Licensing and Regulation. I have also been recently appointed as a voting delegate to the Federation of State Medical Boards. I have been practicing scientifically based complementary medicine for ten years in Grand Rapids, Michigan. I have included for the record a few of the dramatic testimonials written by our patients over the past years of the successes they have experienced by combining the best of alternative and traditional therapies.

My main objective today, however, is to give you a perspective that you may not yet have heard. A perspective that may explain to you why true patient access does not exist currently. Two years ago I attended a National Federation of State Medical Boards conference in Chicago, Illinois. The focus of the very first presentation was how to stop the practice of alternative and complementary medicine. As one Assistant Attorney General of California said at that meeting -"It is difficult for us to get patients to complain about these doctors, so we will have to find ways to get them ourselves." He asked for a show of hands for how many states had prosecuted alternative medicine doctors and many of the state representatives raised their hands. Then he asked for a special conference to compare how other states had been successful in prosecuting alternative physicians simply because they were alternative not because they practiced bad medicine. Doctors may have been prosecuted for recommending nutritional or dietary therapies, not necessarily controversial treatments. In fact out of the four years that I have been on the board in Michigan, the number of alternative doctors that have been prosecuted for incompetence is extremely low. Many physicians practicing complementary therapies combine them with traditional therapies to allow patients their choice of the best in each tradition. Many states have adopted policies that have forced patients to seek medical care in other countries or other states that may have more progressive policies. If competent and well intentioned doctors are forced out of practice because they cannot bring the type of care that their patients need or want

patients will be forced to seek their health care from unregulated and unlicensed practitioners. Many good doctors with advanced degrees are unwilling to provide complementary therapies because of FDA and local medical society pressures. Despite overwhelming evidence that many complementary therapies are more effective and less costly, many doctors are unwilling to incorporate them into their own practices. Every doctor practicing Food medicine should be able to incorporate complementary therapies Into his or her own practice without fear of retribution from FDA and state medical boards .

My job description as member of the State Board of Michigan has been to protect the people of Michigan. I have been amazed at the swift and decisive actions which can take place in an effective medical board while policing it's member physicians. The Access to Medical Treatment Act would do nothing to undermine the board's authority. Physicians who harm their patients or in some way endanger the lives of patients are dealt with swiftly and effectively by State medical boards. The Access to Medical Treatment Act actually compliments these medical boards and allows it to do its work much more effectively as the Bill demands that practitioners have solid reasons for believing that a therapy will work before providing it to anyone. No good doctor would want to jeopardize his or her license and/or lively hood by providing unethical treatments. The Access to Medical Treatment Act provides that the practitioner Doubt know that the therapy will not cause hand. This provision can only help a state medical board while encouraging patient education and patient antinomy. Ensuring that physicians provide the best treatments for patients and the opportunity for the much needed research on complementary medicine are the AMTA's most notable provisions.

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It has been my goal to offer the best and most affordable health care to the most patients that I can. I love my job. I have been able to help hundreds and hundreds of people to live more full and enjoyable lives. I could tell you stories of patients who

had no hope; who had legs amputated or who could only walk twenty feet without getting chest pain and now are able to walk or were able to save their remaining leg from amputation. I have patients who have been outraged that they were not given all of the information available from their doctors. They were not given options concerning their amputation or their surgeries. I have many patients who have traveled to other countries to receive treatments for their cancer or other chronic ailments because no physician in the United States was able to offer this treatment for them. The Access to Medical Treatment Act will allow patients who cannot afford to travel to Czechoslovakia for a cancer treatment or to Italy for and AIDS treatment, a glimmer of hope that these treatments may one day be studied more thoroughly in the United States.

I believe that I have a moral obligation to offer my patients information concerning all therapies that are available to them. I did not enter medical school knowing that I would become interested in and practice complementary medicine. In fact much the opposite. I planned on being a surgeon. However, my own experience and insight has lead me on a path which can only lead me forward. Forward into the future. A future in which all patients are afforded the best medicine that we as physicians can offer.

In a recent Grand Rapids Press article a study was cited in which the administration of significant doses of Folic Acid and Vitamin E were tested in thousands of nurses. The death rate from heart attacks dropped more than 50%. With The Access to Medical Treatment Act the recording of beneficial medical treatment is required, therefore, enabling this very significant information to be disseminated in a much more timely fashion, saving hundreds maybe thousands of lives. The research studies encouraged by The Access to Medical Treatment Act will significantly impact the lives of the American public. Lives will be saved and health care will be administered in a more cost effective and efficient manner. What traditional therapy can offer a 50% reduction in deaths due to heart disease? Yet, are physicians routinely recommending that their patients take high doses of Polic Acid or Vitamin E? Many are not! Rich and poor patients, educated and uneducated people, professional and laborers will all benefit from the research encouraged by this bill.

If the United States is to continue to offer the best medical care in the world we must look at the beet of non-traditional and traditional therapies. Fifteen years ago when I entered medical school we had no courses on nutrition or alternative medicine. In fact there were no lectures in all four years of medical school on the importance of diet and nutrition in promoting a persons health or preventing a

disease. Now a few medical schools across the country are offering courses in alternative medicine. While this is a great beginning, I can see how the impact of The Access to Medical Treatment Act could be much more profound for our already aging and ailing Medicare population. Our illness based curriculum and the attitude of "you give me a symptom and I'll give you a pill" by many medical practitioners is not good medicine and is in many ways unethical today. Many illnesses and diseases can be treated without costly medicines or surgeries if the public is aware of ways in which they can take responsibility for their own health care and their own treatment. Everyone will benefit. While it is very important to teach doctors how to treat a patient when they are ill, it is more important to teach them how to maintain health and wellness. If patients are given the freedom to steer the course of their health care, assisted by trained health care providers everyone benefits.

Mr. BURTON. One thing that you said that struck a harmonious chord, you were talking about the amputations where people later found out there might have been an alternative therapy.

Dr. GEURKINK BORN. Yes.

Mr. BURTON. And it reminded me of the movie, "Kings Row" with Ronald Reagan where he woke up and they cutoff both his legs unnecessarily and that seems analogous to some of the things the FDA is allowing to happen when people's lives are at stake and it just seems unfortunate.

Dr. GEURKINK BORN. That's right.

Mr. BURTON. Anyhow, we'll get back to you in just a moment.

Dr. GEURKINK BORN. Thanks.

Mr. BURTON. And we'll submit the rest of your statement for the record.

Dr. GEURKINK BORN. OK.

Mr. BURTON. Dr. Matthiessen.

STATEMENT OF PETER MATTHIESSEN, M.D.

Dr. MATTHIESSEN. Mr. Chairman, members of the committee, Winston Churchill used to say, "Americans can always be trusted upon doing the right thing, after they've tried everything else." So we Germans have always admired the American way of trial and error and so I feel very honored to be able to speak before you today about the status of complementary, alternative and nonconventional medical practices in Germany today.

Allow me to introduce myself briefly. My name is Peter Matthiessen, I have been wearing two professional hats for many years. Trained as a specialist in neurology and psychiatry, I am active in direct patient care as chief of a medical service in a large community hospital in Herdecke and in addition, I am active scientifically as Head of the Department of Medical Theory that is monitor medicine and complementary medicine at the University of Witten/Herdecke.

The Herdecke Hospital is a community hospital with close to 500 beds and encompasses all customary medical and surgical specialty practices. Established in 1969, it rapidly became known all over Germany as the Herdecke Model and is since then the best known medical institution which includes complementary and alternative medicine in its services. In this hospital we have attempted to create care structures and a working atmosphere which have as their goal the care of the individual patient so that diagnosis and therapy is guided by the person's bodily, psychological and spiritual dimensions as well as their individual biography.

Without exception the knowledge basis of all our physicians practicing there is that of modern scientifically established medicine. But beyond that, we attempt to come to an extended, more encompassing comprehension of health, illness and healing, and thus concern ourselves also theoretically and practically with various modes of complementary medicine. In acute patient care we then also utilize herbal medicines, remedies of anthroposophical medicine, homeopathic remedies, as well as external applications, massage, baths, and so on. And beyond that we utilize various artistic therapies such as music therapy, painting therapy, curative eurythmy. It is our intent with such therapies to activate the patient, as much

as possible, in actively participating in overcoming an illness and insofar as possible, re-establish health.

Not only because of its innovative character and the countrywide interest in receiving care at this hospital, but also because of foreign including American interests, our government has supported a further expansion of the hospital to the tune of 130 million DM.

Now, the other institution I serve as a professor is the University of Witten/Herdecke, the only private university in Germany, established in 1983 by a group of established scientists. They had the goal to engage not only in mainstream scientific pursuits, but to extend the spectrum of scientific investigation; to followup also unconventional points of view and begin to cultivate a rational scientific pluralism. Our faculty and students place upon themselves the demand to followup questions and problems from various theoretical or philosophical and empirical perspectives and cultivate various methodologies.

Medicine in Germany is not a uniform edifice of theory and practice, but rather a highly pluralistic edifice. This became especially evident in 1976 during the intensive and highly controversial discussion preceding and surrounding the passage of our new Medication Law.

The main focus of the debate at the time was the demonstration of therapeutic effectiveness. Our legislators acted wisely, at least from my vantage point. They did not take upon themselves the role of judging the adequacy of science, but rather spoke in support of the actually existing pluralism in medicine. I am quoting from the report of the committee on the legislation in 1976.

> It is the unanimous view of the committee that it must not be the task of the legislator to give preference to the methods of one of the competing lines or schools of therapy in determining effectiveness of a medication. Rather, the committee was guided by the political goal that in the guidelines for acceptance of a medication there must be clearly reflected the existing scientific pluralism.

A consequence was the establishment of a commission for each of the so-called "special lines of therapy," namely phytotherapy, homeopathy and anthroposophical medicine. The task of the commissions being "the evaluation and preparation of scientific data in accordance with the standards and experiences of the corresponding lines of therapy and the formulation of indications for use." The implementation of this expert advice is then taken on by the Federal Department of Health.

The legislative intent and act, to permit nonconventional modes of therapy to exist besides the conventionally established medical practices was then also reflected in the German insurance legislation which applies to the institutions providing health insurance to 90 percent of the population. Accordingly, it is mandated that "treatment methods, remedies, and medications of the special lines of therapy are not excluded from reimbursement." The special lines of therapy must also fulfill the criteria of indication, necessity, cost effectiveness, as well as quality and efficacy.

Let me say a few words about the situation of research and support for research in Complementary and Alternative Medicine—in short CAM. Despite the widespread presence of those methods in the health care of our population, CAM was only marginally represented at German universities. Thus no adequate structural or

personal resources could be developed for efficient and competitive scientific investigation.

For this reason and in view of widespread and ever increasing interest in nonconventional methods of treatment, the Federal Government supported from 1981 to 1996 investigations focused on "Nonconventional Methods of Cancer Treatment" and beyond that, since 1986 a further project covering "Nonconventional Medical Lines of Therapy."

Under my direction a working group at Witten/Herdecke University was given the mandate to: No. 1, provide an analysis and catalogue of the status of scientific research in CAM; No. 2, to establish a directed and efficient method of supporting research in CAM; and, No. 3, to coordinate and support the various scientific endeavors.

A desired goal was to support serious empirical scientific endeavors in CAM and at the same time to separate the what from the chaff.

What have been our experiences so far?

Sooner than we hoped we have arrived at a good overview regarding which direction of investigation are valuable for establishing the scientific basis of CAM, and thus potentially supplement and enrich conventional medicine. We have also gotten a good overview of questionable, even fraudulent procedures for which no plausible theoretical basis existed and where there was not even an interest in unprejudiced investigation, for it became evident that such practices were not able to meet the criteria for research proposals.

Quite aside from the research activities and results of obtained, the most important result of the efforts is that a dialog has been set in motion, a dialog between different modes of thinking and acting in medicine. And this has led to a greater tolerance and exchange of various points of view, theoretical pursuits, and above all different question, so that limits and possibilities are more amenable to evaluation and mutual cooperation is closer at hand.

