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THE IMPACT OF MEDICAL DEVICE AND DRUG REGULATION ON INNOVATION, JOBS, AND PATIENTS: A LOCAL PERSPECTIVE

HEARING

BEFORE THE

SUBCOMMITTEE ON HEALTH

OF THE

COMMITTEE ON ENERGY AND COMMERCE
HOUSE OF REPRESENTATIVES

ONE HUNDRED TWELFTH CONGRESS

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FIELD HEARING ON THE IMPACT OF MEDICAL DEVICE AND DRUG REGULATION ON INNOVATION, JOBS, AND PATIENTS: A LOCAL PERSPECTIVE

MONDAY, SEPTEMBER 26, 2011

HOUSE OF REPRESENTATIVES,
SUBCOMMITTEE ON HEALTH,
COMMITTEE ON ENERGY AND COMMERCE,
Washington, DC.

The subcommittee met, pursuant to call, at 11:28 a.m., at the Scripps Seaside Forum, Scripps Institution of Oceanography, 8610 Kennel Way, La Jolla, California, Hon. Michael C. Burgess presiding.

Members present: Representatives Burgess and Bilbray.

Staff present: Clay Alspach, Counsel, Subcommittee on Health; Carly McWilliams, Legislative Clerk; Allison Corr, Minority Policy Analyst.

Mr. BILBRAY [presiding]. Let's go ahead and move it a little earlier. In the tradition of George Marshall, we will start the proceedings 5 minutes early. Those of you who don't understand that can pull up your history books and find out about the Supreme Court Justice.

I want to thank everybody for being here. Let me say, clearly, we want to thank Dr. Burgess for showing up. The Congressman took a long flight to come out here. I think we all owe him a real thanks for not just caring but acting on his concerns, bringing his expertise in.

And I want to thank you very much, Doctor, for taking the time to come over here. Next time, we will try to arrange these hearings during February, where you might appreciate it a little more.

[Laughter.]

And I bet you the turnout will be much larger, too.

I think that one of the things that we want to point out is that San Diego, obviously, is not just the land of sun and sea and sand. It is definitely a generator of major medical breakthroughs and a major employer; 40,000 people in the life sciences here.

I think, though, too often, those of us in Washington take a look at the life sciences and medical research and see it as an abstract. And this is not just an issue, again—and I will say this again and again—we are not just talking jobs. We are talking lives.

And this is an industry that needs to be addressed from both sides. But we always have to remember, the endgame is to save lives, and not just an elderly mother or grandmother, but, more im-

portantly, make sure that, in the future, our grandchildren can have their most prosperous and healthy life possible, because we did the right thing.

One of the biggest challenges I want to point out is that getting a team to work together—and Tony? Where is Tony? Did he leave?

Mr. BURGESS. He is making a phone call.

Mr. BILBRAY. OK, I always love to thank the guy who allowed us to come hang out in these digs, so we will get to him.

OPENING STATEMENT OF BRIAN P. BILBRAY, A REPRESENTATIVE IN CONGRESS FROM THE STATE OF CALIFORNIA

But let me just start off, right off, that what we need to talk about here is what are the opportunities and the challenges of making sure that we not only maintain a research capability in this country that creates jobs, but one that can—we can change our operations to be able to make sure of those breakthroughs.

And one of the biggest frustrations I have had is that everybody thinks medical research is all about spending money, and that in Washington, it is easy for us to write checks and throw money at a problem. And I think some of the latest scandals you have seen coming out of Washington, directed at certain assumptions, was thinking just spending money will cure a problem. And I really want to go back to the fact that in our lifetime, we saw a major success for the AIDS epidemic, and too many people think it was just because we threw money at the problem, don't go back and realize the changed regulatory oversight. We made the bureaucracy in Washington approach it differently.

And I don't think, in our lifetime, we have seen such a dramatic breakthrough in health benefits. In fact, I think somebody was calling me down that it was not just 37 but probably 50 times longer life expectancy than what they had before once we get into it.

Imagine if we can do that for diabetics, do it for cancer patients, do it for people with MS. That kind of breakthrough may be possible. But we will never know if we don't change the way Washington handles these issues.

And I don't think this is a Democrat or Republican issue. Sick children do not carry a party affiliation with them. They, basically, all have the right to be able to have the same access to medical breakthrough as anybody else. And that is a big challenge I think we need to talk about.

First thing we need to do is remember, though, too, especially those of us who are on our side of the aisle, there is a place for regulation. There is a place to regulate.

What we are worried about is there is a big difference between regulating and strangulating. And I think that is the one frustration that I get through. And I think that if we look at the people who will testify today, good research, good health, knows no party affiliation, knows no political lines. And, hopefully, we will be able to address those issues.

I think the California Healthcare Institute talks about the delays that the FDA has put out and the mindset. I have to remind you though, too, this is not just an FDA challenge. Hopefully, a lot of people here can look at what we are trying to do in Washington and make sure you guys in the private sector not only have the

basic research that government pays for, not only has the regulatory reform that makes it easier for you to create the miracles that we take for granted, but also that we take a look at how Washington operates, and make sure that we bring in capital into this country that you can use to stay in business, so that we see the next generation of miracles.

And I just worry that the American people take you for granted, take medical breakthrough after medical breakthrough for granted. And I think that it is important for us to make sure that that doesn't happen anymore.

Let me just close by saying, again, we are here today not just to talk about something that is very important during one of the most critical economic downturns in the history of this country. We are not just talking about jobs. We are also talking about lives. And it is not very often that you can talk about something that is good for the economy and good for the environment that humans live in. Too often we have these tough choices of, is it good for the economics, or is it good for public health? This is a proposal that is good for both. And it is one we want to build on.

So I would like to throw it over to my colleague, Dr. Burgess. And it is yours.

[The prepared statement of Mr. Bilbray follows:]

Opening Statement of the Honorable Brian Bilbray
Energy and Commerce Subcommittee on Health
"Impact of Medical Device and Drug Regulation on Innovation, Jobs and Patients: A Local Perspective"
September 26, 2011
(*As Prepared for Delivery*)

Mr. Chairman, distinguished colleagues, and guests. Welcome to San Diego, California. America's finest city. I am proud to call this great city home and look forward to listening to our panelists and discussing how these innovative local companies can create jobs and grow our economy if we just get government off their backs.

Special thanks are in order for Tony Haymet for allowing us to use this location for our hearing. Dr. Haymet is the Director of Scripps Institution of Oceanography, Vice Chancellor for Marine Sciences, and Dean of the Graduate School of Marine Sciences at University of California, San Diego. Not only is Scripps a beautiful campus, it is home to some of the most exciting research in the world.

California, and particularly San Diego, is home to many of the most exciting and advanced life science companies in the world. The San Diego life science cluster is one of the largest in the world. There are approximately 40,000 employees at more than 700 companies, including biotechnology, medical device, diagnostic companies, and research institutes. San Diego is internationally recognized for its innovation, producing some of most important discoveries in the world.

It is no secret that the best markets are the freest markets. Removing government barriers to job growth is necessary in any business, whether it be in a biotech company looking for the cure for cancer or a device company making an artificial pancreas for a diabetic. Unfortunately, we have seen the FDA put up roadblock after roadblock for companies looking to expand their businesses and develop the next great breakthrough.

According to the California Health Care Institute (CHI), delays in the FDA regulatory process have alarmed the entire U.S. biomedical industry. For new drugs and biologics, average review times increased by more than four months. This uncertainty has lead to a decrease from investors who fear FDA's policies will impede development.

According to our witness today, Sharon Stevenson, between January and June 2011, venture capital investments in seed and first round medical device start ups declined by nearly 50 percent. This is a major decline in investments that can go towards creating well paying jobs that may someday lead to major breakthroughs. But a lack of certainty from the FDA is hindering investment.

It is not only the delay in approval that is alarming; it is the very culture within FDA that I find disturbing.

We must do more to empower the patients to be part of the entire FDA process: we need to review the conflict of interest rule so that we may have the best and brightest reviewers even if they have industry experience. We need to make the research and development tax credit permanent. We need to lower the corporate tax rate for companies who want to bring money back from overseas. We need the public and private sector research entities to work together to advocate for one another.

The San Diego region contains the best and brightest America has to offer. These innovative companies have the potential to turn our economic climate around. We need to help them by providing a clear path free of government interference. Thank you for taking the time today to tell your story.

###

Mr. BURGESS. Thank you, Brian.

Now, was that your opening statement or was that just a filibuster?

[Laughter.]

Why don't we go ahead and call the subcommittee to order, and I want to thank our witnesses for being here with us here today. I, obviously, want to thank the University for opening up this very stunning facility for us to use today.

OPENING STATEMENT OF MICHAEL C. BURGESS, A REPRESENTATIVE IN CONGRESS FROM THE STATE OF TEXAS

And let me begin by making an opening statement, and first state, for the record, that I am not just happy but I am thrilled to be here today, talking about a very important topic and the impact of the medical device industry in the United States. It is more than just what we do for patients. It affects commerce, technology, as well as the economy by providing quality jobs to Americans, which range from highly skilled technical to those involved in the manufacture of those devices.

Unfortunately, the jobs are slipping away. And with these jobs, new technologies and innovations are being driven overseas, and our economy is suffering, and, most importantly, our patients are denied access to these devices.

In a recent study conducted by PricewaterhouseCoopers, United States consumers are already being shown as not being the first to benefit from medical technology. And without change, the process could eventually—we could eventually be last. In fact, innovators are already going first to the European market, and, by 2020, will likely move into other emerging economies.

And I hear about this literally every week in my office. Because I am a physician by background, someone is in my office with a perplexed tale of woe about what their difficulties have been in trying to get a drug or device approved through the FDA, an FDA that seems to consistently discourage innovation and leads to American job loss. Innovators cite their inability to facilitate a predictable process as reasons to move overseas.

And this was no more evident than a few weeks ago when we had a hearing in our subcommittee back in Washington, and I just reference, I spent a lot of time on the plane this morning. I got to read every word of the Wall Street Journal. And on page three is an article about a new device that has now received provisional approval by the Food and Drug Administration, the MelaFind device.

Now, just a few weeks ago, it was denied. And the Journal, I think, correctly cites—they don't correctly identify Brian Bilbray and myself, but they correctly cite:

"The case was a focus of a House of Representatives hearing this summer at which the FDA's top device regulator, Jeffrey Shuren, acknowledged the agency had mishandled the MelaFind application. Dr. Shuren mentioned the Food and Drug Administration's decision to hold a meeting of its advisers on MelaFind after the agency had already decided to reject the device, the reverse of the usual order.

'"The staff made the wrong call,'" said Dr. Shuren.

Now the United States is currently exporting over $34 million a year in medical devices, and those industries employ more than 27 million people. No, it does not take a rocket surgeon to understand that in this current economic environment, this is the type of stimulus that is needed by our economy.

The Food and Drug Administration's failure to ensure a reliable and consistent approval process not only creates a disadvantage for current devices, but signals their inability to handle the advancements of technology in the future.

The United States has always led the way in innovation and technology. As we customize medicine, it is imperative that the Food and Drug Administration have the ability to thoroughly examine these devices in a timely manner.

Brian is right. We do not want to sacrifice patient safety. That should be the number one priority when looking at devices. However, the long and arduous process that is frequently ambiguous adversely affects everyone, including those patients who desperately need the device.

In addition, the United States medical device manufacturers will also be hit with a 2.3 percent tax on revenues due to new requirements in the Patient Protection and Affordable Care Act. For many smaller businesses, this tax be detrimental and cause many to shut their doors, effectively stifling technological advancements.

President Obama, in the State of the Union Address, advocated for America to lead the way on technology and innovation. In that, there is complete agreement with this Member of Congress. Unfortunately, his signature legislation coupled with the Food and Drug Administration's confusing and somewhat disjointed approval process has instead encouraged the off-shoring of business and brought medical discoveries to a halt. It is essential that the Food and Drug Administration process becomes more transparent for all involved.

[The prepared statement of Mr. Burgess follows:]

Opening Statement of the Honorable Michael C. Burgess, M.D.
Vice Chairman, Subcommittee on Health
"Impact of Medical Device and Drug Regulation on Innovation, Jobs and Patients: A Local Perspective"
September 26, 2011
(As Prepared for Delivery)

The impact of the medical device industry in the United States is further reaching than just the patients that benefit from them. It affects commerce, technology as well as stimulating our economy providing quality jobs to Americans which range from highly skilled and technical to those involved in the manufacturing.

Unfortunately, these jobs are quickly slipping away, and with these jobs, new technologies and innovations are being driven overseas and our economy and our patients are suffering.

In a recent study conducted by PriceWaterhouseCoopers, US consumers are already being shown as not being the first to benefit from medical technology and without a change in the process we could eventually be last.

In fact, innovators already are going first to market in Europe and, by 2020, likely will move into emerging countries.

I hear from device manufactures across the country about the lack of transparency and an ambiguous, if not constantly changing, approval process at the FDA that discourages innovation and ultimately leads to American job loss.

Innovators cite their inability to facilitate a predictable process as reasons to move overseas.

The United States is currently exporting over $34 million a year in medical devices that employs more than 27 million people. In our current economic environment, this is the type of stimulus our economy needs.

The FDA's failure to ensure a reliable and consistent approval process not only creates a disadvantage for current devices, but signals their inability to handle the advancements in technology of the future.

The United States has always led the way in innovation and technology in this field. As we customize medicine it is imperative the FDA have the ability to thoroughly examine these devices and in a timely manner.

In no way do I want to sacrifice patient safety; that should be the priority when looking at devices. However, the long drawn out and ambiguous process adversely affects everyone – including those who desperately need these treatments.

In addition, US medical device manufactures will also be hit with a 2.3% tax on revenues due to new requirements in PPACA. For many smaller businesses this tax will be detrimental and cause many to shut their doors effectively stifling technological advancements.

President Obama, in his State of the Union, advocated for America to lead the way on technology and innovation. Unfortunately, his signature legislation coupled with FDA's confusing and somewhat disjointed approval process has instead encouraged the off shoring of business and brought medical discoveries to a halt.

It is essential the FDA process becomes more transparent for all those involved in the process. I look forward to hearing more from our witnesses and I yield back.

###

Mr. BURGESS.Now, I look forward to hearing from our witnesses. Again, I want to thank all of our witnesses for being with us today. We are going to hear from each of you, in turn.

We will begin, I think, with Mr. Walton, and then we will hear from Dr. Gollaher, who is the president and chief executive officer of the California Healthcare Institute. We have Dr. Sharon Stevenson on behalf of the National Venture Capital Association. We have Dr. Steven Mento, who is the Biotechnology Industry Organization representative; Joe Panetta, president and chief executive officer of BIOCOM; Kevin Larkin, the president and chief executive officer of TherOx, Inc.

Did I leave anyone out? Mr. Donald Casey—where did you go? Oh, there you are. Chief executive officer of the West Wireless Health Institute, who of course is very familiar to me, us having met in Washington on more than one occasion.

I would like to now recognize our first witness, Mr. Bill Walton, for his opening statement.

You are recognized, sir. Five minutes.

STATEMENTS OF BILL WALTON, EXECUTIVE CHAIRMAN, CONNECT SAN DIEGO SPORT INNOVATORS; DAVID L. GOLLAHER, PRESIDENT AND CHIEF EXECUTIVE OFFICER, CALIFORNIA HEALTHCARE INSTITUTE; SHARON STEVENSON, DIRECTOR, OKAPI VENTURE CAPITAL, LLC, ON BEHALF OF THE NATIONAL VENTURE CAPITAL ASSOCIATION; STEVEN J. MENTO, PRESIDENT AND CHIEF EXECUTIVE OFFICER, CONATUS PHARMACEUTICALS, INC., ON BEHALF OF BIOTECHNOLOGY INDUSTRY ASSOCIATION; DONALD M. CASEY, CHIEF EXECUTIVE OFFICER, WEST WIRELESS HEALTH INSTITUTE; JOE PANETTA, PRESIDENT AND CHIEF EXECUTIVE OFFICER, BIOCOM; AND, KEVIN T. LARKIN, PRESIDENT AND CHIEF EXECUTIVE OFFICER, THEROX, INC.

STATEMENT OF BILL WALTON

Mr. WALTON. Thank you, Congressman, and good morning. My name is Bill Walton, and I am here today as a patient advocate, proud, honored, and privileged to have the opportunity to give back with my duty, obligation, and responsibility for those people, like me, who are suffering in extreme pain, and who were, at one point, hopeless that their lives could ever return to normal.

Now in addition to sharing with you the patient perspective regarding the impact medical innovation can have, I also want to ask you, and urge you, to cultivate a statutory and regulatory atmosphere with clear rules and guidelines in the medical innovation field, so that we can continue here in the United States to lead the world in the creation of lifesaving medical devices, drugs, and health care.

Now, while the U.S. continues to maintain its global edge in groundbreaking, game-changing medical technology, much of it occurring right here in my hometown of San Diego, the rest of the world is closing in on us fast. The world, they have copied what we have done here in America. And they are using our techniques and our talents to achieve our unmatched success story.

However, while U.S. companies struggle with longer wait times for regulatory approval in an increasingly uncertain regulatory approval process, our global competitors are streamlining their regulatory systems, which attract capital and companies to their shores from ours.

You, as our congressional leaders, you have the ability to recognize this problem, and create laws and regulations that protect our innovation edge, which means that more patients will have life-changing stories to tell, like the one you will hear today.

My UCLA college basketball coach, John Wooden, he used to tell us all the time, "Don't measure yourself by what you have accomplished, but rather by what you should have accomplished with your ability."

America's history of great innovation is not enough to be satisfied with. There are many more brilliant scientists and doctors who want to help people and cure diseases. But they can only accomplish up to their ability, if our Nation's medical innovation ecosystem continues to encourage discovery and reward risk.

My story of disability and pain, which has turned to healing because of what these emerging companies are creating, is why I am here today. My college basketball career took me all over this great country, but it was a frigid night in January 1974, more than 37 years ago, when our UCLA Bruins traveled to Washington State University at Pullman, Washington, the southeastern part of that great State, and the Bruins, we were riding our 84-game consecutive winning streak, for a big Pac-8 conference showdown.

During the game, I was making a play at the ball, and a guy on the other team, he came from the other side of the court and took my legs out from underneath me and I flipped over, came crashing down, and landed on my spine on a synthetic floor. That night, I fractured two bones in my spine, and things were never the same for me again.

Throughout my NBA game career, I worked as hard as I could to stay in shape and to stay healthy, but that pain was always with me in my spine, the discomfort, the limitation, the restrictions.

After another nearly 20 years in the broadcast business, 200 nights a year on the endless road, in airplanes and through a man-made world ill-suited for a 6'11" body, the back pain ultimately became unbearable. My spine simply could not hold me up anymore.

The pain was debilitating, excruciating, and relentless. Visualize yourself being submerged in a vat of scalding acid with electrifying current running through it, and you could never get out, ever.

My life was over. I had nothing. I ate my meals on the floor face down. I couldn't get any sleep. I couldn't get dressed. Nothing eased the devastating nerve pain radiating throughout my body, coming from my spine. It got to the point where my life wasn't worth living. I was standing on the edge of the bridge, knowing full well that it was better to jump than to go back to what was left of my life.

I was lucky. I was saved. I came across medical innovators that reconstructed my spine and gave me my life back.

I had no idea what life was like without back pain. They saved my life. Dr. Garfin, here at UCSD, the head of the orthopedic spine clinic. NuVasive, a company here in San Diego. They combined on

a new surgical technique operating on my spine by entering through the side and deploying NuVasive's medical devices to rebuild my back. I underwent 8 1/2 hours of XLIF surgery. I was amazed by the relief that came at the end of the relentless, excruciating pain that ran constantly through my entire body.

After I started to get better, I began to do things I hadn't been able to do in years, like put on my own shoes and socks, to be able to bend over and pet the dogs.

It was right around the seven-month mark after my surgery when I really turned a corner and found freedom one more time, pedaling my bicycle with no limitations. Riding on the open road, the wind and the sun in my face, the film of sweat on my body, that was the greatest outcome in the world for me.

I had lost everything. But now I am back in the game of life and climbing to the top of the mountain one more time.

The story of NuVasive is one of those high-growth, job-creating companies that Washington wants to replicate all over the country, but which sadly faces substantial challenges because of the uncertain and prolonged regulatory approval process. NuVasive is a public medical device company focused on developing minimally disruptive surgical products and procedures for the spine.

The company has grown from a venture-capital-backed startup company 10 years ago to one with more than $500 million in annual sales in just 10 years.

NuVasive is now the fourth largest spine company in the U.S. and the fifth largest worldwide. This industry-leading growth has propelled the company to a global presence employing over 1,100 people.

From a technology perspective, NuVasive's sole focus is to advance spine surgery by developing new products and procedures that provide superior surgical outcomes and improve patient health. NuVasive's innovation in surgical technique and devices have great benefits for the patient, including reduced operative time, reduced blood loss and minimal scarring, reduced post-operative pain, reduced hospital stay, and rapid return to normal activities.

Despite NuVasive's innovative techniques and technologies, their future faces serious obstacles because of the uncertain U.S. regulatory approval process and a new tax on medical devices past in last year's health care reform law.

Over the past 18 to 24 months, NuVasive has experienced longer delays related to FDA product clearances and approval in 510(k) and premarket approval applications. Longer FDA approval times will potentially result in significant revenue loss estimated at up to $70 million over the next 2 years, increased operating expenses of over $2 million, and hundreds of new jobs eliminated.

Historically, NuVasive has launched at least 10 new products per year. Because of the stifling regulatory climate, that number is being reduced by half.

It is becoming far more efficient and faster to innovate outside the United States, in places like Europe. Non-U.S. systems have more timely, more predictable, and more transparent processes, and that is not acceptable for us as patients or as Americans.

This means that instead of creating new American jobs, those jobs, manufacturing plants, distribution networks, related innovations, and profits have a substantial likelihood of moving overseas.

As a result of costs associated with protracted approval and clearance processes, NuVasive has committed to fewer R&D projects in 2011 and '12. This results in less innovation and this has caused them to reduce hiring by at least 15 percent, with 150 fewer new jobs versus their 2011 projected headcount.

For many reasons, we can't have a regulatory framework that hinders job creation. NuVasive does not believe a major overhaul of the current FDA process is needed. Instead, they need process predictability and timeliness from the reviewers, and to ensure that approvals are being efficiently processed under the current high safety standards.

Now, as part of the 2010 health care reform legislation, medical device manufacturers will be required to pay a new 2.3 percent excise tax on all FDA-approved devices sold in the United States. This tax is punitive in nature and will likely raise costs throughout the health care system.

In response to the new tax, it is expected that the industry will reduce R&D spending and jobs domestically while pressure will increase to invest overseas. This will not only harm the economy, but, most importantly, it will harm patients. This tax is based on revenue, causing successful, rapidly growing companies like NuVasive to be hit the hardest, making it more difficult to invest in growth and innovation.

Wall Street estimates that large-cap companies will have up to a 5 percent decrease in profitability while small to mid-cap companies see a 10 to 15 percent decrease. Using 2013 Wall Street revenue projections, NuVasive would be required to pay almost $15 million in pretax dollars, equating to an approximate 13 percent reduction in profit.

This type of increased tax burden will potentially result in over 100 new highly skilled jobs being eliminated.

It is puzzling to me why congressional leaders would target an industry that is so fruitful and productive. The medical device industry creates nearly 2 million high-wage jobs nationwide, with wages approaching 40 percent higher than the average national wage. Approximately 1.5 million additional jobs are created in manufacturing, suppliers, and service providers.

In California alone, our home State, the industry employs over 80,000 individuals with $5.5 billion in payroll, and $26 billion in revenue. This results in almost 210,000 additional jobs driving an additional nearly $7 billion in payroll and over $31.5 billion in revenue.

The tax penalizes an industry that is innovative and that achieves the main goals of health care reform: lowering costs while improving patient care.

The tax should be repealed. And I want to commend Congressman Bilbray for his leadership in introducing H.R. 734 to repeal this punitive tax.

Now, I have been most fortunate in my life to have learned from many great innovators: John Wooden, Jerry Garcia, Larry Bird, Bob Dylan, Tchaikovsky, Maurice Lucas, Dr. Garfin, NuVasive, and

my current interactions with the inspirational innovators like Duane Roth at CONNECT, and the founders of all the new companies that are trying to make it with the help of our new organization, San Diego Sport Innovators, the newest division of CONNECT.

I have been able to help these sport innovators in the organization grow but only because, as a patient, I am the beneficiary of medical innovation.

Now as a patient, we see risk and benefit a bit different than regulators sitting in historic buildings in Washington. When I was face down on the floor with extreme pain coursing through my nerves, I desperately needed the treatments that NuVasive was creating, in conjunction with the skill and precision of a master surgeon like Dr. Garfin.

What the regulatory system needs is a balanced approach to create the incentive needed for investment in innovation, coupled with a predictable and prompt regulatory process that still provides reasonably safe and effective medical devices and drugs. I worry that the regulatory process is so focused on perfection, which does not exist, that it thwarts important creations from reaching the marketplace and saving other people that are on that bridge just like I was.

Coach Wooden also tried to teach us a most applicable lesson here: Don't let what you cannot do interfere with what you can do.

Maybe you can't solve all the problems, but that shouldn't dissuade you from doing what you can to move the medical innovation ecosystem toward more discovery, more reward, and more jobs, which will result in more patients living a healthy life.

Here's to a full and pain-free life with lots of jobs for everyone. Thank you, good luck, and please realize that one day everybody in this room, including yourself, will be facing that moment when you need help, when you need that medical practice, device, technique, company to be right there. Act today, so that you can enjoy tomorrow.

Thank you.

[The prepared statement of Mr. Walton follows:]

Testimony of

Bill Walton

Executive Chairman of
CONNECT San Diego Sport Innovators

For the U.S. House of Representatives

Committee on Energy and Commerce

Subcommittee on Health

Hearing on

"Impact of Medical Device and Drug Regulation on Innovation, Jobs and Patients: A Local Perspective"

September 26, 2011

Chairman Upton, Ranking Member Waxman, Subcommittee Chairman Pitts, Subcommittee Vice-Chairman Dr. Burgess, Ranking Member Pallone, Representative Bilbray, and other distinguished House members, my name is Bill Walton and it is a distinct honor and privilege to testify before this Committee of the United States Congress. I want to earnestly thank you for your invitation to testify today which gives me the opportunity to fulfill my duty, obligation and responsibility to other people who are suffering, who are in extreme pain, and who, like me, were hopeless that their lives could ever return to normal.

In addition to sharing with you the patient perspective regarding the impact medical innovation can have, I wish to humbly request your earnest efforts to ensure that Congress does its best to cultivate a statutory and regulatory atmosphere with clear rules and guidelines so that medical innovators can continue to keep the United States leading the world in the creation of life-saving medical devices, drugs and healthcare.

While the U.S. continues to maintain its global edge in ground-breaking, game-changing medical technology, much of it occurring here in San Diego, the rest of the

world is closing in on us—fast. The world has copied the ingredients America uses to achieve our unmatched success story. However, while U.S. companies struggle with longer wait times for regulatory approvals and an increasingly uncertain regulatory approval process, our global competitors are streamlining their regulatory systems which attract capital and companies to their shores.

You, as Congressional leaders, have the ability to recognize this problem and create laws and regulations to protect America's innovation edge which means that more patients will have life-changing stories to tell like the one you'll hear today.

My UCLA college basketball, John Wooden used to tell us all the time, "Don't measure yourself by what you have accomplished, but by what you should have accomplished with your ability."

America's history of great innovation is not enough to be satisfied with. There are many more brilliant scientists and doctors who want to help people and cure diseases, but they can only accomplish up to their ability if our nation's medical innovation ecosystem continues to encourage discovery and reward risk.

On the Bridge

Today, you will hear important data from other accomplished speakers regarding the problems that our medical innovators face with the regulatory approval process along with some suggested solutions.

I want to share with you my story of pain turned to healing because of what these emerging companies are creating.

My college basketball career took me all over this great country, but I'll never forget that frigid night in January of 1974 when our UCLA Bruins traveled to Washington State University in Pullman, Washington riding our 84 game consecutive winning streak for a Pac-8 conference game.

During the game, I was high above the rim making a play on the ball when an opposing player cut my legs out from underneath me, flipping me upside down, resulting in a crash-landing on an unforgiving synthetic floor.

I broke two bones in my spine that night, and things were never the same for me again.

Throughout my NBA career, I worked as hard as I could to stay in shape and stay healthy but that back pain was always there. That discomfort. That limitation. That restriction. Then after another nearly twenty years in the broadcast business, traveling 200 nights a year on the road, in airplanes and through a man-made world ill-suited for 6'-11" bodies, the back pain became unbearable.

My spine simply could not hold me up anymore.

The pain was debilitating, excruciating and relentless. Visualize yourself being submerged in a vat of scalding acid with an electrifying current running through it, and you can never get out—ever

My life was over. I had nothing. I ate my meals on the floor, face down. I couldn't get any sleep. I couldn't get dressed. Nothing eased the devastating nerve pain emanating from my spine. It got to the point where my life wasn't worth living. I was standing on the edge of the bridge, knowing full well that it was better to jump than to go back to what was left of my life.

I was lucky. I was saved.

I came across medical innovators that reconstructed my spine and gave me my life back. I had no idea what life was like without back pain. They saved my life.

Dr. Steven Garfin, Chairman of the Department of Orthopedics at the University of California San Diego Medical School, working with San Diego emerging company NuVasive, combined on a new surgical technique of operating on the spine by entering through the side and deploying NuVasive's medical devices to rebuild my spine. I underwent eight-and-a-half hours of XLIF® surgery and was amazed by the relief that

came with the end of the relentless, excruciating pain that ran constantly through my entire body.

After I started to get better, I began to do things again I hadn't been able to do in years, like put on my own shoes and socks and bend over and pet the dogs. But it was right around the seven-month mark when I really turned the corner and found freedom again, pedaling my bike with no limitations. Riding on the open road-- the wind and the sun in my face, the film of sweat on my body—that was the greatest outcome in the world for me. I had lost everything. But now I'm back in the game of life and climbing to the top of the mountain one more time.

Medical device innovator NuVasive and the uncertain regulatory approval process

The story of NuVasive is one of those high-growth, job-creating companies that Washington wants to replicate all over the country but which sadly faces substantial challenges because of the uncertain and prolonged regulatory approval process.

NuVasive is a public medical device company focused on developing minimally disruptive surgical products and procedures for the spine. The company has grown from a venture capital backed start-up company to $500 million in revenue over the past decade. The company's product portfolio is focused on applications in the $4.6 billion U.S. spine fusion market. NuVasive is now the 4th largest spine company in the U.S. and the 5th largest player in the $7.7 billion global spine market.

This industry leading growth has been driven by a core value of "Speed of Innovation"™ which has propelled the company to a global presence employing over 1,100 people. From a technology perspective, NuVasive's sole focus is to advance spine surgery by developing new products and procedures that provide superior surgical outcomes and improved patient health.

NuVasive's principal product offering is based on its Maximum Access Surgery, or MAS® platform. The MAS platform combines four categories of products that collectively minimize soft tissue disruption during spine surgery with maximum visualization and safe, easy reproducibility for the surgeon; a proprietary software-driven nerve avoidance system; MaXcess®, a unique split-blade retractor system; a wide variety of specialized implants; and several biologic fusion enhancers.

NuVasive's innovative solutions provide the following benefits:

- Maximum access with minimal disruption
- Innovative solutions for a safe and reproducible minimally disruptive fusion
- Continuous assessment of nerve safety
- Innovative implants for fusion
- Unique motion preservation techniques

This innovation in surgical technique and devices have great benefits for the patient including:

- Reduced operative time
- Reduced blood loss and minimal scarring – The MaXcess® retractor dilates the tissue rather than cutting, resulting in much less trauma to the affected area.
- Reduced postoperative pain – The XLIF procedure does not require entry through sensitive back muscles, bones, or ligaments, so patients are usually walking the same day.
- Reduced hospital stay – XLIF typically requires only an overnight stay in the hospital, compared to the standard multiple days of immobility and hospitalization typical of traditional open approaches.
- Rapid return to normal activity – Patients are usually walking the same day after surgery and recovery is typically around 6 weeks, compared to 6 months or more.

With over 65 products today spanning lumbar, thoracic and cervical applications, NuVasive is committed to continuing to expand and evolve its offering predicated on its R&D focus and dedication to outstanding service levels supported by a culture of "Absolute Responsiveness"®.

Despite NuVasive's innovative techniques and technologies, their future faces serious obstacles because of the uncertain U.S. regulatory approval process and a new tax on medical devices passed in last year's healthcare reform law.