On the basis of our experiences in Germany with CAM in public health care, I would like to recommend to the committee that CAM is subjected to careful review and evaluation. However, I would caution that the legislative requirements for proof of efficacy in approving therapies and medications of CAM are not too narrow, constraining and restrictive. Room for different schools of therapeutics should be taken into account. Care must be taken not to endanger the development of potentially valuable therapies or methods of providing health care for the public. That would lead to an impoverishment through paradigmatic uniformity in medicine—established by legislation.

It is our experience that where the legislative framework is provided for the unfolding of a pluralistic medicine—which already exists de facto—the ensuing critical but open dialog is most efficient in distinguishing valuable and promising therapies from fraudulent methods. Thus it is in all our interests that we work for those who are ill in an open, honest and critical fashion, the goal being to help the ill human being.

I thank you for your interest and would like to let you know how impressed I am by your pursuit of providing legislation for a free pluralistic medicine appropriate for the human individual.

Thank you.

[The prepared statement of Dr. Matthiessen follows:]

Mr. Chairman, Members of the Committee, Ladies and Gentlemen:

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The legislative intent and act, to permit nonconventional modes of therapy to exist besides the conventionally established medical practices was then also reflected in the German insurance legislation which applies to the institutions providing health

insurance to 90% of the population. Accordingly, it is mandated that "*treatment methods, remedies and medications of the special lines of therapy are not excluded from reimbursement.*" The special lines of therapy must also fulfill the criteria of indication, necessity, cost effectiveness, as well as quality and efficacy. These too are to be judged on the basis of scientifically reproducible data.

However it was only a few months ago, some twenty-one years after the passage of the original legislation, that the German legislative branch expressly established regulations that require the special points of view and experiences of the various lines of therapy to be taken into account when the state of prevailing scientific knowledge is judged.

Let me say a few words about the situation of research and support for research in Complementary/Alternative Medicine (in short CAM). Despite the widespread presence of those methods in the health care of our population, CAM was only marginally represented at German universities. Thus no adequate structural or personal resources could be developed for efficient and competitive scientific investigations.

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In view of the methodological aspects of proving effectiveness we have come to realize that in many cases the randomized controlled studies may not be appropriate. This is so because of therapeutic concepts which are highly individualized and also in view of the ever increasing autonomy of patients who,

at least in Germany, are ever less willing to permit themselves to be randomized, thus making good randomized studies all but impossible.

Thus other study designs had to be developed and applied which were acceptable to representatives of both conventional and non-conventional medicine so that positive results could be acknowledged by established scientists and negative results would be taken seriously by defenders of non-conventional therapies.

Quite aside from the research activities and results obtained, the most important result of the efforts is that a dialogue has been set in motion, a dialogue between different modes of thinking and acting in medicine. This has led to a greater tolerance and exchange of various points of view, theoretical pursuits, and above all different questions, so that limits and possibilities are more amenable to evaluation and mutual cooperation is closer at hand.

Despite the great significance which science has in medicine, not everything which is fruitful in real life and in the individual's care in medical practice can be scientifically established and proven. Science in medicine is never an end in itself but always has only an ancillary function; it has the task to support and improve the training, contexting and careful judgment by the therapeutician. The Art of Healing however is always more than an applied science, namely it is the Art which strives to comprehend the uniqueness of each individual and to provide her with the best possible help in a situation which may be utterly unique, unexchangeable and never to recur in the same way.

On the basis of our experiences in Germany with CAM in public health care, I would like to recommend to the Committee that CAM is subjected to careful review and evaluation. However, I would caution that the legislative requirements for proof of efficacy in approving therapies and medications of CAM are not too narrow, constraining and restrictive. Room for different schools of therapeutics should be taken into account. Care must be taken not to endanger the development of potentially valuable therapies or methods of providing health care for the public. That would lead to an impoverishment through paradigmatic uniformity in medicine -- established by legislation.

It is our experience that where the legislative framework is provided for the unfolding of a pluralistic medicine (which already exists *de facto),* the ensuing critical but open dialogue is most efficient in distinguishing valuable and promising therapies from fraudulent methods. Thus it is in all our interests that we work for those who are ill in an open, honest and critical fashion -- the goal being to help the ill patient.

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COMPLEMENTARY THERAPIES IN MEDICINE

Research

Unconventional medicine in Germany

A report on the situation of research as basis for state research support

B. Rosslenbroich, S. Schmidt and P.F. Matthiessen

SUMMARY. **In Germany methods of unconventional medicine are widely used, in accordance with long tradition, especially by general practitioners and in some private clinics and sanatoriums. Their application is mostly based on practical experience, since only a few areas have been scientifically evaluated. Now the Federal Ministry for Research and Technology (BMFT) has announced that it will support future research projects in unconventional medicine. In preparation, the BMFT engaged a research team at the University of Witten/Herdecke to analyse the situation of research in unconventional medicine and to evaluate the problems and possibilities for research in Germany. This paper summarizes the results of this work, and includes the recommendations for state research support that were made to the BMFT.**

INTRODUCTION

In the Federal Republic of Germany (FRG), as in other European and non-European countries, the use of unconventional medicine (UM) has been on the increase in recent decades.

The term 'unconventional medicine' describes forms of treatments and diagnostic procedures that are not taught and scientifically evaluated at the universities and that are usually excluded from research funding.

There is not only a growing number of patients demanding unconventional therapies, but also an increasing amount of interest on the part of medical practitioners.[1–4]

The application of UM therapies is at present usually based on practical medical experience, though some have a background of specific medical systems and hypotheses. Despite their increasing popularity, most types of UM are not yet accepted by conventional medicine. For this the reasons are manifold, and include the fact that few treatments in UM have been scientifically evaluated.

Against this backdrop, in 1985 and 1990 the German parliament entrusted the government with the task of assisting the scientific evaluation and future development of UM by means of targeted research support. In preparation for this, a team at the University Witten/Herdecke was engaged by the Federal Ministry for Research and Technology (BMFT) to analyse the situation in UM research, to investigate the research activities and research possibilities in Germany, and to make recommendations for state support of research.

With the help of written and personal interviews with scientists and physicians, data and commentaries on UM research were collected over a period of approximately 3 years. Scientists and study groups were found who were ready and able to carry out research projects in UM on

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a scientific level high enough to qualify for state sponsorship. In addition, a survey of relevant international scientific literature was drawn up. The findings of these investigations have been published in German. The main topics and various other therapies are discussed in detail, including a list of about 1300 references to scientific papers from the field of UM.[5] The following text gives a short summary of the findings of this work.

GENERAL SITUATION OF UM IN GERMANY

Surveys[4] show that about 70% of the German population have used natural remedies at some time or other. 52% are convinced of the effectiveness of natural remedies. From the beginning of the '70s till the end of the '80s the proportion of those regularly using natural remedies rose from 30%–46%. An enquiry among general practitioners and internal specialists[2] revealed that of those questioned 34% frequently, 48% seldom, 10% only at patient's request, and 8% never prescribe natural remedies. Between 60–70% of all general practitioners prescribe natural remedies regularly or sporadically. According to information supplied by the BAH (National Association of Drug Manufacturers), the proportion of herbal remedies on the German pharmaceutical market currently stands at between 20% and 30%. Taken from the whole German turnover in pharmaceutics, sales of phytotherapeutics increased from 7.7% in 1985 to around 10% in 1989.

Acupuncture associations say that an estimated 6000–8000 physicians use acupuncture. Besides its use in the field of UM, acupuncture is also used to some extent in conventional pain therapy. Thus about 77% of the outpatient pain clinics, including those of some universities, use acupuncture. According to information from homoeopathy associations there are about 2000 practising homoeopaths, and altogether about 16000 therapists who prescribe homoeopathic remedies. While complementary medicinal procedures are predominantly used by general practitioners, they are also in use at some hospitals and sanatoria.

Although the various types of UM are very heterogenous, unconventional therapists see the implementation of 'natural therapy' principles in many medical procedures of UM as a common denominator. Natural therapy methods aim at an active participation of the organism and the harnessing of its natural capacity for self-regulation, adjustment, and regeneration. They try to support and direct the 'self-healing capability' of the organism. In contrast, the methods of 'artificial therapy' involve assigning to the organism a passive role, aimed primarily at eliminating pathological changes or their imputed causes, i.e. by operative or chemical intervention, pharmacological steering back to normal, or by means of artificial replacement of substances, functions or organs.

The therapies with the widest distribution and most extensively recognized medical effectiveness are summarized in the first section of Table 1. In addition the second section shows forms of therapy that have some distribution in Germany. Finally there are many other less significant forms of therapy in UM which are not listed here.

Table 1 Methods of UM

- Phytotherapy
- Homoeopathy
- Anthroposophical Medicine
- Physiotherapy and Balneology[1]
- Acupuncture
- Special dietary systems
- Neural Therapy
- Oxygen and Ozone Therapies
- Electroacupuncture according to Voll
- Bioresonance Therapy and Mora-Concept[2]
- Thermoregulation Diagnosis[3]
- Chiropractic Therapy
- Microbiological Remedies[4]
- Organic Remedies[5]

[1]In their traditional therapeutic use as natural medicine, e.g. as Kneipp-Therapy; [2]Diagnostic and therapeutic procedures that try to make use of patients own electrical oscillations; [3]Diagnosis by means of measuring the relative surface temperature of the skin in various locations; [4]Medicines produced from microorganisms; [5]Medicines produced from animal or human organs.

The following text gives short summaries of the situation in the research into UM in Germany, with reference to the first five listed therapy forms. The present stand of scientific evaluation is discussed taking into consideration the available international literature, of which only examples or reviews are cited in this paper. Detailed information can be found in the above-mentioned publication.[5]

PHYTOTHERAPY

In Germany the definition phytotherapeutics is used to describe extracts from plants which are used as remedies. The constituents can be obtained either from the whole plant or from parts of it. Thus they usually comprise a very complex chemical substance. In Germany phytotherapeutics are manufactured by the pharmaceutical industry and subject to German laws pertaining to pharmaceutical products.

The use of botanicals in medication has a long tradition and modern therapies have evolved out of it. Chemical compounds have been isolated from plants and developed into modern drugs. Besides this, the traditional use of herbal medicines is still widespread.

Conventional pharmacologists today tend to refuse to do research on herbal remedies or to recommend them

for drug therapy because of the heterogenity of their compounds. Thus herbal medicines are widely rejected by university clinics and almost entirely excluded from scientific appraisal, especially in the field of clinical research. For this reason phytotherapy belongs to UM, although it is widely used not only by complementary therapists, but also by conventional general practitioners.

A main area of scientific evaluation of phytotherapy is pharmaceutical research. Modern technology makes it possible to gain exact pharmaceutical knowledge about medical plants. Every year about 1500 new herbal components are isolated worldwide. However, despite intensive research, up to now only about 10% of the 400 plants which are of importance to European phytotherapy have been analysed exactly.[6,7,8]

Pharmacological studies are another area of research. Thanks to international research and using modern research methods, well-substantiated findings have been achieved for a number of medicinal plants and their components. Efficacy mechanisms have been successfully studied e.g. in Ginkgo biloba, Peppermint oil, Menthol, Garlic, Echinacea, Silybum marianum, Whitethorn and Camomile. Another example is the insight into the antiphlogistic effects of some plants and their compounds. Nevertheless, pharmacological knowledge about many phytotherapeutics is still fragmentary. In addition to the insufficient knowledge about pharmacodynamics and efficacy mechanisms, there is little information about toxicity, especially for long-term use of drugs or mutagenity and carcinogenity. The question as to the relevance of research on isolated components for their use in phytotherapeutics, and the feasability of developing pharmacological models for full extracts, is posing a general methodological problem. Fortunately, research in this field has been increasing for the last 10 years in Germany as well as in other countries.[9,10]

Up to now, very few phytotherapeutics have been studied in clinical trials, with only isolated research or none at all for most herbal remedies in use. The small group of relatively thoroughly clinically tested phytotherapeutics include Ginkgo biloba, Silybum marianum, Garlic, Crataegus, and Peppermint oil.[11–15]

Further examples of plants for which clinical research papers are available include Valeriana, Podophyllum, Ginger root, Horse chestnut seeds, Ruscus, Serenoa repens, Pygeum africanum, Willow bark, Evening primrose oil, Tanacetum, Mistletoe, Phyllantus amarus, Panax ginseng and Senna leaves.