Over the past 18-24 months, NuVasive has experienced longer delays related to FDA product clearances and approval in 510(k) and Pre-Market Approval applications.

Longer FDA approval times will potentially result in significant revenue loss estimated at up to $70 million over 2 years, increased operating expenses of over $2 million, hundreds of new jobs eliminated, and less investment in research and development. Historically, NuVasive has launched at least 10 new products per year. Because of the stifling regulatory climate, that number is being actively reduced to 50%.

It is becoming far more efficient and faster to innovate outside the U.S. in such places as Europe. Non-U.S. systems have more timely, predictable and transparent processes.

This means that instead of creating new American jobs, those jobs, manufacturing plants, distribution networks, related innovations, and profits, have a substantial likelihood of moving overseas.

NuVasive has seen U.S. delays of 3-70 months which has forced NuVasive to rethink longer term strategies around where to place R&D jobs. As a result, NuVasive has committed to fewer R&D projects in 2011 and 2012 as a result of costs associated with protracted approval/clearance processes.

This results in less innovation and has caused them to reduce hiring by at least 15% with 150 fewer new jobs versus their 2011 projected headcount.

For many reasons, we can't have a regulatory framework that hinders job creation.

A specific example of how regulatory uncertainty causes lost revenue and lost jobs is NuVasive's NeuroVision 510(k) for nerve monitoring that was granted nearly a decade ago.

Since then, *nearly 150,000 cases have been successfully performed*. On submission of a new 510(k) for a next generation nerve monitoring system, *which is already cleared in Europe, Japan, and Asia Pacific*, the FDA sought to re-open the user interface of *the original NeuroVision to reassess the predicate*. Actions like this would lead to significant loss of revenue and added expense despite presenting no new safety issues.

NuVasive does not believe a major overhaul of the current FDA process is needed. Instead, they need process predictability and timeliness from the reviewers and to ensure that approvals are being efficiently processed under the current high safety standards.

Innovation-hindering Medical Device Tax

As part of the 2010 health reform legislation, medical device manufacturers will be required to pay a new 2.3% excise tax on all FDA approved devices sold in the U.S. The tax is punitive in nature and will likely raise costs throughout the healthcare system. Companies will be forced to reduce jobs and research and development investment.

This will not only harm the economy, but more importantly it will harm patients. This *tax is based on revenue, causing successful rapidly growing companies, like NuVasive, to be hit the hardest, making it more difficult to invest in growth and innovation.*

The medical technology industry is making decisions today to address the expected tax, including fewer dollars for research and development and new jobs, and

more pressure to invest overseas. This dynamic is causing companies like NuVasive to focus much more heavily on international markets.

Wall Street estimates predict that large cap companies will have up to a 5% decrease in profitability while small to mid-cap companies could see 10-15% decrease. *Using 2013 Wall Street revenue projections, NuVasive would be required to pay almost $15 million in pretax dollars equating to an approximate 13% reduction in profit. This type of increased tax burden will potentially result in over 100 new highly skilled jobs being eliminated.*

It is puzzling why Congressional leaders would target an industry that is so fruitful and productive. The medical technology industry is a leading exporter and major driver of the U.S. economy. The medical device industry creates nearly two million high-wage jobs nationwide with wages approaching 40% higher than the average national wage. Approximately 1.5 million additional jobs are created in manufacturing, suppliers, and service providers. In California alone, the industry employs over 80,000 individuals, with $5.3 billion in payroll and $26.3 billion in revenue. This results in almost 210,000 additional jobs, driving an additional $6.9 billion in payroll and $31.6 billion in revenue.

The tax penalizes an industry that is innovative and that achieves the main goals of healthcare reform—lowering cost while improving patient care. The tax should be repealed and I want to commend Rep. Bilbray for his leadership in introducing H.R. 734 to repeal the tax.

Conclusion:

I've been most fortunate in my life to have learned from many great innovators: John Wooden, Jerry Garcia, Larry Bird, Bob Dylan, Tchaikovsky, Maurice Lucas, Dr. Steve Garfin, NuVasive and my current interactions with inspirational innovators like Duane Roth at CONNECT and the founders of all the new companies that are trying to make it with the help of San Diego Sport Innovators.

Twenty five years ago, the City of San Diego, UC San Diego and the private sector set out to stimulate the commercialization of science and technology discoveries from the local research institutions. They created CONNECT with the mission of commercializing research discoveries through education, mentoring and access to capital. In 2008 CONNECT board member Marco Thompson created CONNECT Sport Innovators as he recognized that the sports industry, which thrives in Southern California, did not have a trade organization to create this 'culture of collaboration' to connect the people, the research and the ideas to the capital, mentorship, leadership and the programs needed to succeed.

In 2010 CONNECT SI rebranded as San Diego Sport Innovators and I have been able to help the organization grow but only because, as a patient, I am the beneficiary of medical innovation.

As a patient, we see risk and benefit a bit different than regulators sitting in historic buildings in Washington D.C. When I was face down on the floor with extreme pain coursing through my nerves, I desperately needed the treatments that NuVasive was creating, in conjunction with the skill and precision of a master surgeon like Dr. Garfin.

What the regulatory system needs is a balanced approach to create the incentives needed for investment in innovation coupled with a predictable and prompt regulatory process that still provides reasonably safe and effective medical devices and drugs. I worry that the regulatory process is so focused on perfection, which does not exist, that it thwarts important creations from reaching the marketplace and saving other people that are on that bridge like I was.

Coach Wooden also tried to teach us a most applicable lesson here: "Don't let what you cannot do interfere with what you can do".

Maybe you can't solve all the problems, but that shouldn't dissuade you from doing what you can to move the medical innovation ecosystem toward more discovery, more reward, and more jobs, which will result in more patients living a healthy life.

Here's to a full and pain free life, with lots of jobs for everyone.

Thank you. And good luck.

Bill Walton

Mr. BURGESS. Well, I certainly want to thank our witness for the compelling testimony.

I do want to remind panelists, we do typically try to keep our opening statements to 5 minutes, and your full statement can be inserted into the record.

So we will ask to try to stick to the 5-minute timeline and give plenty of time for questions. But your full statement will be part of the record.

Mr. WALTON. I believe, Congressman, that mine came in at 4:52.

Mr. BURGESS. That was my time.

[Laughter.]

Mr. BILBRAY. Mr. Walton, whatever you say the time was.

[Laughter.]

Mr. BURGESS. Yes, I was not going to blow the whistle. I promise.

[Laughter.]

I do ask unanimous consent that the testimony of the chairman of the Oversight and Government Reform Committee, Darrell Issa, be submitted as part of the record. I also want to include Mr. Bilbray's statement as part of the record as well.

Without objection, so ordered.

[The information follows:]

STATEMENT FOR THE HOUSE ENERGY AND COMMERCE COMMITTEE, SUBCOMMITTEE ON HEALTH
SAN DIEGO CALIFORNIA FIELD HEARING
SEPTEMBER 26, 2011
STATEMENT OF DARRELL ISSA

Thank you for convening this important hearing on the U.S. Food and Drug Administration and their implementation of the laws and regulations impacting the health of all Americans and job creation in California.

The California life sciences industry provides a vital lifeline for the economy. The numbers are impressive. Over 2,224 companies infuse more than $114 billion dollars into this state's economy alone, giving 268,000 individuals a job with an average salary of $72,000. At a time of economic unrest, a lingering recession and a pervasively stagnant unemployment rate hovering close to 10%, stabilizing this industry is critically important.

But the importance of the life science industry is not just about money and jobs. It's about improving public health, human medicine and the quality of life for patients. It's about saving lives.

This is also the mission of the FDA. As their website states, the FDA is "responsible for protecting the public health by assuring the safety, efficacy and security" of a wide-array of drug, device and food products, as well as veterinary and cosmetic products, even infant formula and dietary supplements, as well as "advancing public health by helping to speed ***innovations*** that make medicines more effective, safer and more affordable." The scope of FDA's role in our everyday lives is immense. To this day, one out of every four dollars spent by Americans is for a product regulated by the FDA.

However, it is unclear how the FDA is balancing the dual focus of ensuring safety and advancing innovations. Despite numerous hearings, GAO reports, and FDA's own internal investigations, the ability of the FDA to adapt to the modern day innovations of drugs and medical devices are limited. The failures within the FDA to create certainty in the medical device approval process have led to industry lack of confidence. As a result current American jobs are at risk. Life sciences companies seek a predictable regulatory process by relocating to other countries.

We must not let our American companies become less globally competitive because of the FDA's inability to modernize to meet the changing nature of modern life sciences innovations. The life science industry needs certainty such as a case study or a framework for medical device approval – something the FDA's own Working Group on medical devices recommended but that CDRH leadership did not accept.

This year Congress has been working on the medical device and drug device user fee reauthorizations. While it is pre-mature to guarantee any changes in the PDUFA or MDUFA laws, what is clear is that the existing system is not working. The user fee system was set in place to help support the FDA in making drug and medical device approvals faster … not slower.

The importance of ensuring the safety and efficacy of life science products cannot be understated. It not only means the possible support for or loss of American jobs, it means to possible improvement to American health.

I look forward to continuing to conduct oversight of the FDA and working with my colleagues to hold the FDA accountable for its inaction to reform.

Thank you.

Mr. BURGESS. Dr. Gollaher, you are recognized for 5 minutes.

STATEMENT OF DAVID L. GOLLAHER

Mr. GOLLAHER. Thank you. Vice Chairman Burgess, Congressman Bilbray, welcome to San Diego. My name is David Gollaher, and I am president and CEO of CHI, the California Healthcare Institute. And I appreciate the opportunity to speak today regarding the current regulatory environment for medicines and medical devices, and its impact on biomedical innovation.

CHI is a public policy and advocacy organization representing California's innovative biomedical research and development sector. Our membership includes the State's leading research universities and private biomedical research institutes, along with venture capital firms and biotechnology, pharma, device and diagnostic companies.

All told, California is home to 2,200 biomedical companies employing some 270,000 people, making our industry one of the leading high-tech employers in the State.

This morning I would like to address two things. First, I would like to provide an overview of recent trends industry has encountered that the FDA. And, second, I would like to briefly cover one of the problems in FDA performance that CHI believes should be a focus of efforts to get things back on track.

History shows that a strong science-based FDA, and a well-articulated, predictable, and consistent regulatory process, these things are essential to bio, pharma, and medical technology investment, innovation, and patient care.

Recently, however, the number-one policy issue raised by our biotech and medical device companies has been frustration with regulatory processes and communications at the FDA that have become increasingly uncertain, unpredictable, and inefficient.

There is a widespread sense in our industry that things are getting worse at the FDA. And this is confirmed by data reflecting a recent slowdown in product review times and approvals documented in a recent study that CHI did with Boston Consulting Group, called "Competitiveness in Regulation: The FDA and the Future of America's Biomedical Industry." This was published in February.

Our report shows a few things. Particularly comparing today to the 2003–2007 timeframe, drugs and biologics review times have increased by 28 percent, 510(k) device clearances have slowed by 43 percent, and PMA—premarket approval—device approval times have lengthened by 75 percent. That is comparing today to the 2003–2007 timeline.

Now, no single factor explains this decline, but the most important contributor to recent trends appears to be a shift in FDA culture. Faced with accusations that it was too lax and failed to protect the public from safety problems with devices and drugs, the FDA has shifted emphasis in product reviews from the benefits of new products to an increasingly weight on their possible risks.

And from the perspective of an FDA reviewer, it is easy to understand. After all, an individual reviewer has little to gain by approving a product, much to lose by approving a product that has a problem in the future.

In our view, reforming the FDA is about strengthening the agency. CHI and our membership support is strong, appropriately resourced on science-based FDA, and we support strong, science-based safety and efficacy standards. But we do not believe that slower processes mean safer or better. We can have both high safety and effectiveness standards and efficient, predictable, consistent, and transparent processes to get new medicines and technologies to patients who need them.

With this in mind, I would like to turn briefly to one of the main issues we believe needs to be addressed to get the FDA back on track. My written statement covers others, and CHI looks forward to working with Congress and your committee and other stakeholders to find and act on the best solutions. But given the time this morning, I would like to focus on a single issue.

Many of our members point out that a major problem with the FDA is in the period shortly before or shortly after product submission, when discussions and negotiations over the types and amount of clinical data the agency wants to see in its submission are growing lengthier and more difficult, and are seen as less predictable, less transparent, and sometimes unreasonable.

Now certainly, as science progresses, the information the agency considered sufficient yesterday may no longer be adequate, but what is important is that the agency processes promote early upfront communications that clearly convey not only what the agency expects but why they expect it.

Clarity about what is necessary to approve a product is key. Innovators need to know what the agency says it wants and needs is really what it wants and needs, and that requirements won't change midstream.

Let me skip ahead, in the interest of time.

What we have seen in particular, and this is an important focal point for medical devices, is that when the agency issues guidance documents, which change the standards for product approval, and given the agency's current thinking on a given topic, a preliminary analysis of our work has shown that for in-process devices, when the agency issues a new guidance document, the process takes 60 percent longer to approve than average. This is preliminary data, but we suggest that it shows a real opportunity for improvement at the agency in the guidance document process.

I think in the interest of time that I am going to stop there, and I thank you for enabling our testimony.

[The prepared statement of Mr. Gollaher follows:]

C · H · I
CALIFORNIA HEALTHCARE INSTITUTE

Prepared Statement

David L. Gollaher, Ph.D.
President and CEO
California Healthcare Institute (CHI)
La Jolla, CA

U.S. House of Representatives
Committee on Energy and Commerce
Subcommittee on Health

Impact of Medical Device and Drug Regulation on Innovation, Jobs and Patients:
A Local Perspective

September 26, 2011

Good morning and welcome to San Diego. My name is David Gollaher and I serve as President and CEO of CHI, the California Healthcare Institute.

I appreciate the opportunity to speak to you today regarding the current regulatory environment for medicines and medical devices, and its impact on biomedical innovation.

CHI is the statewide public policy organization representing California's innovative biomedical research and development sector. Our membership includes the state's leading research universities and private biomedical research institutes, venture capital firms and biotechnology, pharmaceutical, device and diagnostics companies.

Our mission is to identify and advocate for federal and state policies that promote biomedical research, investment and innovation.

California's biomedical industry is responsible for breakthrough treatments, therapies and technologies that are improving and extending the lives of millions in the United States and around the world. Our industry is also a key component of our state and national economy. As reported in our CHI/PricewaterhouseCoopers/BayBio 2011 California Biomedical Industry Report (hereafter referred to as the "California Biomedical Industry Report"), California is home to over 2,200 biomedical companies, employing 268,000 people, making it one of the top high-tech employers in the state.[1] San Diego County accounts for over 24,000 of those jobs.[2] The sector is responsible for over $114 billion in annual revenues, $15.4 billion in exports and $19.4 billion in wages and salaries.[3] Last year, California's biomedical innovators also attracted $3.2 billion in National Institutes of Health (NIH) research funding and $2.6 billion in venture capital (VC) investment.[4]

Over the past generation, California has developed a remarkably rich and diverse ecosystem that has fostered the growth of vibrant biopharmaceutical and medical technology industries. This ecosystem is shaped and influenced by many factors that

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[1] California Healthcare Institute/PricewatehouseCoopers/BayBio "2011 California Biomedical Industry Report," p. 4. Available at http://www.chi.org/uploadedFiles/2011%20CA%20Biomed%20Industry%20Report_FINAL.pdf.
[2] Id. p. 8.
[3] Id. p. 4.
[4] Id.

can bolster or weaken it. At the federal level, these factors include policies set by Congress and government agencies in areas such as science funding, intellectual property, tax policy, Medicare coverage and payment policy, and regulation by the U.S. Food and Drug Administration (FDA).

This morning I would like to focus on the FDA. First, I would like to provide an overview of the recent trends industry has encountered at the Agency. Second, I will briefly address some of the issues surrounding FDA performance and how it can be improved.

Overview of Recent Trends

History shows that a strong, science-based FDA and well-articulated, predictable and consistent regulatory process are essential to biopharmaceutical and medical technology investment, innovation and patient care. Until recently, FDA policies and organizational structure have served as models for regulators around the globe. Indeed, the technical strength of the Agency and the clarity of its regulatory processes helped the United States become the global leader in medical device and biotechnology innovation.

Recently, however, in conversations across our membership, the number one policy issue raised – by small and large companies alike – has been the product review and approval process at the FDA. More specifically, the issue raised has been concern

and frustration over regulatory processes and communications that have become increasingly uncertain, unpredictable and inefficient.

When asked whether the current FDA regulatory approval process has slowed the growth of their companies, 74 percent of respondents to the 2011 California Biomedical Industry Report CEO survey reported that it had.[5] At the same time, 69 percent of the respondents disagreed with the proposition that the U.S. FDA regulatory approval process is the best in the world.[6]

These views reflect the recent slowdown in product review times and approvals documented in our recent CHI and Boston Consulting Group (BCG) report, "Competitiveness and Regulation: The FDA and the Future of America's Biomedical Industry"[7] showing that, compared to the 2003-2007 period:

- Drug and biologics review times have increased by 28 percent
- 510(k) device clearances have slowed by 43 percent
- PMA device approval times have lengthened by 75 percent

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[5] Ibid. p. 49.
[6] Ibid.
[7] California Healthcare Institute and The Boston Consulting Group, "Competitiveness and Regulation: The FDA and the Future of America's Biomedical Industry," available at http://www.chi.org/uploadedFiles/Industry_at_a_glance/Competitiveness_and_Regulation_The_Future_of_America's_Biomedical_Industry.pdf

No single factor explains this decline. Clearly, part of the problem lies beyond the direct control of the FDA and its leadership. In recent years, for example, Congress has enlarged the Agency's scope into new fields (e.g., tobacco) and added to its responsibilities and authority. Yet federal appropriations have largely failed to keep up with new mandates, forcing greater reliance on industry-funded user fees. Similarly, expanded and tightened responsibilities under the FDA Amendments Act of 2007 (FDAAA), such as intensified conflict of interest rules on advisory committees, have constrained the Agency's capacity.

Perhaps the most important factor in the Agency's recent history, though, has been a change in its culture. Faced with accusations from the press, consumer groups, and some in Congress that its reviews were too lax and failed to protect the public from safety problems with drugs and devices, the FDA has shifted emphasis in product reviews from the benefits of new products to an increasing weight on their possible risks. Increasingly companies have encountered an attitude of risk aversion at the FDA that is expressed in demands for more and more data and larger clinical studies. As a result, drug and device development costs have skyrocketed. Yet from the perspective of an FDA reviewer, this risk aversion is understandable. After all, an individual reviewer has nothing to gain by approving a product, but much to lose by approving a product that has a problem in the future.

To address and reverse this, CHI believes it is critical that Congress, the FDA, industry, patient groups and other stakeholders come together with the will and

ideas to restore Agency performance – to rejuvenate, support and sustain a strong, science-based FDA and efficient, consistent and predictable review process.

In our view, reforming the FDA is about strengthening the Agency. CHI and our membership support a strong, appropriately resourced and science-based FDA. And we support strong, science-based safety and efficacy standards. But we do not believe that slower processes mean safer or better. We can have both: high safety and effectiveness standards ***and*** efficient, predictable, consistent and transparent processes to get new medicines and technologies to patients who need them as quickly as possible.

With this in mind, I would like to turn briefly to an overview of some of the issues we believe can be addressed to get the FDA and its processes back on track. Some of these issues have been acknowledged by the Agency itself, some are incorporated in the recent PDUFA V agreement, and some we understand are being considered by Congress, including this Subcommittee. Some are at the core of CHI's ongoing data analysis work with BCG.

This is, of course, not an exhaustive listing, and CHI looks forward to working with this Committee, the Congress, the Agency and other stakeholders to find and act on the best solutions.

Getting Agency Processes Back on Track

CHI believes that today's worsening regulatory environment at the FDA has been shaped by many factors and forces. Both the Agency and industry have contributed to the problem. As has Congress. As has a popular belief, fueled by some in the media and others, that any adverse event associated with a drug or device must mean that someone, somewhere did something wrong. But the truth is, virtually all drugs and devices carry some level of risk and, because patients vary widely in their responses to different treatments, it is impossible to know precisely in advance what these risks are.

Benefit-Risk

CHI believes that a serious problem for device and drug innovation alike is that there is no shared understanding of the benefit-risk calculus. Most medical advances carry some risks. And a basic principle of medicine is that the risk of any intervention – a procedure, a drug, a device – should be commensurate with the seriousness of the patient's disorder. Accordingly, for example, patients with advanced coronary artery disease are typically willing to accept risks for new minimally-invasive procedures and technologies that have a chance to not only treat the condition but result in faster recovery times and shorter hospital stays. What has happened within the FDA, and likely influenced by internal as well as external forces, is that more and more attention has been focused on the potential direct risks of new medicines and technologies without sufficient appreciation of potential benefits. Addressing the benefit-risk equation should be a top priority.

That said, we do want to acknowledge provisions in the recent PDUFA V agreement that will enhance benefit-risk assessment processes and include a focus on patient-centered drug development. Similarly, we want to acknowledge the Agency's Center for Devices and Radiological Health (CDRH) and its recent release of a draft guidance document addressing "Factors to Consider when Making Benefit-Risk Determinations in Medical Device Premarket Review." Included in the draft guidance is a worksheet outlining the factors presented in the guidance document and which would be used by reviewers in making benefit-risk determinations.

Advisory Committee Conflict of Interest Rules

As mentioned above, intensified conflict of interest rules enacted as part of the FDA Amendments Act of 2007 (FDAAA), have made it increasingly difficult fprim any experienced medical experts to serve on Agency advisory committees. Members of this Committee and the FDA Commissioner herself have spoken to the importance of addressing this issue. And CHI supports a solution that acknowledges the need for conflict of interest rules but better rationalize the approach (through improved transparency processes, for example) to ensure that advisory committees are comprised of the most qualified, objective and experienced experts in the relevant field.

Agency-Industry/Agency-Sponsor Communications

Data in CHI's "Competitiveness and Regulation" report focused most on approval times, illustrating slowdowns across both drugs and devices, including as compared to approval times in Europe. However, a focus on approval times only tells part of the story. In fact, many have suggested that getting a product submitted and through the approval process might be the easy part. Many of our members have stated that a growing and substantial problem is in the period prior to or shortly after product submission when discussions and negotiations over the types and amount of data the Agency wants to see in a submission are growing lengthier and more difficult and seen as less certain, predictable, transparent and even rational.

Certainly, as science progresses, the information the Agency considered sufficient yesterday may no longer be adequate. But what is most important is that Agency processes promote early, up-front communications that clearly convey not only what the Agency expects, but why they expect it. Similarly, those communications and communications processes should be viewed with a sense of certainty – that what the Agency says it wants and needs is what it really wants and needs, and that requirements will not change or expand mid-stream.

This – Agency-industry and Agency-sponsor communications – is one of the key areas CHI is focusing on in more detail as part of our ongoing data analysis and work. Specifically, we will be looking for evidence to suggest policies and mechanisms to improve the timing, substance, quality and certainty of

communications from and with the Agency. What works and where are there problems? Given the relative lack of public data to analyze and measure in this space, one particular issue we are looking at is the guidance document process.

It stands to reason that guidance documents, which represent the Agency's current thinking on a given topic, and the process by which the Agency decides to develop, update, and issue them could have an impact on the product development, submission and review process. Indeed, a preliminary analysis as part of our work with BCG shows some significant correlation between the issuance of guidance documents and approval times: In-process PMA device submissions impacted by subsequently-issued guidance documents take some 60 percent longer to approve than those not so impacted. Again, this is very preliminary data to be further analyzed, but we believe it suggests room for improvement to Agency guidance document practices, such as suggested by Chairman Upton in a recent OpEd piece.[8]

Here, too, the Agency itself has seemingly acknowledged the need for improved communications mechanisms. For example, earlier this summer, CDRH released a draft Standard Operating Procedure (SOP) document for its proposed new "Notice to Industry" (NtI) letters. As the SOP document states, the NtI letters will serve to "clarify and more quickly inform stakeholders when CDRH has changed its expectations relating to, or otherwise has new scientific information that could

[8] Rep. Fred Upton. "A defibrillator for American jobs." Sept. 8, 2011. Available at http://dailycaller.com/2011/09/08/a-defibrillator-for-american-jobs/

affect, data submitted as part of [device submission] that needs to be disseminated in a timely manner." While CHI has questions, concerns and reservations about the scope of the NtI letter mechanism and believes there needs to be important assurances and clarifications that they will be used appropriately in limited circumstances and with sufficient process transparency so as to not preclude stakeholder review and comment opportunities, we do believe that they signal an important acknowledgement by the FDA of the need to improve Agency communications practices and management.

Conclusion

The items above are by no means an exhaustive list of the issues or policies that Congress and the FDA should look at to improve Agency regulatory processes. There are certainly others, including sufficient and appropriate Agency funding by Congress. And there are many opportunities for performance improvements at the Agency that do not require legislation or congressional intervention. After all, the most significant factors in recent trends at the Agency are managerial or cultural in nature. Encouraging innovation by defining appropriate levels of risk for drugs and devices should be a central goal of FDA regulation, and a goal that Congress should support.

I would like to conclude with a quote from our CHI/BCG "Competitiveness and Regulation" report that summarizes where CHI hopes to head from here:

> Industry is committed to strengthening its partnership with Congress and the Agency. Positive policy and operational improvements at the FDA, along with constructive legislation, will encourage biopharmaceutical and device innovation. Indeed, a strong, science-based Agency and an efficient, predictable and transparent regulatory process are essential elements of the biomedical innovation ecosystem. And working together, Congress, the Agency, industry and other stakeholders can maintain the high standards of safety and effectiveness that physicians, patients and their families expect while also strengthening the biomedical sector's ability to attract the investment essential to secure U.S. global leadership in [the] life sciences.[9]

Thank you again for the opportunity to testify and I would be pleased to answer any questions you might have.

[9] "Competitiveness and Regulation." p. iii.

Mr. BURGESS. Great. We will probably get to some of these things during the question-and-answer time as well.

Dr. Stevenson, you are recognized for 5 minutes.

STATEMENT OF SHARON STEVENSON

Ms. STEVENSON. Thank you, Representative Bilbray, Dr. Burgess. I am Sharon Stevenson, cofounder and managing director of Okapi Venture Capital, which is based in Laguna Beach. It is one of a handful of venture firms located in Southern California.

Today I am testifying on the behalf of the National Venture Capital Association, the national trade association for the venture capital industry. Thank you for the opportunity.

For the past several decades, venture capital firms such as Okapi have served as the primary source of risk capital, playing a central role in prospering the development of medical technologies and therapies to treat cancer, cardiovascular disease, diabetes, and a plethora of other conditions.

This important relationship between capital and emerging companies has enabled our Nation's biotechnology and medical device industries to assume their long-held positions as world leaders in medical innovation. The benefits of robust biotechnology and medical device sectors are abundantly clear: better care for patients, cures for diseases and disabilities, cost-effective treatments that can help lower health-care costs, and high-paying jobs with tremendous growth potential.

Although researchers continue to identify promising therapies at a great pace, fewer of these discoveries are being developed and brought to market, in part due to a growing reluctance among the venture capital industry to provide necessary financial backing. Venture capital and emerging technologies exist in a fragile ecosystem. A number of factors, including the current economic environment and lack of an IPO market, have impacted general availability of venture capital. However, the life sciences sector is being doubly hit.

The uncertainty of the FDA processes has undermined the ability of life science venture capital firms to achieve favorable return on their investments and, therefore, to raise funds to support the next generation of innovative biotechnology and medical device companies.

Increasingly, limited partners, the endowments, pension funds, and the institutions that provide much of the capital for venture funds to invest, are telling us that they are reducing or even eliminating the share of investment that they allocate to life sciences venture capital. Some well-established life sciences venture firms have reduced or ceased operations; others are no longer investing in early-stage life sciences companies.

Some firms have changed their investment portfolios, increasing their support for information technology and social networking companies, or have begun to invest in emerging markets, sending private investment dollars previously dedicated to U.S. companies to startups overseas.

This decline in U.S. investment has enormous implications for patients and their care providers, who in the future will have diminished access to breakthrough treatments. It has and will con-

tinue to undermine a key engine of our economy that has long provided well-paying jobs throughout the Nation.

Obviously, there are many factors at play here, but the uncertain regulatory environment at the FDA has been a very significant contributor. Since the late '80s, the agency held firmly to a balanced risk-benefit approach, with a stable predictable regulatory pathway. As a result, a generation of important therapies came to market, and the Nation gained prominence as a leader in medical innovation.

But the agency dynamic has changed dramatically in the wake of a series of high-profile therapies that resulted in significant safety issues. The pendulum has shifted with the FDA culture now marked by extreme caution and risk aversion.

To be clear, protecting patients must be a fundamental element of the FDA's activities, and I am in no way suggesting it should be otherwise. Right now, though, the FDA's approach emphasizes risk over benefit.

Simply stated, the careful balance that historically helped to fuel the development of any number of breakthrough innovations and life-saving treatment no longer exist. Rather, the approval process is now unpredictable and lacking in transparency, creating greater risk for researchers, emerging companies, and investors.

Venture capital is an industry that is all about taking risk. That is what we do. But it is important to distinguish between the two types of risk we consider when making investment: manageable and unmanageable.

When the regulatory pathway is sensible, clear, and consistent, the regulatory risk is manageable, even if the associated activities are expensive and time-consuming. However, in the current regulatory environment, the FDA may require significant changes in the regulatory pathway midstream, or even after clinical trial activities have been completed.

When the rules are changed in the middle of the process, more capital is inevitably needed at the worst possible time for the company to be raising it.

The bottom line is that the current FDA regulatory approach has led to a painful, if not intolerable, increase in unmanageable risk. As a result, many of my colleagues have reached a tipping point and have significantly or completely pulled back their investments in emerging life sciences companies, or are only investing in companies where a clear route to commercialization exists outside of the U.S. This is not good.

The NVCA and its member companies want to work with Congress and the FDA to reestablish a thoughtful risk-benefit equation and respectfully offer the following recommendations for the subcommittee's consideration.

One is to support allowances for variation in risk-benefit assessments to ensure patient-centric drug and medical device development and approval.

Second is to expand the accelerated approval pathway into a progressive approval system for drugs, diagnostics, and medical devices.

Lastly, ensure conflict of interest policies are not hindering patient access to new treatments. The goal is not to eliminate con-

flicts of interest, but to illuminate them, so that they may be appropriately managed. We need the most knowledgeable people around the table for advisory panel discussions.

Thank you very much.

[The prepared statement of Ms. Stevenson follows:]

Testimony of
Sharon Stevenson
Managing Director
Okapi Venture Capital, LLC

on the

Impact of Medical Device and Drug Regulation on Innovation, Jobs and Patients: A Local Perspective

before the

U.S. House of Representatives
Committee on Energy & Commerce
Subcommittee on Health

Field Hearing

September 26, 2011

Scripps Seaside Forum

La Jolla, California

Introduction and Testimony Overview

Chairman Upton, Representative Burgess and Representative Bilbray, I am Sharon Stevenson, Co-Founder and Managing Director of Okapi Venture Capital, based in Laguna Beach. Founded in 2006, Okapi is as much a start-up as the companies it funds. One of a handful of Southern California based venture firms, Okapi provides capital and operating support to entrepreneurs and start-up companies. We take pride in partnering with exceptionally talented entrepreneurs and operational executives to develop their emerging businesses. We currently manage Okapi Ventures I, LP – a venture capital fund chartered to invest in seed and early-stage information technology and life science companies along Southern California's Tech Coast with an emphasis on Orange County startups. Since its inception, Okapi has invested over $11 million, fully half of its investable capital, in five health care companies, including biotechnology and medical device companies. Okapi has invested its remaining capital in four high technology companies.

Over the course of my 11-year venture capital career, I have worked side-by-side with entrepreneurs to create and finance many start-ups, including Volcano Therapeutics; Helixis, which was acquired by Ilumina, a public company based in San Diego; and OrthAlign, Obalon Therapeutics, PathCentral, and WellTok, privately held companies on whose boards I currently serve. Okapi is a small fund. Our model not only is to invest our capital, but also to work closely with our companies to prepare them for growth and investment by larger somewhat later stage funds.