Many of these research papers have been criticised, for the most part because of unsatisfactory study designs. For example, in the case of Ginkgo biloba the quality of study designs, the relevance of basic studies on the clinical application and other problem areas is currently causing heated controversy in Germany. One of the main reasons for this is that Ginkgo biloba is one of the most frequently prescribed medicines in Germany, despite the fact that pharmacologists do not recommend its use in therapy. This explains why Ginkgo biloba trials attract so much more attention than those of many other remedies, and demonstrates that controversies of this nature are frequently caused by factors other than scientific ones.

Very few phytotherapeutics have been as well researched as Peppermint oil for patients with irritable colon, for which there are pharmacological and clinical studies, which in quality and quantity convincingly substantiate their usage, including the recommended dosage.[9,16]

Another example are the clinical studies on Whitethorn, which clearly indicate an efficacy in the treatment of cardiovascular diseases. However, clinically relevant information as to the optimal dosage, dosage frequency or, for instance, the appropriate stage of cardiac insufficiency at which Whitethorn could be most effectively administered, is not provided.[9,11]

Despite their small number, the available clinical studies on phytotherapy demonstrate that phytotherapeutics can be scientifically researched, thereby producing interesting results for medical science.

With reference to the research groups active in Germany, there is efficient pharmaceutical research into phytotherapeutics, and this research contributes to the international knowledge in this field. At universities, work on the components of medicinally relevant plants is undertaken by the institutes of pharmaceutical biology, some of which are directly concerned with their practical application in phytotherapy. There is very little pharmacological research in Germany on herbal drugs. The few existing research groups investigating pharmocological problems are working on a high level on both in vitro and in vivo models, some groups working at university institutes and others in the pharmaceutical industry. They provide the field of phytotherapy with valuable information, but they are quantitatively too few to deal with the large number of herbal remedies in practical use and their pharmacological problems.

Systematic clinical research into phytotherapy in Germany is very rare, although some of the available clinical studies are performed here. Very few clinical research groups at universities are involved in work on phytotherapeutics, and there are no systematic, long-term research programmes investigating problems arising in clinical practice. Most clinical trials are research assignments commissioned by the pharmaceutical industry, and are conducted primarily for the requirements of state registration of drugs. This kind of research throws up many problems, for instance, the clinical relevance of the investigation is frequently unclear, the trials are often limited in size, and there is publication bias, which is also the subject of intensive discussion in conventional medicine. This research is having almost no influence on the practical application of phytotherapeutics, such as indication, dosage, or other details concerning their usage. Phytotherapeutic textbook descriptions are mostly

still based on traditional recommendations and the practical experience of individual doctors.

HOMOEOPATHY

In Germany homoeopathic medicine is usually practised by general practitioners, some of whom specialize entirely in this field, while others incorporate it into their usual medical practice. There are also some clinics and sanatoria which make use of homoeopathy but, as in other countries, it continues to be excluded from the universities.

In Germany there is a long homoeopathic tradition, and a number of homoeopathic associations are active in the collection, processing and structuring of homoeopathic knowledge and experience. Vocational training and further education in homoeopathy are, for the most part, conducted by these associations in their own educational institutes. However, these activities are based more on the homoeopathic therapy experiences, while work on a modern scientific level has not yet become established.

In the international research field there have been many and varied approaches to basic research studies, which attempt to prove principal work mechanisms and the effects of potentised medicines, but only a few of these have as yet been systematically reproduced.

Some of the best research projects have been carried out by a French team on the subject of allergology using the basophil degranulation test, and it is common knowledge that the results of these tests triggered off lively controversy among scientists.[17]

The second important approach was by a German team, who examined various enzyme systems in rats after administration of potentised substances.[18] This approach was carried out using modern biochemical methods and recently also in blind conditions. It is a significant step in biochemical research into the question of potentised medicines, and needs to be urgently followed-up, reproduced, and further developed by other research teams.

The situation as far as clinical studies is concerned is dominated by individual studies on widely differing indications and medicines. There have been hardly any systematic studies on specific indications and illnesses. Study designs which fulfill the special needs of homoeopathic diagnosis and therapy are the exception rather than the rule.

Suggestions have been made for methods of research into homoeopathic treatments using controlled studies.[19,20] These involve the homoeopath incorporating his usual diagnosis and suggestions for treatment; when the chemist supplies the prescription the medicine is given randomized as verum or placebo. A study on migraine using this design is being carried out in Munich. The advantage of this study design is that homoeopathic pharmaceutic procedure does not have to be disturbed.

Further suggestions have been made on the subject of more individualized study designs. It would, for example, be possible to recruit the patient groups for clinical trials according to their homoeopathically specified typologies, in order to form more homogeneous collectives. This procedure could serve as a model for the study of homoeopathic medicines and the homoeopathic system of diagnoses and therapies. The relevance of single-case study designs for clinical trials in homoeopathy has as yet to be evaluated.[21]

Despite all these difficulties there are accounts in the international literature of a number of promising controlled studies, which demonstrate that clinical trials of homoeopathy are possible.[22,23] Particularly, the methodologically efficient studies are additionally able to provide first evidence for the efficacy of homoeopathic treatments, thus confirming the practical experience of physicians. Up to now about 115 controlled clinical trials have been carried out in the field of homoeopathy.

Differentiated judgements will, however, only be made feasible by further studies, which if possible should include a systematic processing of exemplary indications including their reproduction. The meta-analysis of Kleijnen et al[22] demonstrates particularly clearly the need for methodological improvements. However, their analysis of controlled clinical studies on homoeopathy did bring evidence for its effectiveness.

Some of the available clinical studies on homoeopathy were conducted in Germany. There are a number of research teams in Germany working either in basic research or in the field of clinical research. But as isolated groups they are not able to solve the problems connected with homoeopathic research through continuous interdisciplinary efforts.

ANTHROPOSOPHICAL MEDICINE

Anthroposophical medicine is based on the scientific and philosophical principles of anthroposophy, which was founded by Rudolf Steiner at the beginning of this century. Steiner first created an epistemological basis and then went on to elaborate a description of nature and man, including the areas of soul and spirit as well as those of physical and organic functions. On this basis he developed new aspects for various areas of practical life, including the medical field.[24–27]

Today anthroposophical medicine is practised by general practitioners and also in nine hospitals and five sanatoria in Germany. In the hospitals, especially, it is integrated with all forms of conventional medicine.

Anthroposophical medicine does not consider itself to be opposed to conventional medicine, but rather as an extension of it. It is founded on the recognition of four distinct aspects of the human being: the physical body, the life organisation which includes all organic functions,

the soul aspect and intelligent self. While inter-related, none is reducible to the laws of another, for instance the principles of physics cannot be used to describe the principles of life and organic functions, as conventional science predominantly tries to do. Anthroposophic science attempts to develop an original biology embracing the special organization of living organisms and principles of life. The attitude to health, sickness and healing takes into account this 'holistic' concept.[25–28]

In medicinal therapies anthroposophical medicine uses medicaments obtained mostly from mineral, botanical and sometimes animal sources. Potentised medicines are also used. In addition there are a number of non-medicinal therapies, such as special kinds of massage and physical treatments, medicinal baths and various artistic therapies.

Research into anthroposophical medicine is mainly devoted to attempts to understand the relationship between the physical-organic basis and the soul and spiritual aspects of human beings, and their significance in the processes of health and sickness. In addition, the development of new medicines involves a qualitative assessment of their properties to expand the conventional analytical methods.

The research into the efficacy of mistletoe compounds (Viscum album) in the treatment of cancer using various procedures is exemplary. In addition to conventional analytical studies of its active components, attempts are being made to find qualitative characteristics for their application in tumour therapy. Pre-clinical research has produced evidence for the tumour-relevant reactions of mistletoe components, and a number of clinical studies confirm the clinical efficacy of mistletoe in cancer. However, in practice it is still not completely understood why some patients react better than others to mistletoe therapy. In the field of anthroposophical medicine this is seen as an indication of the necessity for individualized research into therapies.[29,30,31]

An important area in basic research concerns the organization of rhythmic functions in human beings and the chronobiological aspects of the processes of sickness and health. The aim is to gain understanding of the organic-physiological principles and also the progress of disease in a functionally dynamic way, in order to understand the organism's reactions and consequently its healing capability. Chronobiological research methods have been successfully used in clinical studies leading to new diagnostic and therapeutic criteria, and for assessment of the course of disease. This has resulted in a number of clinical studies on cardiac physiology and on the efficacy of anthroposophical remedies.[32,33,34]

In addition there is a series of studies which are not prospective randomized trials but predominantly collective case reports on anthroposophical treatments, such as for diseases like sarcoidosis, pseudocroup, memory and concentration problems in old age, otitis media. Most of the available studies are from Germany.

Research groups are usually affiliated to anthroposophical hospitals, some within their medical units and some with their own institutes for basic research.

PHYSIOTHERAPY AND BALNEOLOGY

In Germany the origins of physiotherapy can be traced back to the 19th century 'natural medicine' movement which, as it developed, offered an alternative to the contemporary school medicine. Its aims were to use only natural factors in therapy, such as the use of hot and cold water for ablutions, baths and douches, exposure to sunlight, physical exercise, exposure to various climates, at high altitude or at the seaside. Balneology, the science of the application of healing waters and their therapeutic effects, is frequently used in close coordination with physiotherapeutical treatments.

Table 2 Some forms of treatment in physiotherapy

Physical exercises and gymnastics
Kinesitherapy
Massages
Superficial thermotherapy
Superficial cryotherapy
Electric stimulation therapy
Deep heat therapy (diathermy and therapeutic ultrasonic)
Hydrotherapy
Climatotherapy
Heliotherapy
Combinations of the above therapies
Parts of manual therapy, chiropractics, neural therapy
Embrocations with essential oils, induced sweating, tea preparations, inhalations

Besides the classical forms of therapy, modern physiotherapy integrates some new treatments, which have been developed from modern technical advancements. The most important forms of physiotherapy are shown in Table 2. Physiotherapy is widely use in combination with other treatments. Kneipp therapy, for example, which was founded by the German priest Sebastian Kneipp (1821–1897), today consists of 5 elements: hydrotherapy, exercise therapy, phytotherapy, dietetic treatment, and Ordnungstherapie (regulative therapy).

Today many forms of physiotherapy treatment are fully integrated into conventional medicine and are exemplary for the integrations of parts of naturopathy into conventional medicine. For this reason they cannot be categorically labelled unconventional. However, there is always a certain tendency to ostracize these therapies, especially in the scientific field, and there is no state support for physiotherapy and balneology in Germany, even though physiotherapeutic treatment is an integral part of basic health care, such as in rehabilitation or the treat-

ment of rheumatism. Thus it is very difficult to conduct high quality research in this field and there are considerable gaps in the scientific appraisal of many of these methods.

There are three additional features which distinguish some areas of physiotherapy and balneology from conventional medicine:

1. Many of their methods depend on the support and stimulation of autonomous healing agencies for their efficacy, and must therefore be defined as natural therapies.
2. Diagnosis and treatment are frequently influenced by typological features, i.e. they are adjusted according to the patient's personal characteristics.
3. Some diagnostic approaches and therapies use the principles of cuti-viscero and viscero-cutaneous reflexive connections, which play a very important role in many complementary therapies.

It can be concluded from the above description that physiotherapy and balneology stand between 'school medicine' and naturopathy.

International publications report on research projects investigating the working principles and mechanisms in some of the physiotherapeutic methods.[35,36] These include research on transcutaneous electrical nerve stimulation, hydrotherapy, UV-ray treatment, electric stimulation, massage, physical exercise, thermotherapy. Most of the studies investigate the acute physiological and pathophysiological reactions to physiotherapeutic treatments, some of them using an animal model, some in the human organism. Studies investigating the long-term effects of those therapies are less frequent. In many cases it has not yet been possible to bridge the gap between these physiological findings and the clinical efficacy of therapies.