In addition to representing Okapi and its portfolio companies, I also am testifying on behalf of the National Venture Capital Association (NVCA) based in Arlington, Virginia. The NVCA

represents the interests of more than 350 U.S. venture capital firms including Okapi. These firms comprise more than 90 percent of the venture industry's capital under management. On behalf of the venture industry and entrepreneurs, it is my privilege to share our perspective on the impact that the current Food and Drug Administration's (FDA) regulatory approach has on innovation in the U.S. biotechnology and medical device industries, and more importantly, on patient access to novel medical therapies. And I can think of no better place to assess the impact of the current regulatory framework on local economies than California's Tech Coast – home to more than 500,000 small businesses and more than a dozen world-class universities, research institutions, and medical centers, many of which are directly involved in developing novel medical therapies. Nearly 270,000 Californians are employed in the biomedical industry.[1] In the Tech-Coast, there are nearly 45,000 employees in the industry.[2]

I will begin with a brief overview of venture capital's historical role in the biotechnology and medical device sectors. I will then turn to the challenges that both venture capital firms and seed and early-stage life sciences health care start-ups face in the current regulatory environment, and the implications for patients and the economy. I believe that these challenges can be successfully addressed and will close with a series of recommendations developed by NVCA and its members for your consideration.

Venture Capital: Helping Translate Promising Research into Breakthrough Treatments, Promoting Jobs and Economic Growth

It is widely known that for medical therapies and technologies it can take years, if not decades, from initial discovery to FDA approval. In addition to time, it requires money. It is not

[1] CHI California Biomedical Industry Report 2011
[2] See www.sce.com/economicdevelpment

unusual for a company to require hundreds of millions of dollars to support the development of innovative therapies, which is where venture capital comes into the picture. For the past several decades, venture capital firms, such as Okapi, have served as the primary source of risk capital, playing a central role in fostering the development of medical technologies and therapies to treat cancer, cardiovascular disease, diabetes, multiple sclerosis, HIV and orphan diseases, among many others. Most, if not all, of our nation's cutting-edge biotechnology and medical device companies got off the ground with venture capital support. That support is not uniquely financial: early stage venture capitalists provide mentoring, experience, strategic guidance, and access to networks that facilitate the growth and development of young companies and management teams.

This important relationship between capital and emerging companies has enabled our nation's biotechnology and medical device industries to assume their long-held positions as world leaders in medical innovation. Other nations seeking to grow these sectors of their economies envy our model rooted in venture capital support, and for good reason. In 2010, venture capital-backed and developed U.S. biotechnology companies employed more than 1.7 million individuals. Over the last five years, the investments of Okapi alone, a very small, young venture firm, have created over 150 new, high-quality jobs in southern California. Small businesses with fewer than 100 employees comprise the majority of these companies and are making significant contributions to our nation's economy, particularly in these challenging times. These companies are central to the U.S. medical innovation ecosystem, but they are small businesses whose ability to survive and innovate can be affected by seemingly minor regulatory factors that increase the cost, time and risk required to develop new products.

Successful Model of Venture Capital Support for Medical Innovation is Struggling

Although researchers continue to identify promising therapies at a great pace, fewer of these discoveries are being developed and brought to market, in part due to a growing reticence among the venture capital industry to provide necessary financial backing. Today, developing a new drug takes on average $800 million to $1 billion and 10 years to 15 years. [3,4,5,6] Bringing a low to moderate 510(k) product from initial concept to FDA clearance requires just over $30 million, while higher risk premarket approval (PMA) products can take $95 million.[3,4,5,6] These development costs for both drugs and medical devices have and are expected to continue to grow at an unprecedented rate. These development costs also tend to skew U.S. investments toward high cost drugs and devices, just to make the economics of the investment work. Emerging-market countries are practicing a fundamentally different form of innovation that emphasizes "small, faster and more affordable devices" that reduce healthcare costs system-wide.[3] As there is no indication that these devices are less safe,[4] it seems to me that the U.S. could benefit from this approach.

The current situation has undermined the ability of venture capital firms to achieve a favorable return on their investments and to raise funds to support the next generation of innovative biotechnology and medical device companies. Increasingly, limited partners – the endowments, pension funds, institutions, and in the case of small funds like ours, family offices and high net worth individuals that provide much of the capital for venture funds to invest – are telling us that they are reducing or even eliminating the share of investment that they allocate to life sciences venture capital. Some well-established venture firms that solely provide capital to

[3] See http://www.pwc.com/us/en/health-industries/health-research-institute/innovation-scorecard/index.jhtml
[4] See http://www.advamed.org/NR/rdonlyres/061A4AC8-D6A3-4690-826B672214A0A623/0/REPORTBCGEuropeanUSSafetyFINAL.pdf

life sciences companies have reduced or ceased operations, others are no longer investing in early stage life sciences companies because of the growing regulatory challenges. Some firms have changed their investment portfolios, increasing their support for information technology and social networking companies. We are seeing companies move away from supporting development of critical therapies in areas of significant unmet need, such as diabetes and obesity, in favor of less risky prospects. Still others have begun to ramp up their strategies in emerging markets, sending private investment dollars previously dedicated to U.S. companies to start ups overseas, or have made plans to commercialize their products outside of the U.S., no longer just to demonstrate commercial feasibility but now as genuine ongoing businesses. To illustrate the depth of what I consider to be an unfolding crisis, I offer the following statistics:

- The total amount of capital raised by venture capital funds decreased by 25 percent in 2010 compared to 2009, representing the third consecutive year of decline.[5]

- Between 2009 and 2010, the share of venture capital invested in biotechnology declined from 18 percent to 12 percent, the lowest level since 2001.[9] Between 2008 and 2010, venture investment in U.S. life sciences companies declined by $2 billion dollars.[6]

- Between January and June 2011, venture capital investments in seed and first round medical device start-ups declined nearly 50 percent.[7]

This decline in investment has enormous implications for patients and their providers, who in the future will have diminished access to breakthrough treatments. It has and will continue to

[5] Ernst & Young. Beyond Borders. Global Biotechnology Report. 2011.
[6] PricewaterhouseCoopers/National Venture Capital Association Money Tree Report: Thompson Reuters.
[7] BioAdvance, MedTRACK Venture Finance Database

undermine a key engine of our economy that has long provided well-paying jobs throughout the nation. Other nations have recognized and seized the opportunity this situation presents to energize their own biotechnology and medical device sectors by encouraging companies to bring their research and development enterprises abroad. In 2007, the European Union (EU) and European Federation of Pharmaceutical Industries and Associations established a $2 billion Innovative Medicines Fund. In 2010, Europe saw a 29 percent increase in capital invested in biotechnology companies, compared to a 3.2 percent decline in the U.S.[9] As part of a five-year national economic plan, China identified biotechnology as one of seven emerging markets and committed $1.5 billion for investments in the sector.[9] India has followed suit announcing last year its intent to create a $2.2 billion venture fund to support drug discovery and research infrastructure development.[9]

The flagging venture capital support in the U.S. along with the significant investments in Europe and emerging nations has led me to believe that without serious and timely intervention, our nation's position as the global leader in medical innovation will be severely threatened in the coming years.

Uncertain Regulatory Environment Key Contributor to Escalating Costs, Extended Approval Timeframes and "Unmanageable" Risk

A number of factors, including the current economic environment and lack of an IPO market, have contributed to the decline in venture capital investment in developing new therapies and technologies. But hands-down, the uncertain regulatory environment at FDA has been the most significant contributor. Over the course of my career, there has been a noticeable, significant shift in the FDA's approval approach. In the late 1980s and early 1990s, the Agency came under

attack from patient groups, who viewed the FDA as a bottleneck in getting life-saving therapies to patients. Congress worked with the Agency to develop changes that sped up the approval process, while at the same time maintaining a benefit-risk balance to ensure drug and patient safety.

For many years, the Agency held firmly to this balanced approach with a stable, predictable regulatory pathway. As a result, a new generation of important therapies came to market and the nation gained prominence as a leader in medical innovation. But the Agency dynamic has changed dramatically in the wake of a series of high-profile therapies that resulted in significant safety issues. The pendulum has shifted, with the FDA culture now marked by extreme caution and risk-aversion. To be clear, protecting patients must be a fundamental element of the FDA's activities, and in no way am I suggesting that it should be otherwise. However, the FDA also has a responsibility to ensure the timely development of life-saving therapies. Right now, though, the FDA's approach emphasizes risk over benefit. Simply stated, the careful balance that historically helped to fuel the development of any number of breakthrough innovations and life-saving treatments no longer exists. Rather, the approval process is now unpredictable and lacking in transparency, creating greater risk for researchers and investors.

Let me elaborate on this point. Venture capital is an industry that is about taking risks – it is what we do, but it is important to distinguish between the types of risk we consider when making an investment. We encounter two categories of risk: manageable and unmanageable. I would consider the cost of enrolling each patient into a clinical trial as manageable. We can predict those costs and the difficulties of enrollment. What we cannot predict, especially in the current regulatory environment, for example, is whether or not the FDA will require a change in the clinical trial design, such as requiring more patients or additional endpoints well into the

planning process. We also are challenged in predicting how often they might want further changes in trial design or information related to other dimensions of the approval process. Companies raise, and venture capital invests, capital to achieve specific milestones. If the rules are changed in the middle of the process, more capital is inevitably needed at the worst possible time for the company to be raising it. The bottom line is that the FDA regulatory approach has led to an intolerable uptick in unmanageable risk. As a result, many of my colleagues have reached a tipping point and have significantly or completely pulled back their investments in emerging life sciences companies or are only investing in companies where a clear route to commercialization exists outside the U.S.

Imperative for Action and Recommendations

The benefits of robust biotechnology and medical device sectors are abundantly clear: better care for patients; cures for diseases and disabilities; cost-effective treatments that can help lower health care costs; and high-paying jobs with tremendous growth potential. Unfortunately, all of these benefits are at great risk – primarily because of the current FDA regulatory framework. Safeguarding and preventing future erosion of these benefits will require the restoration of balance between the assessment of benefit and risk. The NVCA and its member companies believe there exists a path to achieve this important objective and respectfully offers the following recommendations for the Subcommittee's consideration:

1. Support Allowances for Variation in Benefit-Risk Assessments to Ensure Patient-Centric Drug and Medical Device Development and Approval

Background: In the past, the FDA has taken a population-level perspective when assessing the possible risks of a therapeutic, diagnostic or medical device relative to its possible benefits, despite the fact that disease severity can vary markedly within the population. Risk tolerance

among patients also can vary significantly with patients suffering life-threatening conditions more likely to accept greater risk. In addition, individual preferences and values also can influence levels of risk tolerance. Finally, the advent of new tools, such as personalized medicine, necessitates a benefit-risk assessment process that recognizes differences in genetics, preferences and circumstances.

The lack of transparency in the current benefit-risk assessment process makes it impossible to understand the extent to which the FDA is considering these factors. In the absence of a clear benefit-risk framework, it appears that the FDA is making important decisions on approval or application of a safety program such as Risk Evaluation and Mitigation Strategies (REMs) on an ad hoc basis with little if any input from affected stakeholders.

Recommendations:

(i) The NVCA urges development of a qualitative framework for benefit-risk assessment of new therapeutics and diagnostics that incorporates robust input from stakeholders, including patients and consumers. In evaluating risks, the FDA must consider the size of the population affected, the range of existing and available alternative treatments and the risks of living with that specific condition in the absence of the therapeutic or diagnostic under consideration.

(ii) NVCA recommends FDA's adoption of a more flexible benefit-risk paradigm that has the capacity to make adjustments to reflect clinical need and patient risk tolerance. This flexible benefit-risk paradigm should allow for differentiation in the level and amount of evidence required, degrees of efficacy to be shown and minimum proportion of a population needing to benefit. Under such a paradigm,

the FDA should be allowed to approve new drugs and devices based upon a 'weight of the evidence' standard in certain cases. This type of flexibility could be particularly crucial in assessing treatments for rare diseases for which conducting multiple clinical trials of sufficient size to achieve statistical significance may be impractical or impossible.

(iii) The NVCA implores the FDA to engage in a formal process of interaction with stakeholders involved in developing medical innovations and to explicitly consider the effect of its decision-making on medical innovation. The process should facilitate and promote innovation consistent with ensuring that all medical products are safe and effective.

2. Expand the Accelerated Approval Pathway into a Progressive Approval System for Drugs, Diagnostics and Medical Devices

Background: The current drug and medical device review process continues to drive up the cost, time and risk of drug and medical device development. This is especially challenging and unsustainable for early-stage venture capital-backed companies. Many patients suffer from illnesses where there are no treatments. They want access to promising new therapies and technologies earlier in the drug and medical development process. We need to find ways to develop and provide safe and effective drugs and medical devices to patients in a manner as timely as possible, particularly in areas of unmet need.

Recommendation: The NVCA encourages the FDA to consider new approval frameworks, such as a progressive approval system, that would provide early provider and patient access to

promising therapies and devices that offer significant advances over standard care, improve treatment for a specific subpopulation of patients and are likely to provide benefits that exceed risks. The new approval frameworks would include robust monitoring of safety and efficacy outcomes. As the Agency collects additional evidence for full approval and assesses the benefit-risk balance, the therapeutic or medical device could be restricted, withdrawn or fully approved. This approach would allow for more robust learning by patients, providers and technology developers to occur in the development process; provide patients with earlier access to innovative medicines; and allow refinement of products in an efficient, safe process.

3. Ensure Conflict-of-Interest Policies are Not Hindering Patient Access to New Treatments

Background: Conflict-of-interest policies are important tools to ensure the credibility and transparency of advisory committees that provide the FDA expert advice crucial to fulfilling its mission. The current rules, however, may preclude well-qualified experts from participating, resulting in an undermined advisory committee process, delays and meeting cancellations. This is particularly a concern with respect to committees convened to provide input on highly specialized topics, such as rare disorders.

Recommendation: The NVCA urges Congress to evaluate conflict-of-interest rules and consider amending those rules to ensure that they do not do more harm than good in helping the FDA fulfill its mission. The goal is not to eliminate conflicts-of-interest, but to illuminate them so that they may be appropriately managed. Additionally, the FDA should have the ability to recruit more widely from non-academic pools of candidates who have the expertise to evaluate medical products.

Conclusion

The long-standing and effective model of venture capital investment in medical innovation is under siege. If left unaddressed, the situation will have significant negative consequences for patients and the economy overall. The ecosystem comprised of limited partners with the willingness to entrust their capital to venture investments; knowledgeable, experienced venture capitalists with the capital to commit to early stage life sciences technologies; and management teams with the expertise to grow the companies is at risk. Okapi and NVCA appreciate the enormity of the tasks before the FDA in approving therapies and devices and the responsibility the Agency has in ensuring patient safety. As I discussed, a number of factors have contributed to the decline in venture investment in life sciences, but a key factor is the FDA's risk-averse regulatory and approval approach.

We believe, however, that steps can be taken to correct this situation and to restore a balance between benefit and risk assessment that has underpinned therapeutic advances that have changed patients' lives and contributed to our nation's dominance in medical innovation. We are grateful for the Subcommittee's interest in this issue and look forward to working with you as deliberations on this topic continue.

Mr. BURGESS. Thank you.

Dr. Mento, you are recognized for 5 minutes for your testimony.

STATEMENT OF STEVEN J. MENTO

Mr. MENTO. Thank you, Congressman Burgess, Congressman Bilbray. My name is Steven Mento, and I am cofounder, president, and chief executive officer of Conatus Pharmaceuticals.

I am here testifying on behalf of Biotechnology Industry Organization, where I serve as cochairman of its Emerging Companies Section Health Committee. It is important to note that the majority of BIO's member companies are small emerging companies like Conatus working on the arduous and capital-intensive task of developing innovative treatments and therapies.

I personally have over 25 years of experience in the biotechnology and pharmaceutical industry. Conatus is focused on the development of innovative human therapeutics to treat liver disease and cancer. Our lead development candidate, Emricasan, a novel drug being tested as a potential anti-fibrotic drug to delay the progression of hepatitis and prevent the development of cirrhosis. Our pipeline also includes a potential oral therapy to reduce tumors associated with lymphomas.

Everyone would like to see innovative treatments and therapies to treat liver disease and cancer be successfully developed. However, it is essential to understand the difficulty in accomplishing this shared goal in today's environment.

While we in the United States are currently the global leader in the development of biotechnology treatments and therapies, intense competition from China and India means this is a position we have to fight to keep. Indeed, when it comes to small venture-backed startup biotechnology companies, such as my own, our industry is facing a crisis.

In addition to the economic downturn, regulatory uncertainty, longer drug development timelines, and an increasing regulatory and congressional focus on risk instead of reward in pharmaceutical innovation, are deterring investors from investing in biotechnology, as you have just heard.

While these projects may have the highest risk, they also have the highest potential to positively impact society. Investors have always known that the science is difficult and fraught with risk, but FDA's shift in recent years to an increasingly cautious, risk-averse posture has had the unintended consequence of diverting investment in life sciences innovation toward things like Groupons and iPads.

In addition to the past potential medical breakthroughs we offer to patients, the bioscience sector accounts for over 7 million direct and related high-paying, high-quality jobs. We have a national imperative to foster the development of innovative treatments and therapies. With baby boomers now entering into the Medicare system, the costs associated with chronic care diseases will skyrocket unless we work to develop novel medicines to treat these diseases.

We as a Nation need to focus policy discussions on how to unleash the promise of biotechnologies so that the American public can realize the benefits it has to offer. A fundamental part of our ability to innovate and raise private investment is having an FDA

with the resources and mechanism required to effectively and consistently review and approve innovative products in a timely manner.

These decisions must be understood by stakeholders, industry investors, patients, and physicians, and then must be made in the context of patients and diseases being treated.

The FDA is rarely praised for approving a novel therapy, but they are often maligned if there are unforeseen adverse events that occur once a product is approved. It is imperative that policymakers understand the scientific realities of approving novel medicines.

The remainder of my testimony will focus on solutions. BIO has developed a set of policy proposals designed to encourage innovation through the creation of a 21st century FDA, which we have submitted for the record. I will highlight a few of those proposals today.

FDA has been perceived by many as the global standardbearer for regulatory review in drug and biologic applications. However, scientific and medical knowledge, techniques, and technologies are advancing at a more rapid pace today than at any other time. And FDA's capacity to access information about these advancements has not kept pace. It is essential that FDA's access to scientific and medical advice be enhanced by improving the operations of the FDA advisory committees, establishing chief medical policy officers in the immediate offices of the center directors, and providing FDA staff with additional avenues for accessing external scientific and medical expertise.

Two, patients, particularly those with illnesses where no adequate therapy exists, want to access the promising new therapies earlier in the drug development process. Expanding and improving the accelerated approval pathway into a progressive approval mechanism would help provide patients more timely access to needed therapies. This pathway would also ensure risk-benefit analysis that incorporates the safety and needs of patients in the real world.

And three, FDA's current statutory authority requires that the agency approve applications for new drugs when they have been demonstrated to be safe and effective under the intended conditions of use. The law provides that effectiveness is established where FDA is satisfied that there is substantial evidence that the new drug has the intended effect that it is purported to have.

FDA typically requires two adequate and well-controlled studies under this standard. A weight of evidence approach to data analysis, however, would allow the decisionmaker to look at all the data and information, and give appropriate consideration.

Thank you for allowing me to testify, and we look forward to working with you on developing policies for a 21st century FDA that will serve to unleash the promise of biotechnology in the United States.

[The prepared statement of Mr. Mento follows:]

TESTIMONY OF STEVEN J. MENTO, PH.D.
PRESIDENT & CEO CONATUS PHARMACEUTICALS INC.
SAN DIEGO, CA

ON BEHALF OF THE BIOTECHNOLOGY INDUSTRY ORGANIZATION (BIO)

ENERGY AND COMMERCE SUBCOMMITTEE ON HEALTH
FIELD HEARING AT SCRIPPS INSTITUTION OF OCEANOGRAPHY
LA JOLLA, CA

IMPACT OF MEDICAL DEVICE & DRUG REGULATION ON INNOVATION, JOBS, & PATIENTS: A LOCAL PERSPECTIVE

SEPTEMBER 26, 2011

Chairman Pitts, Ranking Member Pallone, and Members of the Subcommittee, my name is Steven Mento and I am the Co-Founder, President and Chief Executive Officer of Conatus Pharmaceuticals Inc. I am here testifying on behalf of the Biotechnology Industry Organization where I serve as Co-Chairman of its Emerging Companies Section Health Committee. BIO represents more than 1,100 members involved in the research and development of innovative healthcare, agricultural, industrial, and environmental technologies. The majority of BIO's member companies are small emerging companies working on the arduous and capital intensive task of developing innovative treatments and therapies. In fact, ninety percent of BIO's research and development company members have fewer than 100 employees. Additionally, 43 percent of typical biotech companies have less than one year's worth of cash on hand and 48 percent are at least three years away from having product revenue.[1]

[1] BIO Emerging Companies Section Membership Survey, 2011.

I have over 25 years of experience in the biotechnology and pharmaceutical industry. My current company, Conatus Pharmaceuticals Inc., is focused on the development of innovative human therapeutics to treat liver disease and cancer. Our lead development candidate is Emricasan is a member of a new class of drugs to modulate caspases (cell death proteases) involved in the apoptosis and inflammation pathways. Emricasan is being tested as a potential anti-fibrotic drug to delay the progression of hepatitis and prevent the development of cirrhosis. Our pipeline also includesIDN-13389, a potential oral therapy to reduce tumors associated with lymphomas as well as breast, pancreatic and colon cancers. While, I believe it is safe to assume that everyone would like to see innovative treatments and therapies to treat liver disease and cancer be successfully developed, it is important to understand the difficulty in accomplishing this shared goal in today's environment.

Developing innovative treatments and cures is a time- and capital-intensive endeavor reliant on private investment. It generally costs over $1 billion and 8-10 years to research and develop an FDA-approved drug.[2] In order to encourage innovation, we must have an FDA that is empowered and able to effectively and consistently review breakthrough treatments and therapies. There are several troubling trends that threaten to severely hamper our ability to innovate. For example, only half of the products submitted to the FDA are approved on the first submission.[3] From the average of the previous PDUFA rounds of 2003-2007 to today, drug and biologics approval times have increased 28 percent.[4] Between 1999 and 2005, the average length of clinical trials grew by 70%.[5] And despite the extraordinary advances in science and huge increases in research and development spend over the last two decades, the number of new drug approvals per year remains flat (i.e., an average of 23 NME approvals per year over the past decade).[6]

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[2] J Dimasi and H. Grabowski, "The Cost of Biopharmaceutical R&D: is Biotech Different?" Managerial and Decision Economics No 28 (2007): 469–79
[3] BIO. Biomed Tracker. 2011.
[4] CHI. BCG. 2011. "Competiveness and Regulation: The FDA and the Future of America's Biomedical Industry."
[5] Tufts Center for the Study of Drug Development. 2008. "Growing Protocol Design Complexity Stresses Investigators, Volunteers." Impact Report. 10.1.
[6] FDA. http://www.fda.gov/downloads/AboutFDA/Transparency/Basics/UCM247465.pdf

While the most recent data for 2011 show FDA on pace to approve more drugs and biologics this year than in recent years is welcome news, this does not address the core issue of how FDA is balancing the benefits versus risks of new therapies. Additionally, this is not an indicator for a consistent review process that incorporates modern science and takes into account the disease and patients being treated.

The U.S. biotechnology industry is poised to be a major driver in an innovation-driven economy. And while we are currently the global leader in the development of biotechnology treatments and therapies, intense competition from China and India means this a position we have to fight to keep. Indeed, when it comes to small, venture-backed start up biotechnology discovery companies, such as my own, our industry is facing a crisis. In addition to the economic downturn, regulatory uncertainty, longer drug development timelines, and an increasing regulatory and Congressional focus on risk instead of reward in pharmaceutical innovation are deterring investors from investing in biotechnology discovery companies. In fact, the hardest thing to raise capital for today is for early stage innovative projects. While these projects may have the highest risk they also have the highest potential to positively impact society. Investors have always known that the science is difficult and fraught with risk but FDA's shift in recent years to an increasingly cautious, risk-adverse posture towards innovative drug approvals, demands for more and more data, have had the unintended consequence of diverting investment in life sciences innovation towards things like the next Groupon or IPAD. This is a phenomenon we are already beginning to see. Between 2010 and 2011 to date, first-time fundings of life sciences ventures – a key leading indicator of the health of the innovation ecosystem – have decreased by more than 50% compared to prior years.[7]

We are in danger of losing our position as a global leader in medical innovation and our ability to keep private investment dollars and jobs in the United States as Europe, China, and India continue to develop aggressive strategies to entice companies to take their research and development enterprises abroad. In 2007, the European Union and the

[7] BioAdvance, MedTRACK Venture Finance Database

European Pharmaceutical Industry Association (EFPIA) sought to attract life sciences companies to Europe by establishing the $2 billion Innovative Medicines Initiative (IMI), which is described as "Europe's largest public-private initiative aiming to speed up the development of better and safer medicines for patients...[which] supports collaborative research projects and builds networks of industrial and academic experts in order to boost pharmaceutical innovation in Europe." A March 2011 press release indicates that the IMI has recently launched a second wave of research projects (focusing on areas including cancer, infectious disorders and electronic health), with a total of 23 current research projects and over €450 million (approximately USD $658 million at the time of publishing) committed by the European Commission and the EFPIA. In 2010, while the amount of capital invested in private U.S. biotechnology companies declined 3.2%, Europe saw a 29% increase.[8]

Additionally, last year, the Chinese government unveiled a 5-year plan for national economic and social development, and the biopharmaceutical industry was identified as one of the seven strategic emerging industries that China would target. The plan includes a $1.5 billion commitment and the establishment of new venture funds to invest in emerging start-up companies. India has similar plans to expand biopharmaceutical activities and in 2010 announced a plan to establish a $2.2 billion venture fund for supporting drug discovery and research infrastructure development projects.[9]

Our industry is offering real solutions to our most pressing health care needs: curing disease, reducing costs, increasing quality, and ensuring that people enjoy not only live longer lives, but better and more productive lives. Despite the extraordinary promise offered by biotechnology, government policies and practices are negatively impacting our ability to innovate and are thus restraining our ability to reach our full potential to provide new health solutions to patients and address our most pressing public health needs. If we do not provide FDA with the resources, incentives and clear mission to

[8] Ernst & Young. Beyond Borders. Global Biotechnology Report. 2011
[9] Ernst & Young. Beyond Borders. Global Biotechnology Report. 2011

advance innovation we run the risk of weakening the innovation pipeline due to lack of investment in early stage research for the next generation of medical breakthroughs..

In addition, to the past and potential medical breakthroughs we offer to patients, the bioscience sector accounts for over 7 million direct and related jobs.[10] Not only do we create high paying jobs for scientists, clinicians, manufacturing technicians, and support staff internally at our companies, we also create jobs and vital revenue for our universities and medical centers through the clinical trials we conduct.[11]

We are innovators. Of the 172 scientifically novel and orphan drugs approved from 1998-2007, 52% were discovered and/or developed by biotechnology companies.[12] We offer tremendous hope to patients with over 3,700 new biotherapies in development that have the potential to offer significant advances in treatments for patients suffering from cancer, diabetes, Alzheimer's, cardiovascular disease, and rare genetic disorders.

The public benefit of medical innovation is well-documented.

- Medicines can help offset overall medical costs by preventing or delaying the need for other costly services, such as emergency room visits and hospitalizations. For example, a 2009 Medicare study found that use of prescription drugs reduced hospitalization costs for Medicare beneficiaries.[13]
- Medicare ultimately saves $2.06 for every additional dollar it spends on drugs.[14]
- Reducing cancer deaths by 10% would be worth approximately $4 trillion in economic value.[15]

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[10] The Batelle Technology Partnership Practice. 2010. "Gone Tomorrow? A Call to Promote Medical Innovation, Create Jobs and Find Cures in America." Prepared for the Council for American Medical Innovation.
[11] Battelle Technology Partnership Practice. 2010. Battelle/BIO State Biosciences Initiatives.
[12] Kneller, Robert. 2010. "The Importance of New Companies for Drug Discovery: Origins of a Decade of New Drugs." Nature Reviews/Drug Discovery.
[13] B. C. Stuart, J. A. Doshi, and J. V. Terza. 2009. "Assessing the Impact of Drug Use on Hospital Costs." *Health Services Research* 44, no. 1: 128–144.
[14] Shang, Baoping and Dana P. Goldman. 2007. "Prescription Drug Coverage and Elderly Medicare Spending." NBER Bulleting on Aging and Health.
[15] Murphy, K.M. and R.H Topel, 2003. "Measuring the Gains from Medical Research." University of Chicago Press

- Medicare spends $91 billion each year caring for individuals suffering from Alzheimer's disease and delaying the onset of Alzheimer's by just five years would save $50 billion per year.[16]

We have a national imperative to foster the development of innovative treatments and therapies. Baby boomers are now entering into the Medicare system. By 2030, almost one out of every five Americans – some 72 million people – will be 65 years or older.[17] Currently, Medicare is projected to equal 5.1% of the U.S. GDP by 2030.[18] One of the main drivers of rising health care costs is treating chronic disease, with approximately 75 cents of every health care dollar spent on taking care of individuals suffering from a chronic disease.[19] This is even more concerning when you take into account that 45% of the population (133 million Americans) has at least one chronic disease.[20]

We as a nation need to focus policy discussions on how to unleash the promise of biotechnology so that the American public can realize the benefits it has to offer. Small biotechnology companies like my own depend on private investment to advance our innovative treatments and therapies programs and ultimately make it through the FDA approval process. A fundamental part of our ability to innovate and raise private investment is having an FDA with the resources and mechanisms required to effectively and consistently review and approve innovative products in a timely manner. These decisions must be understood by stakeholders – industry, investors, patients, and physicians – and then must be made in the context of patients and diseases being treated. The FDA is rarely praised for approving a novel therapy, yet they are often maligned if there are unforeseen adverse events that occur once a product is approved. It is imperative that policymakers understand the scientific realities of approving novel medicines. When determining if the benefits of a novel product outweigh the risks, examination of current standard of care and what level of risk patients and physicians

[16] Alzheimer's Association
[17] Alliance for Aging Research
[18] 2010. Medicare Trustees' Report
[19] Partnership for Chronic Disease
[20] Partnership for Chronic Disease

find acceptable must be part of the analysis. It is important to maintain a functioning regulatory system. Increasing requirements and associated costs without a balanced assessment of what is reasonable to accomplish and in the best interest of patients risks slowing innovation in the U.S.

The remainder of my testimony will focus on solutions. BIO has developed a set of policy proposals designed to encourage innovation through the creation of a 21st century FDA. Commissioner Hamburg said it best – "Discoveries in biomedical research are slow to find their way into patient care because the agency relies on 20th century methods to evaluate 21st century science." It is imperative that we have an FDA that is empowered and able to consistently and effectively review innovative treatments and therapies. It must be an agency which has review processes and requirements that are understood by patients, physicians, industry, investors, and policymakers. And lastly, it must be an agency that takes the diseases and patients being treated into account when evaluating innovative treatments and therapies.

POLICY PROPOSALS

First and foremost, the Congress needs to quickly reauthorize the Prescription Drug User Fee Act, or what is commonly called PDUFA. PDUFA V will enhance the drug development and review process through increased transparency and scientific dialogue, advance regulatory science, and strengthen post-market surveillance. Most importantly, PDUFA V will provide patients and doctors with earlier access to breakthrough therapies.

PROPOSALS TO RE-INVENT THE IDEA-TO-MARKET PATHWAY

Last year BIO began the process of interviewing thought leaders in our industry with the purpose of envisioning game-changing strategies. Following those conversations, BIO began a rigorous policy development process to develop a forward-thinking set of policies focused on revamping incentives for investment and improving the regulatory approval pathway. As part of this process, BIO sought, and will continue to seek, input from Members of Congress, federal agencies and institutes, patient organizations, former high-level government employees, former Members of Congress, and other policy

experts. The culmination of all of these efforts to date are described in a document entitled "*Unleashing the Promise of Biotechnology: Advancing American Innovation to Cure Disease and Save Lives*," which was unveiled during BIO's 2011 International Convention in June.

The policy recommendations we developed are designed to ensure a clear and effective pathway for turning ideas into realities that will benefit patients and improve public health. The proposals are focused on creating a 21st century FDA and creating more effective clinical research and development processes. With an increasingly aging population, it has never been more critical to support an American industry that offers solutions to the most pressing health care needs of today and tomorrow. It is imperative that FDA be an agency that recognizes its national role in advancing innovation, maintains the ability to effectively review innovative products in a timely manner, and promotes a consistent and science-based decision making process that is reflective of patient needs. The proposals described below are designed to address each of these principles. They are organized under three main headings: Elevating FDA and Empowering Operational Excellence; Advancing Regulatory Science and Innovation; and Enabling Modernized Patient-Centric Clinical Development.