For some aspects of physiotherapy and other therapies, experimental clinical examinations and case studies according to the principles of natural therapy have resulted in the concept of a therapeutic physiology evolving in Germany. Against the background of spontaneous organic rhythms, the reactive periods and the timing and structure of adaptive responses to therapies which demonstrate that reactions to adequate therapeutic manipulations always have a periodic structure, have been observed with the methods of chronobiology. These findings demonstrate that it is possible to select adequate moments in the reaction process of the organism for appropriate manipulations.[32,33,34]

Controlled clinical studies have been carried out in a few therapeutic areas, but generally speaking clinical research is rare.[37] For example, there are studies which indicate that in comparison with the treatment with standard antihypertensives, minor hypertension can be lowered adequately by means of specific physical exercise programmes. Comparative studies have been undertaken for certain kinds of lipometabolic malfunction, respectively raised levels of triglyceride and cholesterol.

Other examples are controlled studies into hydrotherapy.[37] They show that clinical research on a level corresponding to modern research standards is possible and advisable. Papers on clinical research also exist e.g. for thermotherapy, electric stimulation therapy, exercise treatment and respiratory physiotherapy.

Also, physiotherapy techniques are often based solely on practical clinical experience, and that is presumably the reason why much of their potential within the framework of contemporary medicine is overlooked to some degree in favour of drug therapies with their frequent negative side-effects. There are a few research groups working on physical medicine in Germany, some of them affiliated to universities, some to medical centres. Currently a number of new groups are emerging. Individual institutes have a long tradition in research on physiotherapy and balneology and have contributed considerably to present scientific knowledge in these areas.

ACUPUNCTURE

Acupuncture is widely used in Germany, partly by specialized acupuncturists, partly by naturopaths and additionally in outpatient pain clinics. Some of the physicians using acupuncture use it against a background of traditional Chinese medicine, some more in the context of Western diagnosis and therapy. Despite the integration of acupuncture into pain therapy it is not accepted by university teaching hospitals.

The situation in international acupuncture research is handicapped by the fact that it has not yet been possible to bridge the gap between Chinese philosophy and medicine and the Western scientific view of organisms. Despite various scientific approaches, the significance of vital energy and meridians, and the specificity of acupuncture points have remained unclarified. This may explain why acupuncture research so far limits itself to two areas: on the one hand the search by means of physiological and pathophysiological models for effects of needle treatments, and on the other, the proof of the therapeutic success of acupuncture treatment on humans.

In the field of current pain research there is research into how far the pain threshold can be modulated by means of influencing synapses through acupuncture. Discharges of neural transmission have been measured on various levels in the central nervous system. Another research priority concerns the subject of segmental and non-segmental reflexive contacts within the human body. The trigger-point-concept is a topic in this area which has also undergone some investigation.[38]

Clinical research into acupuncture includes a great number of non-controlled studies as well as a series of controlled trials. Migraine, headaches and back pain are

the indications which have been investigated most extensively. Altogether there is some good evidence from clinical trials for short-term effectiveness of acupuncture in some pain conditions, while the evidence for long-term effectiveness in pain-treatment is still weak. Trials indicating effectiveness in chronic pain are contradicted by negative findings in other trials. Further clinical studies, some with interesting findings, have examined the use of acupuncture in indications like asthma, cardiovascular diseases, sinusitis, pollinosis, obstetrics and gynaecology, as an anti-emetic, and during withdrawal from addiction.[39–46]

In the majority of studies problems are posed by the unsatisfactory quality of their research methods and the inadequate presentation of findings in publications. It is consequently not yet possible to make definitive scientific statements on the therapeutic efficacy of acupuncture. This situation demonstrates the gap between the practical experience of a considerably growing number of physicians and an adequate scientific evaluation. There is an urgent need for properly conducted clinical studies to make possible a more accurate assessment of the therapeutic relevance of acupuncture.

In future clinical trials it will be essential to define the system of acupuncture being used in treatments, as to whether they are using the classical Chinese or Westernized acupuncture or any forms in between. These and other details have been widely neglected in clinical studies, although attention to them is a prerequisite for adequate judgement of findings and for study comparability.[37]

In Germany there are some research teams engaged in clinical research on acupuncture, most of them being based in outpatient pain clinics at universities, but the majority of clinical research papers do not come from Germany. Up to now it was not possible for research teams to develop the necessary know-how in continuous work over a period of some years, but there are a number of physicians who would be interested in specializing in research in this field. Basic research is carried out by only a very few teams.

SUMMARY OF RESULTS

The results of the evaluation of research into UM can be summarized as follows:

- In the first five above mentioned forms of therapy, which enjoy the widest distribution among UM in Germany, some research activities and interesting scientific findings do exists. Some of these are of a high scientific standard, but generally speaking there are considerable deficits in all scientific disciplines, including clinical and preclinical research. Examples of systematic research of high methodological quality into specific topics are rare, and the main reason for this situation is that the UM are excluded from university research, making it necessary to conduct research beyond the bounds of established research institutes. Consequently, the organisational and methodological conditions are not conducive to good quality research; research is for the most part carried out by scientists working in an enclave. The complete lack of state support contributed to this situation, whereby support of UM research was blocked by the decision-making committees formed by experts in the conventional field.
- In clinical trials research designs are frequently of poor quality and their presentation in papers is often incomplete. Nevertheless, the research papers demonstrate that a large proportion of UM is accessible for scientific evaluation, and that they justify further intensified research and state support. The problem of finding adequate methods for clinical trials in UM is currently under discussion.[5,19,20,21]
- The lack of basic research leads to an inadequate understanding of the therapeutic mechanisms of many unconventional therapies. The present inability to explain the mechanisms of treatments, for instance, homoeopathy with its potentized medicines, or acupuncture with its meridian system, against the background of modern scientific understanding, is one of the main reasons for their lack of acceptance in conventional medicine.
- Basic research has to include detailed studies of the principles of stimulation of the so-called self-healing capability of the organism (in the sense of natural healing-processes), and their therapeutic relevance. It can be expected that this will lead to fundamental knowledge of the therapeutic approaches of natural medicine.
- There are a number of research teams in Germany qualified to work in the field of UM at a high scientific level on both pre-clinical and clinical topics. Until now their work has been hampered to a great extent by inadequate conditions. A few research groups work at the universities, but are usually isolated. Working on unconventional topics can ruin the reputation of a scientist. Recently there has been a slight increase in the interest in such groups. There is hardly any scientific discourse between representatives of UM and those of conventional medicine, and the few discussions held tend to be polemic rather than objective. However, the difficulties are on both sides, with a tendency towards dogmatism.
- Scientific discourse can best be initiated where research projects are established in close cooperation between the unconventional and conventional fields. This is confirmed by the experience gathered during UM research projects on oncological topics, which have already been sponsored by the BMFT. Such

cooperation can also lead to a great improvement in the quality of research applications and projects.

RECOMMENDATIONS FOR STATE SUPPORT OF RESEARCH IN UM

The evaluation resulted in the following recommendations for research support, which were addressed to the BMFT and also to other sponsors in Germany:

1. The financing of the necessary infrastructure, making it possible for research teams to work continually and long-term on UM themes and problems, thus gathering experience and achieving the competence to engage in high-level research with inter-disciplinary cooperation.
2. In the first phase of the research sponsorship the main emphasis should be on the five therapy forms discussed in this paper. Clinical studies on chosen topics representative of the effectiveness of each respective therapy form should be sponsored. When at a future date the research teams have gained enough experience and the infrastructure has improved sufficiently, other relevant therapy forms could be included.
3. In selected main topics, investigation into the working principles of the therapies should be sponsored, in order to improve their plausibility.
4. The development and adoption of designs for clinical-therapeutical studies especially suited to the specific characteristics of UM should be sponsored.
5. A critical examination of international scientific literature in the field of UM, including the compilation of a databank, is seen to be an essential supporting measure.
6. The scientific discourse between UM and conventional medicine should be institutionalized.

By means of systematic research in the field of UM, it should be possible to show clearly the differences between therapeutically appropriate treatments and valuable diagnostic and therapeutic principles on the one hand, and those which are unsuitable for integration into contemporary medicine on the other hand.

The BMFT has been sponsoring UM research projects in the field of oncology since 1984. Now the BMFT has established a new research task force based on the recommendations of the team at the University of Witten/Herdecke. On 1st December 1992 the BMFT announced that clinical studies in the fields of phytotherapy, acupuncture, homoeopathy, anthroposophical medicine and parts of physical medicine will be sponsored, with the main emphasis on the cooperation between research teams and on studies which compare UM and conventional treatments. The BMFT appointed an independent expert committee for selection of research projects for central government support.

Closing date for application for government support was 15th March 1993, by which time 230 applications in short form had been received for consideration. The great number of proposals for clinical studies included many promising research outlines, thus confirming the results of the survey, which concluded that UM research can be intensified and that scientists can be found to carry out the work.

The applications have been assessed by the independent expert committee, which selected the most interesting projects for the formulation of detailed research applications. It is anticipated that the first research projects sponsored by the BMFT can be started during 1994.

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Grants awarded to the Government's Funding Area „Unconventional Medical Approaches" (UMA) (up to December 1997)

Title	Performing Organization/ Head of Project	Time of funding
Clinical and immunodermatological study on the effectiveness of external psoriasis therapy with Mahonia aquifolium in comparison to normal therapy with dithranol	Univ.-Hautklinik Freiburg Dr. Augustin Dr. Simon Prof. Dr. Schöpf	01.08.1994 -30.06.1997
Effectiveness and tolerance of ethereal plant oil preparations in primary headache complaints	Universität Kiel PD Dr. Göbel	01.08.1994 -31.12.1997
Comparison of the clinico-therapeutic efficacy of a phytotherapeutic plant extract (Kava-Kava root), chemically defined monosubstance (Kavain), and placebo on outpatients with anxiety, tension and restlessness, and development of suitable biometric procedures	Universität Ulm Prof. Dr. Gaus Prof. Dr. Faust	01.10.1994 -30.09.1997
Controlled clinical trial on "toughening" by hydrotherapy in children with frequent upper respiratory tract infections	FU Berlin Prof. Dr. Bühring	01.05.1995 -31.08.1997
Clinical investigation of Acupuncture as acute treatment for recurring headaches	Evang. Krankenhaus Oberhausen Dr. Pothmann	01.05.1995 -30.04.1998
Investigation of the Therapeutic Effects of Curcuma Xanthorrhiza and Fumaria Officinalis in Patients with Irritable Bowel Syndrome A clinical prospective randomized double-blind placebo-controlled study	Universität Erlangen/ Nürnberg Prof. Dr. Hahn	01.06.1995 -31.12.1997
Investigations on the effectiveness of Acupuncture therapy for chronic back pain in a prospective randomised study	Universität Göttingen Prof. Dr. Ramadori Dr. Li	01.07.1995 -30.06.1999
Homoeopathy in female and male infertility	Universität Heidelberg Prof. Dr. Gerhard	01.08.1995 -31.07.1999
Randomised clinical trial to investigate the effectiveness of traditional chinese Acupuncture in comparison to sc applied Sumatriptin (5-HT1-receptor agonist) and placebo drug therapy as early treatment (arrest of attack) for patients in the initial stage of an acute migraine attack	Projekt „Münchener Modell" an der LMU München Dr. Melchart	01.11.1995 -31.10.1997
Biometrical realisation ot three clinical studies within the funding area „Unconventional Medical Approches"	Universität Düsseldorf Prof. Dr. Mau	01.08.1995 -31.10.2001

A study for the clinical appraisal of Acupuncture as a therapy for chronic scapulodynia in outpatients	Praxis Dr. Molsberger Düsseldorf Dr. Molsberger	01.02.1996 -31.01.1999
Proof of effects of Acupuncture in cervical spine syndrome compared with massage as conventional treatment and with a placebo	Universität München Dr. Behrens	01.03.1996 -28.02.1998
Clinical study of the anthroposophical concept of therapy for early rheumatoid arthritis in comparison with conventional long-term therapy	1. Filderklinik Filderstadt Dr. Simon 2. Krankenhaus Hamburg Rissen 3. KVG Klinik Bad Pyrmont Dr. Schendel 4. Zweckverband Stadt- u. Kreiskrankenh. Klinikum Minden, Prof. Lakomek 5. IMF Gesellschaft für Interdisziplinäre med. Forschung GmbH	01.12.1995 31.05.2001

Mr. BURTON. Thank you, Dr. Matthiessen.
Dr. Gordon.