ELEVATING FDA AND EMPOWERING OPERATIONAL EXCELLENCE

Update the FDA Mission Statement

FDA needs a clear mandate to encourage the development of innovative products. In addition, FDA must have the capacity and commitment to incorporate the latest scientific advances into its decision making so that regulatory processes can keep pace with the tremendous potential of companies' leading edge science. Congress can help by updating FDA's statutory mission to underscore the need for FDA to advance medical innovation by incorporating modern scientific tools, standards, and approaches into the Agency's work, so that innovative products can be made available to those who need them and in a timely manner.

Establish a Fixed Term of Office for the Commissioner of Food and Drugs

The Commissioner of Food and Drugs is charged with leading a science-based regulatory agency to advance the public health. As required by statute, the President appoints the Commissioner with the advice and consent of the U.S. Senate. However, a presumption of replacement with each new President has politicized the appointment and confirmation process. The Federal Food, Drug, and Cosmetic Act (FFDCA) should be amended to provide that the President appoint the Commissioner to a six-year term of office. Once confirmed, the Commissioner would be removable by the President only for pre-specified reasons – neglect of duty, malfeasance in office, or an inability to execute the agency's mission. Encouraging consistent and stable leadership at FDA, with protection from political influence that typically occurs during a presidential administration transition, better equips the Agency to fulfill its mission to protect and promote the public health.

Grant FDA Status as an Independent Agency

FDA regulates nearly a quarter of the consumer goods supplied to the American public. As such, the Agency should have the same authorities to make budget, management and operational decisions as afforded other independent agencies such as the Environmental Protection Agency. This would empower the Agency to work more effectively with the President and Congress to carry out its mission to promote and protect the public health. Creating an independent agency would also enhance the Agency's ability to obtain quality and consistent leadership.

Establish an External Management Review Board for FDA

FDA is a large, complex organization, and in order to fulfill its responsibilities effectively, it must be well-organized and well-managed. It is critical that the Agency's organization and management capabilities be periodically analyzed, and that the Commissioner of Food and Drugs be provided with fresh, visionary, and independent thinking on how to improve the ability of the Agency and its centers to promote and protect the public health, as well as the support necessary to implement recommendations. An external advisory board composed of individuals with experience in organizational management could help the Agency address operational challenges.

Current law should be amended to establish a Management Review Board (MRB) to conduct periodic reviews of FDA's management and organizational structure, and to provide recommendations to the Commissioner about ways to improve FDA operations. This idea is modeled upon the Scientific Management Review Board at the National Institutes of Health, which was developed and passed by this Committee and the Congress as part of the NIH Reform Act of 2006.

ADVANCING REGULATORY SCIENCE & INNOVATION

Support Regulatory Science Public-Private Partnerships

Under the Food and Drug Administration Amendments Act of 2007 (FDAAA), Congress established the Reagan-Udall Foundation for the Food and Drug Administration, an independent non-profit organization intended to support public-private partnerships for the purpose of advancing the mission of FDA to "modernize medical [and other] product development, accelerate innovation, and enhance product safety." The Foundation could, for example, form collaborations to advance the use of biomarkers, surrogate markers, and new trial designs to improve and speed clinical development. However, Congressional appropriations bills for the Agency have subsequently restricted FDA's ability to transfer federal funding to the Foundation. These funding restrictions should be lifted so that the Reagan-Udall Foundation can fulfill its promise.

Create an FDA "Experimental Space," led by a Chief Innovation Officer, to Pilot Promising New Scientific and Regulatory Approaches

FDA has developed several initiatives to advance regulatory science. These include the FDA/NIH Joint Leadership Council, the academic Centers of Excellence in Regulatory Science, and FDA's Critical Path Initiative. However, FDA's ability to incorporate modern science into its regulatory processes has been limited because there is no entity within the Agency with unified responsibility for systematically analyzing the findings and recommendations from these groups, and with clear authority to pilot promising scientific and regulatory approaches. An FDA "Experimental Space," led by a new Chief

Innovation Officer, should be established with the responsibility and authority to ensure that promising new approaches are integrated into Agency operations at all levels.

Enhance FDA's Access to External Scientific and Medical Expertise

FDA is the preeminent federal agency charged with evaluating cutting-edge science as it is applied to the prevention, diagnosis, and treatment of human disease. FDA also has been perceived by many as the global standard bearer for regulatory review of drug and biologic applications. However, scientific and medical knowledge, techniques, and technology are advancing at a more rapid pace today than at any other time, and FDA's capacity to access information about these advances has not kept pace. It is essential that FDA's access to scientific and medical advice be enhanced by improving the operations of FDA Advisory Committees, establishing Chief Medical Policy Officers in the immediate offices of the Center Directors, and providing FDA staff with additional avenues for accessing external scientific and medical expertise.

ENABLING MODERNIZED PATIENT-CENTRIC CLINICAL DEVELOPMENT

Increase Access to Innovative Treatments and Therapies through Progressive Approval

Patients, industry, Congress, and others are eager to find ways to deliver safe and effective new drugs and biologics to patients. Patients, particularly those with illnesses for which no adequate therapy exists, want access to promising new therapies earlier in the drug development process. Smaller biopharmaceutical companies that develop those therapies are sometimes unable to maintain operations through extensive phase III testing without revenue from the sale of products. Expanding and improving the accelerated approval pathway into a progressive approval mechanism would help provide patients more timely access to needed therapies. This pathway would be limited to innovative products for unmet medical needs, significant advances to standard of care, targeted therapies, and those that have been approved by the European Medicines Agency (EMA) or other mature regulatory agencies. This pathway also would ensure risk-benefit analysis that incorporates the safety and needs of patients in the real world.

Empower FDA to Utilize a Weight-of-Evidence Approach

FDA's current statutory authority requires that the Agency approve applications for new drugs when they have been demonstrated to be safe and effective under the intended conditions of use. The law provides that effectiveness is established where FDA is satisfied that there is "substantial evidence" that the new drug has the intended effect that it is purported to have. FDA typically requires two "adequate and well controlled" studies under this standard. A weight-of-evidence approach to data analysis, however, would allow the decision-maker to look at all data and information, whatever its value, and give each appropriate consideration.

Leverage Electronic Health Records to Facilitate Clinical Research

Every new drug's sponsor spends years designing and conducting clinical trials to show their drug is safe and effective. Using health information technology (IT) such as electronic health records (EHRs) in clinical research will improve and speed up the drug development process, and decrease costs. However, there are significant barriers preventing wide-spread use of health IT in clinical research, including slow adoption by providers and lack of standards development. FDA can help remove those barriers. Congress should create a Clinical Informatics Coordinator in the Office of the Commissioner of Food and Drugs charged with developing processes to validate and encourage the use of health IT in clinical research, and establishing pilot projects to use health IT in clinical research.

Require FDA to Disclose to the Sponsor Reasons for Non-Approval

The FFDCA implies that licensing or approval applications contain a binary question – approve or deny – due to phased, investigational review of applications; however, there is in practice a third response. In this case, FDA neither approves nor officially denies the application (which would require FDA to give the sponsor specific procedural rights such as a hearing); rather it finds the application to be incomplete in some way that makes the application ineligible for approval. When FDA makes such a finding, it should communicate to sponsors in clear terms why risk was determined to outweigh benefits, and why other Agency authorities such as Risk Mitigation and Evaluation Strategies

(REMS) – which are designed to mitigate risk for approved products – are insufficient (in addition to indicating what must be done to address any deficiencies). Such an approach would help create a consistent and transparent evaluation of risk-benefit, and provide the sponsor with better information on what, if any, additional studies are required to achieve approval.

Conclusion

We have a national imperative to support and foster advances in medical innovation. The full potential of biotechnology industry to cure disease and offer real solutions to our nation's most pressing health care needs has yet to be realized. We look forward to working with you on developing policies for a 21st century FDA that will serve to unleash the promise of biotechnology in the United States.

Mr. BURGESS. Thank you for your testimony.

Mr. Casey, you are recognized for 5 minutes.

STATEMENT OF DONALD M. CASEY

Mr. CASEY. Thank you, Congressman Bilbray and Congressman Burgess. Thank you for inviting me to testify. And thank you for coming to San Diego, and you get to see the weather.

And I would also like to add it has been a pleasure working with your committee and your fine aide Clay, who has been very, very helpful in us understanding the process.

My name is Don Casey, and I am the CEO of West Wireless Health Institute, and I spent 30 years working in the health-care industry.

West Wireless Health Institute is a not-for-profit medical research organization whose mission is to lower health-care costs through technology and innovation. With $100 million in funding today from Gary and Mary West, the institute has hired more than 60 scientists, engineers, and other experts who are incubating promising health-care technologies and engaging with policymakers on broad efforts to dramatically lower health-care costs.

I would offer the following points today. First, novel medical technologies can save us literally billions of dollars while creating tens of thousands of new jobs. But they have to get through the FDA first. Second, the current regulatory climate, as you have heard from other testifiers today, is more challenging than ever and is slowing innovation and, indeed, driving U.S. jobs overseas. And third, there are several practical solutions the FDA can undertake today, including modifying the agency's de novo 510(k) regulatory review process, that would actually substantially aid in this process.

The institute believes that novel medical technologies will play a huge role in health-care delivery in the future, providing better care for chronic diseases and dramatically lowering health-care costs, while literally creating tens of thousands of new U.S.-based jobs that can't be shipped overseas. We base this on our own research as well as major studies, including one by the VA. These studies show that we can reduce hospital readmissions, emergency room visits, and overall lower costs by up to 20 to 30 percent by using an infrastructure independent model for health care in the future.

However, the regulatory environment that exists today is making it extremely difficult for such an innovative healthcare delivery system to be, A, developed, and then, B, deployed.

The FDA is slowing development in novel health-care technologies in a number of ways. First, in our industry, by determining that once a device that has a wireless communications component, it may require different review process. With traditional medical devices, a manufacturer in the 510(k) process can cite a predicate device; i.e., a thermometer is a predicate for a new thermometer. The minute you add a wireless component now, a wireless thermometer can no longer cite a thermometer as a predicate device, which sets off a lengthy and somewhat circuitous regulatory review process where they ask you to cite the predicate even though you want to say that there is no predicate.

A second is regulatory overreach. The FDA is exercising authority over more and more areas of health-care information technology, particularly where they are intersecting with medical devices. By taking an expansive regulatory approach to data collection, data transmission, and data analysis, the FDA is increasingly raising the bar so high that we are discouraging new entrants into the field.

We ask ourselves all the time at the Institute, where is the goal in health care? It is information-intensive. It should be able to be searched. You can't do that, though, if the FDA continues to take a very aggressive and expansive approach to regulation.

I would offer the institute's own experience with our first prototype, Sense4Baby. And I would be remiss if I didn't show you Sense4Baby.

Sorry, if Bill can have a tall chair, we can have a Sense4Baby.

This is the device that we are looking for, to focus on high-risk pregnancy, which basically takes a $4,000 to $5,000 device that is currently sold in a hospital, and we can break this down into components that might cost us under $100 when using a cell phone to transmit this data.

We are very excited about the potential it has to dramatically lower health-care costs—and I know, Dr. Burgess, you would appreciate this—by identifying high-risk mothers and giving them the capacity to be monitored on a regular basis and pushing that data to you.

We are, however, once we have developed this process right up the road in La Jolla, going to actually begin to do our preliminary validation studies in Mexico. That is driven strictly by our interpretation of FDA regulations that says wireless health-care devices need to be looked at as—the wireless component—as a medical device in and of itself.

For us, it is a more expeditious path to actually get experience with this product by taking it to Mexico, which does not look at this in the same way.

New medical innovation is pushing the bounds of FDA's current regulatory paradigm, particularly those where a predicate does not exist. Traditionally, the FDA has relied on the de novo process to provide approval pathway for low-risk medical devices products that have no identifiable predicate. But the de novo pathway is not working efficiently today.

Review times for de novo products during the last 4 years are now almost twice as long as the FDA's promise for a PMA submission. So basically, we are taking a 510(k) device that should be much more accelerated versus a PMA, and we are now seeing this de novo process taking it to be longer than the PMA, and this is very discouraging to industry.

The fundamental problem with de novo today is it can only be pursued after the manufacturer applies for and completes the initial 510(k) review and then receives an official letter from the FDA explaining that there is not an approved predicate device. This time-consuming process must be completed even if the manufacturer is willing to acknowledge right up front that there is no predicate device before submitting the device for approval.

We recommend that Congress reform the de novo process to allow device manufacturers to proactively initiate the de novo approval process without having to undertake the circuitous process that is required today.

The FDA could also foster greater innovation and speed of delivery for safe, effective medical technology by making the 510(k) process, as you have heard today several times, more predictable, transparent, and reasonable.

The FDA's own reports indicate today that the average time for a 510(k) review has increased from 96 days to 140 days over the last decade. That is not even looking at industry studies that might show that that data is almost triple that.

Manufacturers are increasingly being asked for new information late in the approval process, subjecting them to changing endpoints and delays. This stance has deterred venture capital, as we just heard, even for something in the low risk, like something like we are developing.

In sum, we would ask the Members of Congress and the FDA to work to preserve patients' access to innovative products by taking the following steps: reject the IOM's recommendation and ensure that FDA preserves the 510(k) process to allow low-risk devices to come to market in a timely manner. We want to strengthen the 510(k) process so that manufacturers have more certainty, clarity, and predictability in the approval pathways. And finally, we want to reinvigorate the de novo process by permitting medical devices without a predicate to initiate an approval process without having to exhaust the 510(k) process.

We believe that all of that will, A, increase jobs, B, dramatically lower costs, and the final benefit of that is that you're going to get better patient care over time.

We will look forward to working with Congress and building upon its leadership role in spurring innovation, creating new jobs for America, and helping all patients benefit from an infrastructure independent model for health care. Thank you.

[The prepared statement of Mr. Casey follows:]

House Committee on Energy and Commerce
Subcommittee on Health
September 26, 2011
Statement of Donald M. Casey
West Wireless Health Institute

Congressmen Bilbray and Burgess, thank you for convening this field hearing regarding the impact of medical device and drug regulation on innovation, jobs and patients and inviting me to testify. My name is Donald M. Casey, and I am the Chief Executive Officer of the West Wireless Health Institute, based here in San Diego, California. I have spent almost 30 years working at the intersection of medical devices and innovative health technologies, including serving as a corporate officer and executive committee member at Johnson & Johnson, and most recently, in my role at the Institute, focusing on novel medical technologies to lower health care costs.

The West Wireless Health Institute (WWHI) is a non-profit medical research organization that was established in 2009 with the primary mission of lowering health care costs through technology and innovation. With more than $100 million in philanthropic funding to date from Gary and Mary West, the Institute has hired more than 60 scientists, engineers, economists and other experts who are focused on incubating promising health technologies; validating their value; and, engaging with policymakers on broader efforts to dramatically lower health costs.

I want to offer three points today:

1) Novel medical technologies such as wireless health devices can save the United States billions of dollars in health care spending, while creating tens of thousands, if not hundreds of thousands, of new jobs – but they have to get approved by FDA first;
2) The current regulatory climate is more challenging than ever and is slowing innovation, preventing the deployment of these solutions and indeed driving U.S. jobs overseas;
3) We understand that the Food and Drug Administration (FDA) has a tremendously difficult job, but we believe there are several practical solutions that they can undertake, like a potential modification to the agency's De Novo process, that will spur innovation rather than inhibiting it.

WWHI believes novel medical technologies will play a huge role in health care delivery in the future – providing better care for chronic disease and dramatically lowering health care costs, while creating tens of thousands if not hundreds of thousands of U.S.-based jobs. We base this on our own research, as well as on major studies including one by the Veterans Administration (VA) published in 2008. The VA's Care Coordination/Home Telehealth (CCHT) program, which currently has 71,000 patients enrolled, demonstrated a 25 percent reduction in bed days of care (including 50 percent for patients in highly rural areas) and a 19 percent reduction in hospital admissions in its first six years by linking chronically ill veterans with health care providers through messaging and biometric devices, and other remote monitoring technologies. The program has demonstrated tremendous results and cost savings – and we should be pushing for a system like this on a national basis, not just within the confines of the VA health system, but for all patients with chronic conditions who would benefit from such care.