STATEMENT OF DR. JAMES S. GORDON, DIRECTOR, THE CENTER FOR MIND-BODY MEDICINE

Dr. GORDON. Thank you very much, Mr. Chairman.

Let me introduce myself. I am a physician practicing here in Washington, DC, and I've worked in and around Washington since 1971. I'm the director for the Center for Mind-Body Medicine, a professor at Georgetown Medical School, author of a book called "Manifesto For a New Medicine: Your Guild to Healing Partnerships and the Wise Use of Alternative Therapies." And I also was the first Chair of the Program Advisory Council to NIH's Office of Alternative Medicine.

I've been talking with people here in Congress for many years, probably over 20 years, and I have been sitting here this afternoon smiling a good deal to myself because I see how far we've come in these last—not only last 20 years, but even in the last 10 or the last 5 years. It's extremely refreshing and hopeful for me to hear you and Representative DeFazio and Representative Moran speaking in the way you've been speaking this afternoon.

The reason that medicine is changing so profoundly in this country is because people want and need it to change. It's not been a concerted effort of scientists or physicians. It's been the demands of people, like the people we heard this afternoon, who have been suffering and not getting adequate care from the conventional medical establishment. They've been finding answers to certain problems and not to other problems.

I believe we're in the midst of a revolution, a transformation in all of health care and I want to just highlight a few items that I think are particularly important and I go into them in more detail and more formally in my written presentation.

First of all, if we just approve or make available a few other therapies, we will have only done a very small part of our job. This is not just about adding a little acupuncture here or a few herbs there. This is about deeply changing the way we do medicine and making all our care responsive to people's needs and teaching people what they need to care for themselves and educating them so that they can make informed choices and creating environments and attitudes that are truly healing for all people. This is a very profound change that we need because our health care system is in sorry straits in many ways.

There are several important ways that we can help to bring this about and I want to highlight those now. In the long run we need thorough investigation of some of these promising new therapies, of new therapies that are developed in the laboratory of traditional, truly traditional therapies that have been used in other cultures for hundreds or thousands of years or indeed, tens of thousands of years. We need to take a good look at them. We need to take an intelligent look. We need to develop the appropriate methodologies and not insist that the therapy fit into a previously used methodology but to develop methodologies that are effective for that particular therapy.

In that vein, the Office of Alternative Medicine has since its inception 6 years ago, been a beacon of hope for people. At times it's been a faint beacon. Increasingly, the light is getting stronger. We need to sort of beef up the generator for that office. The office was initially funded at $2 million. The funding has gone up to $7, $12 and now $20 million. That's a tiny fraction of what's needed in order to study promising therapies.

Earlier there was some discussion about studying St. Johns Wort as a treatment for depression. That study will cost $1.5 million a year for 3 years. That's one-twelfth of the office's budget and that's one therapy among hundreds that needs to be studied. So we need to create an office that has enough funding so that it can study the therapies that we need to take a look at, that we desperately need to take a look at. And we need to convert it from being an office, in which case it is dependant on the approval of other institutes in order to study therapies, to becoming a center on its own so that it can be funded at a much higher level, initially I would hope around $100 million and it can have the independent authority to look into those therapies that the people want to be looked into, not therapies that only the scientific establishment is interested in. This is very important.

Also, making the office into a center will mean it will have its own review committees. At present there are 25 standing review committees at NIH with 126 members, none of whom is fully credentialed in any of the complementary or alternative therapies. So even with the best will in the world, the people on those committees simply are not familiar with and are not really appropriate to be judging applications regarding those therapies. I'm not saying committees should be formed only of people who practice those therapies, but at least there need to be representatives on those committees. So first is creating a center and an adequately funded center.

Second and more immediately, is passing the Access to Medical Treatment Act. You've heard an example today of someone who has had extraordinary difficulty in having access to a promising therapy. We need to make, according to Representative DeFazio's and Senator Daschle's bills, we need to make those therapies available responsibly to anyone who wants to make use of them and available through people, through practitioners who are duly licensed and duly responsive to their State licensing boards. So this is crucial.

I recently got back from China where I was giving a speech at a World Congress on Integrated Medicine and incidentally, in China, fully one-third of the physicians belong to this group of integrated physicians. This is the wave of the future in the largest country in the world and it should be here.

One of the therapies that I heard about was a remarkable therapy using wet herbal dressings for third degree burns, and I saw and met with people who had been burnt over 90–95 percent of their bodies, who are now not only living but well and scar free. This therapy has aroused considerable interest in Europe and other parts of Asia. It is stalled here in an approval process. I spoke with the surgeon who originated it and he predicted, according to what he had heard from the FDA, it would take many years to get this

life-saving therapy approved. And that's just one of many. So I feel the Access to Medical Treatment bill needs to be passed as soon as possible to make these therapies available to all Americans.

Third, it is crucial that we have information about what we know and what we don't know about the therapies that are out there. We need that information reliable, carefully evaluated information available now. One of the things that we're doing here at our Center for Mind-Body Medicine and I think you and your staff knows about this, is we are having a Conference on Comprehensive Cancer Care integrating complementary and alternative therapies this June. And the whole purpose is not to say, oh, we love this therapy or we love that therapy; the purpose is, let's take a look. Let's bring together the best people from around the world who have developed and are using these therapies, bring them together with the pillars of the cancer establishment to take a fair look. And then, once we've done this, to provide the information as freely as possible to as many people as possible.

This process which we're doing in a private way with some support from the Office of Alternative Medicine, this needs to be made a major priority. One of the office's mandates is to have a data base and a clearing house but it's insufficiently funded to move it ahead in an appropriate time. So these three areas seem to me to be vitally important now to help the transformation in medicine and to make available to all of us the best therapies and the best information about those therapies.

[The prepared statement of Dr. Gordon follows:]

My name is James S. Gordon, M.D. I'm a Clinical Professor in the Departments of Psychiatry and Family Medicine at the Georgetown University School of Medicine and Director of the Center for Mind-Body Medicine. I was the first Chairman of the Program Advisory Council of the National Institutes of Health's Office of Alternative Medicine, and after my initial two-year term, I was reappointed by NIH to an additional year as Chairman. I've published well over 100 articles in the scientific and popular press and written or edited ten books, including most recently Manifesto for a New Medicine: Your Guide to Healing Partnerships and the Wise Use of Alternative Therapies. For more than twenty-five years, I've been integrating a variety of complementary and alternative approaches into my practice and teaching of medicine and psychiatry.

We are in the midst of a revolution in the practice of medicine and a transformation in the kind of health care Americans want and receive. We are in the processing of shifting the center of gravity of our system from high-tech diagnosis and treatment to self-care and mutual help; from a Western biomedicine preoccupied exclusively with finding the ultimate cause of and instituting aggressive treatment for discrete disease states to a "world medicine" which is equally concerned with balance and harmony within the individual and between the individual and his or her natural and social world; from a relentlessly secular system of treatment to a profoundly spiritual approach to care. For tens of millions of Americans, it is no longer a question of either modern science or ancient wisdom, but of combining *both* in a new, richer, more effective and more humane synthesis.

Thirty years ago, Americans and their physicians believed that blood pressure and heart rate, the pain of cancer and the level of responsiveness of the immune system were utterly beyond the control of the individual. Acupuncture and Chinese medicine were anthropological curiosities whose practices were limited to the Asian community in the

United States. Physicians could lose their membership in state medical societies for referring patients to chiropractors. And massage was a subject for dirty jokes.

Today we know that ordinary human beings are capable of mobilizing their minds--through biofeedback, relaxation, imagery and hypnosis--to improve and alter virtually every physiological function. Between twelve and fourteen thousand acupuncturists practice openly in the United States, and some 3,000 are physicians. Chiropractors have won anti-trust suits against the AMA, are licensed in every state and serve approximately 10% of the population. Massage therapy is a growing profession whose practitioners are providing relief from stress, enhancing the mood of depressed patients, and giving help to those with cancer and post-operative pain.

In their 1990 national survey, Dr. David Eisenberg and his colleagues found that some 34% of Americans were already using these and other "alternative" therapies. The word "alternative" designated practices other than those taught in medical school or in post-graduate training. Seven years later, it is likely that over 40% of Americans use these therapies and that the vast majority use them as a "complement" to conventional therapies, as part of a self-created program of health care. Physicians in increasing numbers (close to 90% of family physicians in one study) are looking for information about these approaches, and studying and incorporating them into their practices. More than one-half of all American medical schools presently have electives which offer an overview of these alternative and complementary therapies.

When attacks are launched against "alternative medicine," the attackers tend to turn their sights on practices they believe to be inherently foolish. "Imagine actually giving research money to studying massage therapy," they say. Or ,"Why bother with herbs when we already have drugs?" or, "Do you really expect us," as one major figure in American

medicine recently said to me at Grand Rounds at one of our most respected teaching hospitals, "to take prayer seriously?" Some laugh at homeopathy--the use of infinitely small doses of substances to relieve symptoms that larger doses of those substances could produce. And many simply state that all of the therapies for which there is good evidence (for example, biofeedback) are already included within the medical canon, while there is obviously no "good" evidence for the others.

The complaints about insufficient data are rarely grounded in thorough study. There is, in fact, a sizable body of research information on a variety of different alternative and complementary therapies. I cite several hundred epidemiologic and randomized controlled studies from peer-reviewed journals in Manifesto for a New Medicine. "Alternative Medicine: Expanding Medical Horizons," a report prepared by over 200 researchers and clinicians for the Office of Alternative Medicine contains many hundreds more citations. And, those who deny the possible utility of therapies for which there is no clear mechanism or resist funding studies of them, I think, are both obtuse and forgetful. Aspirin was happily used by conventional physicians long before we had any notion of why it worked.

We know, to cite just a few examples, that meditation, relaxation therapies, imagery and hypnosis can contribute in a major way to decreasing stress, relieving pain and insomnia, as well as altering blood pressure, enhancing immune functioning, and helping to reduce the frequency and intensity of epileptic seizures. There are hundreds of carefully controlled studies in peer-reviewed journals, mostly from Europe and Asia, on the utility of herbal preparations, for example, astragalus and echinacea for enhancing immunity and St. John's wort for alleviating depression. Massage appears to make a major difference in the growth, development and well being of premature babies. And homeopathy--improbable as it may seem--does in a careful meta-analysis seem to have very real effects

on a variety of conditions. In some cases, the evidence is far more impressive than that brought forward to justify a host of surgical procedures and other expensive, side-effect laden, commonly used, high-tech interventions such as electronic fetal monitoring of normal births or the insertion of tubes in the ears of babies with chronic infections.

There are, of course, a number of alternative and complementary therapies that have not been adequately studied. This is precisely why Congress established the Office of Alternative Medicine at the National Institutes of Health. These include therapies that hundreds of thousands or, indeed, millions of Americans are looking to for answers to their health problems, therapies about which patients would hope to query their doctors, just as they might about the latest antibiotic or the newest surgical technique. Half a million people have used intravenous EDTA chelation to treat heart and peripheral vascular disease. Many of these people claim that chelation has been a life-saver. Most conventional physicians regard the success as an illusion, if not a hoax. People with HIV look to herbal therapies to enhance immune functioning. And cancer patients--as many as 70-80% of them in some studies--scour the bookstores and search the Internet for help with tumors that are poorly treated by conventional physicians or for side effects of even successful treatment.

The Office of Alternative Medicine (OAM) was created to make information about what is and is not known about alternative and complementary therapies available to patients, clinicians and researchers. It was mandated by Congress to "investigate and validate" promising new therapies. Funded at $2 million, in 1992, it was a small but bold initiative. It was seen as a "beacon of hope" by many who were desperate for reliable answers about the efficacy of unconventional therapies, as well as by those who practiced or studied

these therapies. For the first time, the government would pull together information scattered in thousands of journals across all the continents and sort through it, culling what was valuable and discarding what was not, and making the results as widely available as possible. For the first time, there would be a body within the world's premier research organization, the National Institutes of Health, committed to a fair study of these therapies and rapid dissemination of the results of these studies.

In the six years of its existence, the OAM has funded forty small studies on specific alternative therapies and has established ten academic centers, some of them at the nation's most respected medical institutions, devoted specifically to studying these therapies. It has brought together unconventional practitioners and conventional researchers so that they might work together to develop methodologies appropriate to the therapies and the therapeutic systems under study and rigorous enough to satisfy the most exacting scientist. It has published Alternative Medicine: Expanding Medical Horizons and begun to make available the results from the studies it has funded. It has begun to build bridges with other NIH Institutes and the researchers in them and with other federal agencies, including the Food and Drug Administration and the Center for Disease Control. It has provided technical assistance to dozen of investigators who are committed to the scientific study of their treatments. The OAM has developed a Consensus Conference on the use of relaxation therapies and acupuncture. It has helped open the way to the approval and use of herbal therapies and has just recently funded a major prospective study on the treatment of depression which will compare in head-to-head clinical trials St. John's wort with one of the Prozac-like, selective serotonin re-uptake inhibitors in the treatment of depression.

The OAM has moved too slowly for some, particularly with regard to life-threatening illness for which there is no conventional medical answer, and has not always been

responsible to the needs of its constituents. For others, its progress has been too rapid. Still, in spite of its still minuscule budget (its first director, Joseph Jacobs, M.D., called it "homeopathic") and opposition within and outside of it, the OAM provides an opportunity for authoritative data collection, evaluation and dissemination; a focus for scientific exploration of the efficacy of alternative and complementary therapies; a forum for debate about research priorities and methodologies; and the possibility of the systematic study of the usefulness of these therapies and of the ways they may enlarge and enrich medicine in America.

In order for the OAM to move ahead more effectively, significant increases in its funding and changes in its structure are necessary. I'll address these later. They hold great promise for the authoritative evaluation of complementary and alternative therapies and for the creation of a means by which people can most quickly obtain the most promising new treatments. Now, however, I want to turn my attention to another matter: the Access to Medical Treatment Bill

Scientific evaluation takes a considerable amount of time. Randomized, controlled studies that satisfy the criterion of the FDA require a great deal of money as well as time. The Access Bill makes it possible for people to safely obtain treatments that have not been approved by the FDA while the engines of scientific progress move slowly ahead.
The bill, which has been introduced in both the House and Senate, permits any individual to be treated by a licensed health care practitioner with any method of treatment that that person requests; whether or not it has been approved by the Food and Drug Administration. The bill, whose co-sponsors range from conservative Republicans like Orrin Hatch (Utah) to liberal Democrats like Tom Daschle (S.D.), would not only expand health care options, it would also bring alternative therapies safely within the embrace of our health care system.

At present, fears of punitive action have some clinicians and researchers reluctant to share information - positive or negative - on the alternative therapies they use. Some practitioners have been arrested for practices that the FDA deems illegal. Others, including a number who treat cancer or HIV, have moved their clinics to Mexico, the Bahamas and Latin America. Some of these people appear unethical as well as unscientific but others are offering treatments that seem to hold genuine promise. The net result of forcing them off-shore is that patients who cannot get the care they want in the United States must go where it is unregulated, and physicians and other health practitioners in this country are unable to practice or study the medicine they believe will help.

The threat of overzealous regulation has made impossible exactly the kind of scientific investigation that the FDA and other regulatory agencies say they want. By requiring that practitioners who wish to offer alternative therapies be licensed, the legislation will help keep these practices within the compass of state regulatory boards. It will require that all practitioners report both positive and negative effects to the Department of Health and Human Services. And by insisting that practitioners who use these treatments not derive any financial benefit from them (other than fee-for-service) the bill removes the legitimate concern that unscrupulous practitioners can make huge profits from the drugs or devices they use.

The Access to Medical Treatment Act will make it possible for all of us to explore, with some reassurance of safety, all of the complementary and alternative therapies that are available. It will, as well, provide some feedback about therapies that have been, at least in individual cases, helpful or damaging. But it will not do the job of providing us with the rigorous scientific data that we need to fully evaluate these therapies. That job rests,

as it should, with the Department of Health and Human Services and most particularly with the NIH and the Office of Alternative Medicine.

The Office of Alternative Medicine, as first established, was an office within the Office of the Director of NIH. A year ago, it was transferred to the Office of Disease Prevention. Congress increased its budget from $2 to $12 million and most recently to $20 million. This increase is, however, a pale reflection of the interest in the office. The OAM receives some 1200 calls a month, as many or more than institutes with 50 or 100 times its budget, from people desperate for information (up to 80% of them have cancer).

With its current budget, the OAM cannot afford to establish the database that Congress mandated and evaluate the existing literature on alternative and complementary therapies. It can not adequately fund academic centers for the investigation of promising therapies for particular physical and mental disorders. And it certainly can't investigate and validate promising and/or controversial therapies. An adequate study on St. John's wort for depression - a single herb for a discrete condition - to be funded by the Office but through and under the auspices of the National Institute of Mental Health, will cost $1.5 million a year for three years, or approximately one-twelfth of the OAM's entire budget. An appropriate clinical trial of chelation therapy, the kind that is needed to help Americans determine whether or not this procedure actually works and, if so, for what conditions, would cost significantly more. The size of the Office's staff is also completely inadequate to investigate the hundreds of therapies that tens of millions of Americans are using. A budget of $100-150 million with staff large enough to manage it would enable the Office to begin to address the scientific and human questions that are continually being addressed to it.

However, more than money is needed. The current position of the OAM as an Office

restricts its capacity to do the research it is mandated to perform. All of its grants have to be funded in collaboration with and through the administrative mechanism of other Institutes. This means, quite simply, that other Institutes with other priorities and other means of calibrating scientific importance and public accountability may frustrate the research agenda set by sectors of the scientific community and the representatives of public organizations who advise the OAM. When the National Institute of Mental Health agrees that a study comparing St. John's wort, which has been used regularly by over 20 million Germans, is a worthy subject of study, the granting mechanism proceeds apace. If, however, the National Heart, Lung and Blood Institute, based on its evaluation, decides it is not important to study chelation therapy, or the National Cancer Institute disagrees about the value of investigating a new, widely-used and apparently promising but controversial, unconventional cancer treatment, careful, scientific investigations of these widely-used approaches simply cannot proceed. The OAM needs to become the National Center for Complementary and Alternative Medicine, an independent NIH body with its own granting capacity and an advisory council with the authority to approve these grants.

The OAM is also limited in a second, and equally important, way. It is not able to create its own standing review committees, committees which would include members with expertise in complementary and alternative approaches as well as in scientific methodology. At present, there are approximately 26 standing review committees at NIH, with 125 people. At last count, none of the members of these review committees had degrees or licensure in any of the commonly used complementary or alternative therapies. There were, for example, no chiropractors, acupuncturists nor, so far as we were able tell some months ago, did any committee member with M.D.'s and Ph.D.'s have adequate expertise on complementary and alternative therapies. Transforming the OAM into a Center would enable it to appoint distinguished researchers and clinicians with significant expertise in these areas to its review panels. It would put the scientific study of

complementary and alternative practices on equal footing with the scientific investigation of conventional medical and surgical therapies.

We also need a well-funded, independent Center to explore the utility of comprehensive approaches to the diseases which kill and disable large numbers of Americans, approaches which include a variety of alternative and complementary, as well as conventional, medical treatments. We need to see if shifting the emphasis from high-tech treatment controlled by physicians to teaching self-care and helping people to help one another can alter health status as it does mood, self-esteem and sense of control. We need to move beyond our single minded focus on modern biomedicine to explore the richness and relevance of the world's many healing traditions. We need to determine if some plants in the world's pharmacopeia may have greater benefits and fewer side-effects than manufactured pharmaceuticals. We need to be open-minded enough to see if the "vital energy"--the Chinese call it "chi," the Indians "prana"--which is regarded as an integral part of every healing system in the world can be measured scientifically and used therapeutically.

We need a Center committed to finding out not only whether these therapies work and if so, how, but how they can be implemented in real life, in hospitals and clinics across the country, for the poor who cannot pay out of pocket as well as for the wealthy who can. And we need to see if these approaches are indeed capable, as a number of them have already been shown to, to save us significant amounts of money.

We need a Center where we can create models for the health care of the future and for the education of those who will practice it on themselves and others. This Center could help create a larger, more humane, more intellectually and humanly responsible professional education that will enrich and humanize the lives and practices of medical and nursing

students and their future patients.

Finally, a Center with wide-ranging authority is necessary because complementary and alternative therapies are not simply specific approaches to specific disease states. When used appropriately, they embody a new way of approaching health and illness with implications not only for research and treatment but for every aspect of health care and education: for the financing of health care; for the education of future health professionals; and, indeed, for our conceptualization of health and illness.

Mr. BURTON. You said you were the Chair of the alternative therapies commission?

Dr. GORDON. I was Chair of the advisory council. I was the first Chair and then I was reappointed for a year. I'm no longer the Chair now.

Mr. BURTON. Are the people at the FDA paying much attention to the recommendations of that committee?

Dr. GORDON. Well, there's an ex officio member on that committee from the FDA, and I think that that's been helpful in moving the dialog along. I think a great deal more needs to be done. I think the FDA needs to be educated about these therapies. I think there needs to be a major push from Congress to insist that there be other ways of evaluating these therapies. We heard some testimony on that earlier.

Mr. BURTON. What other ways?

Dr. GORDON. Well, I think for example, and Dr. Matthiessen can address this, some of the ways that traditional therapies have been looked at in Germany simply by saying here is use that's been going on over a long period of time, and he can talk about it in much greater and more accurate detail, these therapies seem to have been helpful and most importantly they have not created harm. And then also pulling together the data that is available, the scientific data.

One of our problems in this country, and this will be no surprise to anyone in this room, is that we're incredibly chauvinistic. We have not really opened ourselves to the rest of the world and to information from the rest of the world in the way that we should. I have no doubt that scientific methodology in Germany and in most other European and some Asian countries is every bit as sophisticated as ours. There's no reason why we shouldn't accept those studies just as we would accept our own.

And I think there has to be an expedited process. You were hearing earlier about someone who needs—and you made the point I think very clearly—it's not merely a matter of life-threatening illness, it's also chronic illness. There is no earthly reason if therapies are not on the face of them harmful, if they've been used elsewhere and they're not harmful or no one has any reason to believe that they're harmful, to not make them available to people with informed consent. And I think the FDA needs to either develop processes to enable that to happen or that somehow that responsibilities should be transferred elsewhere.

Mr. BURTON. I would appreciate it if maybe if you have a few recommendations, since you've worked on that commission, if you could give us a few that we might incorporate into our discussions with the FDA when we meet with them.

Dr. GORDON. I'd be very happy to do that.

Mr. BURTON. Dr. Matthiessen, did you want to comment on what he just said?

Dr. MATTHIESSEN. Perhaps one point in regards to the methodology. It was our experience that randomized studies are a big problem in this area, because patients are so autonomous and generally they are not willing to be randomized. They have certain decisions and certain intentions, so we had to look out for other methodologies. And our impression was even the single case studies have

been chronically underestimated, and that was a decisive area of finding that we also have to look at appropriate methodologies.

Mr. BURTON. You said one thing and I'll let Dr. Born make any comments she might want to make, and then I'll ask my colleagues if he'd like to ask a question. You said that in China they have a procedure where if someone was burned over 90 percent of their body and they put some kind of coating on the skin?

Dr. MATTHIESSEN. Yes.

Mr. BURTON. And they not only survived, but there was no scarring?

Dr. GORDON. Yes. This was a surgeon who had developed this therapy. When we were in China we not only read the case reports, saw the pictures of the patients before and after, but met the people who had been so badly burned and they were quite recognizable from their initial picture. It's very dramatic. It's just one of a number of therapies that can't be brought here or that have not been able to be brought here.

I can get you some more information about it.

Mr. BURTON. And how long has FDA been fooling around with that?

Dr. GORDON. Well, according to the surgeon, and I have not gone and checked it out with the FDA since then and I'm waiting for more information from him, he told me that it had been a couple of years that he had presented it.

Mr. BURTON. And there's pretty substantial evidence that it works?

Dr. GORDON. Certainly the evidence looked good to me. It certainly looked good enough to me so that it was worth a trial or at least worth a visit.

This is something that has been very important I know to Senator Harkin and to former Representative Bedell in particular, very dear to his heart and to mine as well, is that when something like this comes up when there is such a therapy, we need to go and take a look.

Mr. BURTON. It's not that hard.

Dr. GORDON. Not that hard. And I think that this is one of the areas in which the Office of Alternative Medicine is hoping to move ahead together with the CDC is to have people go out and I would say not just in this country, but all around the world where somebody is getting this kind of result and to really take a hard and fair look and see what they have to offer.

Mr. BURTON. Dr. Born, did you have any comment?

Dr. GEURKINK BORN. I think one of the other things that was mentioned earlier was that the great number of people that are interested in preventive medicine; that this is a train that's going to keep rolling. And doctors and doctors who are against complimentary medicine and FDA can get in the way of this train or they can stand and watch it go by, but I think that the Access to Medical Treatment Act is a really good bridge to allow some control and some good studies to be formed from the research that is encouraged by this bill to allow patients access without encouraging doctors that may be unscrupulous and promoting quack cures or promoting themselves selfishly. I really believe this is a great bridge to fill that gap.

Mr. BURTON. I wish all my colleagues were here, but I don't think anybody who has listened over the past 2 days of hearings that we've had could help but feel that the FDA instead of being a help in many cases is an impediment and it kind of makes you very angry, especially if you've had anybody who has been very ill.

Dr. GEURKINK BORN. Or especially if you've been a doctor who is trying to help their patients and unable.

Mr. BURTON. I'm sure you feel that frustration as well.

Do you have any questions?

Mr. DEFAZIO. Thank you, Mr. Chairman. Yes, Mr. Chairman, if I could.

Dr. Gordon, if I could just followup. I guess what puzzles me, and I know you can't give me a definitive answer either, but I've speculated and talked to many people about whether the problem is with the law or the problem is with—I mean people want to point to the FDA and say they're the villains. Well perhaps it has to do with the charge we've given them or their interpretation of that charge, I think in part, but still they seem to be overzealous gatekeepers in cases like this burn therapy you mention. And it's not unique, as I found out in the case of saw palmetto. I mean they said, as I understand it, at the end of the process the results are statistically significant but they wouldn't allow health claims to be made for saw palmetto because they didn't find them to be medically significant. Well, I don't know how you can have a statistically significant response that wasn't medically significant to individuals. I mean, do you understand?

Dr. GORDON. Well, I am a psychiatrist, but I'm not—I think that you put your finger on it in the beginning of what you said. The problem is that the language that defines what the FDA does has to be changed. I think that they are adhering in a sense to the letter of the law but they're missing the spirit. And I think that potentially in my experience that Congress can enforce—if this isn't a paradox—can enforce the spirit by saying we want you to open up your way of looking at these substances, at these procedures. We want to diminish the watchdog function. We want to make that only where it's absolutely necessary. And we want you to take as your charge making available as much as possible that is not going to be harmful.

And I think it has to be a very fundamental shift. And I know you've had hearings, and I've been at hearings where the FDA has testified. The FDA is just a kind of representative of the scientific establishment, in a sense, one wing of it. And I think that it will change.

NIH, for example, is beginning to change because of the consistent pressure, because of pressure of hearings, pressure of all of you so that, for example, Dr. Varmus has now suggested, and I think rather strongly, to all of his institute directors that they take a look at some of these promising alternative therapies. And I think that what has to happen is that you all have to make very clear what you want to have happen, and tell them that they have to come up with language which permits rather than restricts.

Mr. DEFAZIO. Yes. And I think the key in what you said is that they prove that they don't do harm. And I guess, Dr. Matthiessen, how do you deal with this in Germany? For instance, saw palmetto

I think is made available in practice in Germany, as I understand, and yet here we're not allowing claims to be made. I guess it would be something from your E list, so it would be something that had—I guess it's observed through clinical practice and history, is that how it is? I think what you hinted at was people who seek out alternative medicine don't want to end up in a placebo, in a study, is that what you were saying in terms of they were resistent to that?

Dr. MATTHIESSEN. Well, that was one topic, yes. They didn't want to end up with a placebo. But they just didn't want to be randomized objects. They had certain demands and certain expectations and so it was very difficult to establish comparable trials. But there are methods and we can develop findings about new methods, appropriate methods which allow individualized therapies.

Now one main problem is, I think, that the conventional medicine is very much science centered and scientist centered. Now many CAM are practitioner centered and patient centered; that means that the problem of individualization within the art of healing, it's person bound. And it's very difficult to cut down on formal knowledge. But the reality is, of course, this let me say degenralization and re-individualization of our knowledge in regards to the unique situation and the highly complex situation meeting one single patient.

Dr. GORDON. I think what you're saying is very interesting because what needs to be created is an open space in which whatever is known can be presented. There's no reason not to present the fact that this is statistically significant. I don't understand why something that has been proved can't be presented publicly, and I think there must be some kind of strange convolution in the FDA regulations that prevents it. So that needs to be freed up. And once that's freed up, once all the information is out there, then one can begin to work in a very individualized way with people and to begin to promote other methodologies and other ways of approaching people. But they go together that the openness has to be created and then we can really work at providing both individualized and comprehensive care.

Mr. DEFAZIO. Dr. Born, I think you were alluding to this from your practice and your observations. I mean what I see now most commonly is if I'm in the health food store, or whatever, and I'm looking for something that I've sort of researched on my own or talked to a naturopath in Oregon about, I see other people there and they'll start asking me questions. I'm saying "Well, I don't think this is a good idea," or a lot of these health food stores will have a clerk who specializes in that. But instead of being able to go to you and get information with confidence or someone else who is conversant and trained we're really inhibiting that. I mean wouldn't you say now we're really got the worst of all possible worlds? It's happening but it's happening in a way where, as Jim says, there's no body. We're just not moving the information forward and making it available for people nor are we allowing them to act as experts regularly, and I just wanted you to reflect both on that and this other thing. Because I think the most chilling testimony I've heard is what you said the Assistant Attorney General from California said: It is difficult for us to get patients to complain

about these doctors, so we'll have to find ways to get them ourselves. So the patients are satisfied but the regulators still want to get these doctors? I mean, that's an accurate——

Dr. GEURKINK BORN. That's very true. I couldn't believe it when I was sitting there either. I think I was the only complimentary doctor in the room, but there must have been 300 to 400 other doctors there. It was scary to me that that's their philosophy.

I think that they see this train rolling along that maybe they're willing to look into some kind of regulated passage of an alternative medicine bill. I'm not sure that this is one that they would agree with, but I think that it's a great start and I think it's a great bill personally. But, yes, there are many, many doctors who do not agree that conventional medicine has anything to do with vitamins and minerals.

Mr. DEFAZIO. Yes, I mean I guess I had an experience with a physician in my own district, the same thing. They couldn't find a single patient to complain, but they pulled his license for using unapproved therapies, yet they could not get one patient to complain. In fact, the patients were deluging my office saying get this guy his license back, he was the only person that did me any good. But, you know, the State board for whatever reason had pulled his license. He finally did get it back.

Dr. GEURKINK BORN. I hear of that all the time.

Mr. BURTON. Thank you, Mr. DeFazio.

I want to thank you all for being here.

I understand you work for Muhammed Ali and his wife?

Dr. GEURKINK BORN. Yes, they have been patients of mine.

Mr. BURTON. They are patients of yours?

Dr. GEURKINK BORN. Yes.

Mr. BURTON. I've had the pleasure of getting to know him, and I think he thinks very highly of you, so you're to be commended for giving him some help.

Dr. GEURKINK BORN. Thank you. He improved dramatically with our treatment. We do a therapy called chelation therapy and his wife noticed improvements immediately. In fact, before he had treatments you hardly ever saw him in the press and since he had treatments, within 6 months he lit the Olympic torch that year. And since then he's been in the press and been out to engagements and events frequently. But he could hardly talk. It took him a half hour to walk down our hallway, which is half the size of this room, he was walking so slowly and he's doing much better now.

Mr. BURTON. Well, I want to congratulate you on your hard work and I know he thinks highly of you.

Any other questions?

Dr. Matthiessen, in particular I want to thank you for coming all the way from Germany to be here. I promise you that the information you gave to us today will be used and we will present this information to the Food and Drug Administration, along with the testimony of the other panelists here.

And with that, thank you for being here. We stand adjourned.

[Whereupon, at 4 p.m., the committee adjourned subject to the call of the Chair.]

[Additional information submitted for the hearing record follows:]

Statement of Susan Haeger, President/CEO
Citizens For Health
Submitted to the House Government Reform and Oversight Committee
February 12, 1998

Citizens For Health is a grassroots, non-profit health advocacy organization whose goal is to protect freedom of choice in healthcare and the right to truthful and nonmisleading information to allow consumers to make their own educated healthcare choices. We have chapters in all 50 states and several international member organizations.

Citizens was originally established in April 1992 to organize consumer grassroots efforts to pass the Dietary Supplement Health and Education Act of 1994 (DSHEA). Many of you worked with us on that successful effort and have continued to work hand-in-hand with us to further the principle of consumer right to choice in healthcare. Our membership crosses party lines and represents the broad spectrum of Americans--choice in healthcare affects people of all ideologies and the Access to Medical Treatment Act (AMTA) reflects those interests.

I want to thank Representative DeFazio for being a constant voice for alternative medicine in Congress and for consistently speaking out about the issue of consumer right to choice. He has been the primary sponsor of AMTA in the House and has been steady in his commitment to educate his colleagues about the importance of allowing consumers to choose from the full range of medical options.

The right to choose is a basic American right and is one of the four consumer rights outlined by President Kennedy 38 years ago. Citizens For Health has adopted that right as one of our bedrock principles. In no facet of life is the right to choose so elemental and necessary as in the issue of citizens' health and well-being.

Citizens For Health has been working on AMTA since the bill was first introduced in 1995 by Representative DeFazio in the House and Minority Leader Daschle in the Senate. It has been a frustrating and slow-moving process to get Congress to focus on AMTA. During that time, American voices have only grown in strength advocating access to alternative therapies. Under the present restrictive system, FDA approval of a treatment is required before it can be administered. As we all know, FDA approval of new drugs costs an average of $400 million and takes nearly 15 years. That's too long for people facing life-threatening or chronic illnesses to wait if a safe, efficacious treatment--not on the FDA approved list--is available.

The current system is driving Americans to great lengths--in some cases, even to other countries--to receive treatments that are safe and effective. Last week in the Boulder Colorado newspaper, the *Daily Camera*, the local section featured a 24-year old diagnosed with cancer last year who has sought alternative treatment to control his disease. The son of a surgeon and a former nurse, he felt that conventional medicine was not the answer. He has chosen to travel to Mexico at a cost of over $37,000 to receive a German homeopathic treatment and his cancer is now 80 percent in remission. It's tragic that the option to choose and receive such a treatment doesn't exist in this country--a place where freedom of choice is supposed to be a fundamental right.

Citizens strongly supports the principles of consumer access and choice represented by AMTA. Too many American consumers have suffered from being denied the ability to take care of serious health concerns because of restrictions on available treatments. This Committee heard the testimony of Dr. Stanislaw Burzynski's patients last week and got

firsthand accounts of the importance of being able to choose and pursue effective alternative treatments.

Anyone who sees a family member suffer, especially from a life-threatening illness, would do anything they could to find the best, most effective treatment possible. That is the idea behind AMTA--to allow consumers to find and choose the treatment they want as long as the treatment doesn't cause harm and the patient is fully informed of its risks and side effects.

The concepts behind AMTA may be new here in Congress but the idea of choice shouldn't be. Consumers are way ahead of legislators--well over one-third of Americans already seek alternative treatments and make more visits to alternative practitioners than to primary care physicians.

Consumers have joined forces with alternative care practitioners to pass medical freedom bills similar to AMTA in nine states already--Alaska, Colorado, Georgia, New York, North Carolina, Oregon, Oklahoma, South Dakota and Washington. Advancing patient choice and protecting alternative practitioners from harassment by state health boards has been at the heart of this legislative trend. Similar legislation is pending in 12 additional states. These efforts show consumers' strong desire to make their own healthcare choices and the momentum behind medical freedom legislation is progressing from state to state. Passage of AMTA would set a national precedent and send a clear message that consumers have the right to choose beneficial, safe, non-FDA approved therapies from trained health professionals.

Twenty-two of the 125 medical schools in this country--including Johns Hopkins, Yale, Columbia, Georgetown, Harvard and others--have recognized the value of alternative medicine and have incorporated teaching about alternative methods into their programs. Unfortunately, without legislation like AMTA consumers' treatment options are limited to only those procedures approved by FDA and there is no impetus for more research into alternative therapies.

Over the three years AMTA has been before Congress, Citizens has received a lot of feedback concerning whether the bill contains sufficient consumer protections. Consumer protections do exist in the legislation: • practitioners must meet state licensing requirements; • the treatment cannot pose a significant health risk; • a patient must be informed that the treatment has not been federally approved and that they receive it at their own risk; • a patient must be informed of the nature of the treatment, benefits, side-effects and past results and; • no advertising claims can be made about a treatment. At the same time, Citizens shares the legitimate concern that consumers be allowed choice but are not taken advantage of by ill-meaning practitioners.

We have already begun working to modify the bill as it is written to build in more consumer protections without restricting choice or access. We hope that through these modifications we come up with a stronger bill that allays the fears expressed by some members of Congress and other consumer groups. We welcome the assistance of other interested parties and hope that members of Congress and consumer groups who support peoples' right to choose safe, efficacious treatments will join us in our efforts.

I realize this is not an AMTA hearing, but I hope we've convinced you that we should work to schedule a hearing devoted to considering that bill. I would like to thank Chairman Burton and the other members of the Committee for allowing Citizens For Health to submit testimony on this very important issue.

Drs. T. Geurkink and G.R. Born
2687 44th Street, S. E.
Grand Rapids, MI 49512
June 2, 1996

Dear Drs. Geurkink and Born:

During one of my first visits to your office, beginning late April of 1994 , you stated that one of the problematic medical conditions, namely macular degeneration in the retina of the left eye, might be improved by chelation treatments, combined with appropriate vitamin therapy (antioxidants). However, at that time I was still trusting the alleged effectiveness of laser surgery. The "retinal specialist," whose advice I was following, had been very sensitive to my questions about alternate treatments. He claimed that laser surgery was the only effective treatment available. I naively assumed he knew all the relevant information on the subject.

The macular degeneration of my left eye was first diagnosed in early March 1993 by an optometrist. He immediately made an appointment with the above mentioned "retinal specialist." The first laser surgery took place in early April, 1994 (I only have billing date records). The results were positive, with a reduction in the visual "gray" spot and a slight change to a more opaque appearance. However, deterioration probably began almost immediately. The bleeding apparently was never completely stopped. In early May of 1994, I received the second laser surgery, followed a month later by a third. Neither of these surgeries was highly successful. The "specialist," with considerable resignation, claimed that this was the best that could be done at this time. Following a few follow-up examinations through the summer of 1994, I have not been , to my knowledge, contacted by his office again.

It was in June of 1994 that I receive the first of slightly more than thirty (up to this time) chelation treatments. At the time I was thinking more of improvement in blood circulation and an improvement in vigor and overall health. I definitely feel that such an improvement has occurred. I can, at 65 years, engage in strenuous physical activity for six to ten hours a day, six days a week, with only an old knee injury and the sciatic nerve to the left leg complaining. It wasn't until late in the year of 1994, or even early 1995 that I noticed that the visual "gray" spot (which I noticed only with the dominant right eye closed) was completely gone. I could recognize the outlines of large objects with distinct lines. I tried driving a vehicle, using just the left eye, but there was only very limited depth perception and inability to judge the movements of other vehicles. Over the last year, especially early in the morning, I can pick out letters of large print through parts of the "opaque" spot in the center of my left visual view. I cannot read with the left eye, but I can detect that there are lines of print in front of me.

Since there was only limited temporary improvement with laser surgery, and no promising prognosis for any long-term benefit, I cannot attribute the present stable condition of my left eye to that procedure. (Sometimes I am tempted to seek an "impartial " examination of my damaged eye to establish whether the laser surgery may have contributed to what appears to be permanent damage to the retina). I believe this present stable condition (for about one and a half years now) is due to the only other treatments that I have had, namely, chelation with supplemental antioxidants, under the supervision of you and your staff.

I thank both of you for providing this alternative service to standard treatments which, in my case, were ineffective and possibly even detrimental.

Respectfully,

George G. Janzen, 204 Rose Ave., Big Rapids, MI 49307

Lawrence Clark
817 N. Clinton Lot 718
Grand Ledge, MI 48837

June 24, 1994

To Whom it may concern:

In 1990 I had open heart surgery and in 1993 my M.D. sent me to a neurologist for a circulatory test commonly referred to as a "dye" test.

Upon completion of the above test I was informed that my left veck vein, which supplies a major part of the blood to my brain, was filled with plaque and beyond surgery, and that the other neck vein had also accumulated plaque buildup. I was then informed that when both of these neck veins are plugged off with plaque the result is a major stroke.

The diagnosis of a major stroke awaiting me was not pleasant news. I decided not to sit around and wait for a stroke, I was going to act and act now.

Upong investigation I found a procedure known as Chelation. This procedure is as simple as an IV drip into the arm, with no harmful side affects. After I talked with some people who already had the treatment I decided further investigation was necessary.

Upon arrival at the clinic and a detailed discussion with the doctor, I decided to go ahead with the treatment. Upon completion of multiple tests I started Chelation treatments. Upon completion of 4 treatments I found that I felt very much improved, aches and pains I had had for 40+ years had vanished. Upon completion of the first 20 treatments I felt like I was alive again and had a renewed joy of life.

I am very excited and pleased to have found the Born Preventative Clinic, and am very confused as to why anyone would object to Chelation and preventative medicine.

I feel I have saved my insurance company alot of money, by not having to go through surgerys again. I am sure I have saved myself alot of worry and recovery time in a hospital.

Respectfully,

Lawrence Clark

Lawrence Clark

pc

284 Bona Vista NW
Grand Rapids, MI 49504
August 8, 1994

Dr. Tammy Geurkink
Born Preventive Health Care Clinic
2687 44th St. SE
Grand Rapids, MI 49512

Dear Dr. Geurkink:

You are already aware of the positive results I have had from the treatments and tests I received at your clinic. However, I am restating them here because I would like you to send this letter on to the "powers that be" that will be deciding the future of food supplements and other natural remedies as well as chelation.

For many years a persistent and often deep cough has dogged me. Apparently run down after having cared for my husband who died of ALS (Lou Gerig's Disease), I got a bad case of pneumonia which later recurred. The cough that stemmed from the first care has plagued me very persistently much of ten years, getting more severe during the last two or three years.

I had been going to an M.D. Internist and was very happy with him except for the fact that he was never able to help me get rid of that cough (which made people think I was "really sick" when I wasn't). Having heard of Dr. Born and his preventive health care measures for years and since our family had gone to him when we lived near his office, I decided to try him again.

When an X-ray was taken of my chest, a doctor noticed that a carotid artery was filling with plaque and asked if I had ever considered chelation. I hadn't, at least, not seriously. But when further tests showed that I had had one heart attack (earlier a cardiologist had told me the same thing but I thought very little of it after a heart cath reported other arteries to be fine), and also showed that blood was not getting to certain areas of my body easily, I decided to have chelation. After 20 treatments, a Doppler test revealed dramatic improvement. That would be enough to make me a believer, but the stories from "real, live people" who sat in chairs near me during the treatments were fantastic evidence that it works and works wonders. A retired teacher, for example, had had bypasses and was told they could do no more for him surgically. Then he developed a blood clot from mid-calf to mid-thigh. His cardiologist told him Mother Nature would have to take care of that and that he would always have pain and swelling at times. After only 10 treatments, this man was, in his estimation, 90% better. One woman whom I learned to know quite well was told to swim and exercise in her swimming pool. But, she was unable to walk to the pool because of poor circulation to her left foot. After chelation, she was invited by a friend to stay for a couple of months on a private island in Florida where she walked 4 miles a day on the beach! Similar stories heard regularly make me an avid sales person for chelation.

Because of various tests and treatments and many food supplements given me, I am now free of that horrible, embarrassing cough that plagued me for so long, and have been for quite some time. I neglected to say that I ran out of breath VERY easily. When picking up my small granddaughter, for example, and just walking out the door with her, I huffed and puffed. When I walked up steps, it was the same story. Always people with me would have to wait till I caught my breath. About 2 months ago, I picked up that same (now a bit heavier) granddaughter from the floor, walked away with her and suddenly realized I wasn't one bit out of breath! I go up and down hills during my morning walks and get along just fine.

I honestly feel that all these wonderful things done for me at the Born Clinic are an answer to many prayers . I am thrilled beyond words to awaken each morning with no

horrible squeaks and rattles and groans coming from somewhere deep down in my lungs.

Please do everything you can to ensure that supplements will not be taken away from us and that chelation and other treatments that might not be covered by insurance will be covered. It was not easy financially to pay for chelation, but, believe me, it was worth every penny.

Thank you, Dr. Geurkink, and Dr. Born for providing this kind of service!

Sincerely,

Lois Johnson

Susan Morse

April 30, 1993

Grant R. Born, D.O.
Born Preventive Health Care Clinic
2687 - 44th St. S.E.
Grand Rapids, MI 49512

Dear Drs. Grant & Tammy Born,

I would like to properly thank you for the preventive care you have provided me since October, 1992. It has significantly changed my life.

It is incredible to me that our insurance company refuses to pay the costs of preventive medicine. How can this be? Since becoming your patient the costs have been substantially reduced from the expense of reactive treatment.

In 1991, after many months of feeling exhausted (one year of which was spent "bed resting") I was diagnosed as having CFS (Chronic Fatigue Syndrome) by my internist. I appreciated the diagnosis, because; I could not understand how after many years of being socially, physically and intellectually active there was a reason for my fatigue and mental confusion.

At the request of my physician, I returned to his office every two weeks for routine checkups, blood tests, consultations, etc. He basically said the only thing I could actively do to help myself was to rest.

I rested for almost two years and did not improve. I began having frequent migraine headaches. I would receive medical attention for these headaches. The cost incurred are as follows and insurance paid for all the treatments with no questions asked. These figures speak for themselves.

1991 $ 5,997.99 - Jan. 1992 through Oct. '92 $ 4,345.00

Born Preventive Care 10/16/92 - 4/29/93 $ 1,192.00

Since I have been a patient of yours, I have not had one migraine and have resumed activity almost 100% to where I was two years ago.

When I first came to Born Clinic you told me that you could help me and I would feel better, and you were right. The cost comparison speaks for itself. The Gaby Wright injections have significantly helped me. My quality of life has increased to normal levels and I am very thankful to you. We need doctors like you to guide and educate us.

How can I help to make this available to other people when under our health care system your preventive care is not covered by insurance?

Is it possible for our health care programs to be unaware of preventive medicine or, are they simply ignoring it? We need to work together to educate the health care companies and our government about preventive medicine, rather than reactive medicine.

Thank you again and if I can ever be of help, please let me know!

Sincerely,

Susan Morse

Susan Morse