However, the regulatory environment that exists today is making it extremely difficult for such innovative care delivery – and the very technologies that make it possible to deliver such infrastructure independent care – to be developed and deployed on a widespread basis. This environment includes a lack of regulatory clarity and the FDA operating at a very cautious end of the risk analysis spectrum, which is prohibitive to innovators.

The FDA is slowing the development of novel health technologies in three ways:

1) By determining that once a device that has a wireless communications component it requires different clinical validation. With traditional medical devices, a manufacturer can cite a predicate device (i.e., a thermometer is a predicate to a thermometer). However, by adding a wireless communications component, a manufacturer can no longer cite a thermometer as a predicate device for a *wireless* thermometer. As illustrated above, this sets off a lengthy and counterproductive regulatory cycle.
2) Regulatory overreach. The FDA is exercising authority over more and more areas of healthcare information technology. By taking an expansive regulatory approach to the data collection, transmission and analysis side of healthcare information technologies, the FDA may raise the bar so high as to actively discourage new entrants into the health care innovation field, including the mHealth or wireless health sector.
3) The current lack of urgency and transparency at the FDA is also slowing U.S. job growth, preventing us from realizing a whole new vision for health care and indeed may be driving jobs overseas.

WWHI offers our own experience with our wireless fetal and maternal monitoring prototype, Sense4Baby. Sense4Baby is a hand-held and portable wireless prototype for standard fetal cardiotocography, which is used to monitor pregnancies, and is particularly critical for monitoring high-risk pregnancies. The prototype is designed to be a very low cost way to allow a care coordinator or clinician to get hospital-like monitoring in a non-hospital setting. While the prototype is being developed in the U.S., the Institute will be soon conducting validation studies in Mexico. This decision is based on our ability to test the technology in a country that does not regulate wireless communications technology as a medical device. We will learn more about the prototype in a medically-supervised and safe environment and then eventually bring it to the U.S. for regulatory review. But even on this small project, we will be spending money and creating jobs outside of the U.S.

New medical innovation, like wireless health technology, is pushing the bounds of FDA's current regulatory paradigm, particularly where a predicate device may not exist. Traditionally, the FDA has relied on the Evaluation of Automatic Class III Designation, or De Novo Process, which was originally intended to provide an approval pathway for low-risk medical device products that have been classified as class III solely because there is no identifiable predicate device. But the De Novo pathway has not worked efficiently. Review times of "De Novo" products during the last four years have been steadily increasing and are now almost twice as long as FDA's promised review times of panel-track PMA submissions, and more than three-times longer than the committed review time of 510(k) submissions, with a total average review time of 482 days. As the length of review times for the De Novo process has grown, the number

of innovative devices being approved through the pathway has waned, and to date only 54 products have been approved under the De Novo process since 1998.

The fundamental problem with the current De Novo pathway is that it can only be pursued after the manufacturer applies and completes an initial 510(k) review and receives an official Not Substantially Equivalent (NSE) letter indicating that there is not a currently approved predicate device. This time-consuming process must be completed even if the manufacturer is willing to acknowledge and stipulate that there is no predicate device before submitting the device for approval. This mandatory requirement to have exhausted and failed a 510(k) submission is wasteful of time and resources for both the FDA and manufacturers.

To promote innovative devices and help free up FDA resources, we recommend that Congress reform the De Novo process to allow device manufacturers to proactively initiate the De Novo approval process without having to undertake and await FDA review on a 510(k) application. Specifically, for devices that the manufacturer knows and acknowledges lack predicate device, the agency should not require they submit and await a full 510(k) review just to receive an NSE determination declaring there is no predicate. This process should be limited to low-risk devices that do not require clinical trials and are unlikely to be designated class III after review.

We were pleased to see the FDA's statement opposing the Institute of Medicine's recommendation to eliminate the 510(k) process, which is a key part of the agency's ability to evaluate and approve medical devices. The current 510(k) process, despite its flaws, provides a

known pathway for innovators and investors to make iterative improvements to medical technology, which results in continual improvement of medical devices available to patients.

However, we believe FDA could foster greater innovation, speed delivery of safe and effective technology to waiting patients, and also spur U.S. job creation by making the 510(k) approval process more predictable, transparent and reasonable. As we work with medical device innovators, we are hearing increasing frustration with unpredictable regulatory requirements, frequently changing review goals, and inconsistently applied standards that result in confusion and unnecessarily slow approval. Further, in Josh Makower's study of the medical device industry, respondents indicated that while it took an average of 10 months to get through the 510(k) process in the United States, an equivalent device took only 7 months from first communication with regulatory agencies to market in Europe. For full PMA applicants, products in the US took an average of 54 months to reach approval, while a comparable device took only 11 months in Europe.

FDA's own reports indicate that total average time for a 510(k) review has increased from 96 days to 140 days over the last decade. The FDA also reported an increase in the number of review cycles, as well as an increase in the percentage of applicants receiving Additional Information (AI) Letters- up to 77% of 510(k) applicants in 2010. Manufacturers are increasingly being asked for new information in the middle of the approval process, subjecting them to changing endpoints and unnecessary delays and obstacles. This stance has deterred venture capital and capital formation in the device industry even for low-risk devices. Regulatory authority around novel medical technologies including wireless health devices, healthcare information technologies and electronic health records must be narrowed, and we must also look for novel regulatory pathways for those devices that should be cleared through the agency.

In sum, we ask that Members of Congress and the FDA work to preserve patients' access to innovative products by taking the following steps:

1. Reject the IOM's recommendation and ensure that the FDA preserves the 510(k) process to allow low-risk devices to come to market in a timely manner.
2. Strengthen the 510(k) process so that manufacturers have more certainty, clarity and predictability in the approval pathway.
3. Reinvigorate the De Novo process by permitting medical devices without predicate to initiate that approval process without having to needlessly waste time exhausting the 510K process.

We are on the threshold of a paradigm shift in health care delivery, one in which we realize the full potential of the digital and wireless revolution and make 'anytime, anywhere' care a reality. This shift requires that the FDA be prepared to review and address innovative devices that may not have a predicate, but will increase the reach of health care providers and reduce costs by preventing the continued reliance on catastrophic care for many illnesses. We look forward to working with the Committee in building upon its leadership role in spurring innovation, creating new jobs for Americans, and helping all health care consumers benefit from the evolution of an infrastructure-independent model of health care.

Mr. BURGESS. Thank you.

Mr. Panetta, you are recognized for 5 minutes for your opening statement.

STATEMENT OF JOE PANETTA

Mr. PANETTA. Thank you, Vice Chairman Burgess and Representative Bilbray. Thank you both for being here in the center of global innovation of the biomedical industry of Southern California.

My organization, BIOCOM, is the advocacy organization for our industry here in Southern California. Ninety percent of our members have fewer than 50 employees, and most have products in research and development that hopefully will commercialize them in the near future, if we can create an environment that is conducive to their being able to do this. And today I am glad to be here to be able to talk to you about some of the challenges that we face.

I also want to acknowledge the great work of committee staff, and Mr. Bilbray's committee staff as well, his personal staff, Gary Kline, the great work that we have done with Gary. We appreciate the relationship that we have had. And we appreciate the work that Congressman Bilbray has done to address the challenges that we face as an industry.

I want to address in sequence here some of the challenges that we face as a result of FDA's medical device regulation, and that impact on innovation jobs and patients.

As you have heard indirectly here, our companies face one challenge. Their fate is completely determined by a single federal agency, and that is the FDA.

Without approval from the FDA at each stage of the commercialization process, nothing else about the company matters. Life science companies must be afforded transparent, predictable, clear regulatory processes to encourage the immense investment that it takes to get a concept from discovery to commercialization.

However, as you have heard, according to our members, the current environment at the FDA is perceived by investors to be unpredictable, hesitant, and risk-averse. This environment has created a funding crisis for many of our small to midsize companies, and these are the companies that have been central to the growth of the life science industry over the past 20 years.

And I will remind you that this is an industry that has constantly outperformed other sectors during the past economic downturn.

It is essential that FDA focus on innovation to be strengthened in order to maintain this country's leadership in biomedical innovation, and all of the jobs that go with that global leadership. Even some of the most strident critics of the life science industry, including California's own raking member Henry Waxman, agree that the FDA is perceived as being a failed agency by much of the American public.

The U.S. is headed for a crisis as more and more boards and investors are demanding that companies first commercialize their products outside the U.S., where safety standards may be just as rigorous, but the approval process is more consistent, transparent, and predictable. And the companies, therefore, have a better chance of realizing a revenue stream.

Unfortunately, that FDA's leadership has made recent public statements that appear to indicate that the agency is in denial regarding this migration of products to Europe. I can assure you that our members would reinforce the fact that this is correct.

For example, a recent BIOCOM member survey revealed that 59 percent of our respondents are developing strategies to seek approval and commercializing outside the U.S. before seeking FDA approval.

Sending innovation to another country first has many grave implications. Jobs related to clinical trials and development are moved to those countries in which approval is being sought. Once approved, manufacturing facilities and distribution are set up in and around those countries. And those jobs are not just limited to Ph.D.s.

This isn't a matter simply of jobs. Patients become aware of the most current and effective treatments and technologies, but only those who can afford it can travel to foreign countries to access them. This is ironic, because it indicates a reversal in the prior trend over years of patients from the E.U. and other places coming to the U.S. to access cutting-edge treatments.

It also should be noted that many insurers won't provide coverage for a therapy or device not approved in the U.S., so this option is limited to those who can afford to pay all costs out-of-pocket. For the vast majority of Americans, however, it just means that they must go without the best and most current therapies and technologies.

The impact on patients in the U.S. is undeniable. They are being denied access to the most cutting-edge lifesaving, or life-changing products.

I want to remind you, members of the committee, that FDA's mission is very clear. Its primary mission is both to protect public health and to promote public health as it relates to approval of drugs, devices, and diagnostics.

BIOCOM industry members share a common goal with the agency and support the desire to improve public health by bringing innovative, high-quality products to patients in a timely fashion. But we continue to hear from our members that this system is creating delays, it is more unpredictable, and more data seems to be required than in previous submissions.

We don't believe that this is a reflection on the quality of the data that our companies are submitting so much as a lack of effective communication between reviewers in the industry as well as the issue of reviewers' access to training and education of the newest technologies.

We have conducted a survey five times over the last 16 years with PricewaterhouseCoopers called "Improving America's Health." I have provided copies to all the members of the committee.

Our survey continues to indicate a growing concern that the FDA is becoming more detached from the industry that it regulates. The survey seeks to evaluate the relationship between the FDA and the companies regulated by it. Sixty percent of our survey respondents stated that the FDA changed its position during the course of a review, and that was up from 40 percent 4 years ago. Almost half of the people responding to the survey felt the products were being

denied in part because of inadequate resources at the agency, and 48 percent reported a break in continuity during the review process.

This level of uncertainty isn't surprising when taken in the context of the current state of the agency's human resource pool. Dr. Schoen, head of CDRH, recently said in a town hall meeting that the average CDRH reviewer has less than 3 years of experience.

With that level of turnover, it is not surprising the review times become elongated as new reviewers are brought up to speed on the industry. Sometimes these new reviewers may request information or data on issues never previously raised with the applicant. Often these requests can require studies costing millions of dollars and a year or more to complete.

I want to point out that FDA has recognized that they have a problem, and they are implementing measures to address some of these specific measures. However, BIOCOM is concerned that that these changes won't go far enough. Therefore, we have developed specific, focused recommendations that we intend to share with FDA and with Congress regarding potential organizational improvements and efficiencies to improve the agency's overall performance. And I would be glad to discuss these as well.

I just want to turn briefly to a few other issues that add uncertainty to the uncertainty that we already feel as an industry.

First, the Independent Payment Advisory Board enacted as a part of health-care reform is an unelected body, which could institute broad changes to Medicare with no congressional input or oversight. And we don't support this approach.

Medicare Part D, which provide seniors with long-sought drug benefits, and part B, which provides oncology products for administration in physician offices, has been under threat.

You have heard about the medical device excise tax. This will be devastating to many of our members, and we support the efforts of both Representative Bilbray and of Representative Paulsen of Minnesota to repeal this unfair tax, which, as you have heard, takes dollars away from R&D and job growth.

And finally, there are those, including the President of the United States, who continue to seek to reduce the 12-year data exclusivity period for biologic products. That 12-year period has been supported overwhelmingly in Congress on a bipartisan basis.

Innovation in the life science industry is meaningless if it doesn't reach the patient, so we at BIOCOM welcome constructive dialogue with agencies, with Congress, the industry, FDA leadership, and the patient population we all seek to help, so that we can come together to ensure patient safety and advances for cures and treatments that will reduce overall health-care costs, create jobs, and benefit all Americans while ensuring that our citizens have access to them.

Thank you for this opportunity, and thank you for your time in coming to San Diego.

[The prepared statement of Mr. Panetta follows:]

BIOCOM

Prepared Testimony of Joe Panetta, President and CEO of BIOCOM

House Committee on Energy and Commerce Field Hearing

"Medical Device and Drug Regulation on Innovation, Jobs and Patients"
September 26, 2011

Vice Chairman Burgess and Members, I am Joe Panetta, President and CEO of BIOCOM.

BIOCOM leads the advocacy efforts of the Southern California life science community with more than 550 members that include biotherapeutics, medical device, diagnostics, industrial biotechnology and biofuels companies, contract research or manufacturing organizations, universities and research institutions.

The Southern California life science cluster is one of the most robust in the world. There are approximately **40,000 employees** in the life science community in just San Diego County. We are rightfully proud of the fact that Southern California is internationally recognized for producing some of the best and most exciting discoveries in the life science research arena.

We have been asked to speak to the impact of medical device and drug regulation on innovation, jobs and patients. BIOCOM's member companies share one thing in common: their fate is completely dependent on a single federal agency, the FDA. Without approval from the FDA in

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each stage of the commercialization process, nothing else about the company matters. Life science companies must have transparent, predictable regulatory processes to encourage the immense investment it takes to get a concept from discovery to commercialization. However, according to many of our members, the current environment at the FDA is perceived by investors to be arbitrary and risk-averse. This environment has created a funding crisis for many small and midsize companies. These are the companies that have been central to the growth of the life sciences industry over the past 20 years, an industry that has consistently outperformed other sectors through past economic downturns.

It is essential that the FDA's focus on innovation be strengthened in order to maintain this country's leadership in biomedical innovation...and all of the jobs that go with that global leadership. Even some of the most strident critics of the life sciences industry, including California's own Ranking Member Henry Waxman agree that the FDA is in danger of being perceived as a "failed agency" by the American public. The U.S. is heading for a crisis as more and more Boards and investors are demanding that companies first commercialize their products outside the U.S., where safety standards may be just as rigorous as the U.S. but the approval process is more consistent, transparent and predictable and the companies therefore have a better chance of realizing a revenue stream. In fact, a recent BIOCOM member survey revealed that 59 percent of respondents are developing strategies to seek approval and commercialization outside the U.S. before seeking FDA approval. Sending innovation to another country first has many

grave implications. Jobs related to clinical trials and development are moved to those countries in which approval is being sought, and once approved, manufacturing and distribution chains are set up in or around those countries. The types of jobs created are not limited to PhD's. Many of the jobs can be filled by people holding a community college certification to people holding a Master's Degree.

This is not a matter of simply jobs lost, although that should be enough to garner the attention of Congress. Patients become aware of the most current and effective treatments and technologies, but only those who can afford it can travel to foreign countries to access them. It should be noted that many insurers will not provide coverage for a therapy or device not approved in the U.S., so this option is limited to those who can afford to pay all costs out of pocket. For the vast majority of Americans, however, it just means they must go without these best and most current therapies and technologies. The impact to patients in the US is undeniable…they are being denied access to life saving or life changing products.

The FDA's mission is clear. The agency's primary mission is to protect and promote the public health as it relates to approval of drugs, devices, diagnostics, and the food supply. BIOCOM industry members share a common goal with the agency and support the desire to improve public health by bringing innovative, high quality products to patients in a timely fashion.

BIOCOM continues to hear from its industry members that with each coming year, review times become longer, more unpredictable, and more data seems to be required than in previous submissions. Our members believe this is not a reflection on the quality of the submissions so much as a lack of effective communication between reviewers and the industry, as well as the issue of reviewers' access to training and education on the newest technologies.

The 2010 edition of a survey conducted by PwC and BIOCOM called "Improving America's Health - V" [1](I've provided copies for all members of the committee), indicates a growing concern that the FDA is becoming more detached from the industry it regulates. The survey seeks to evaluate the relationship between the FDA and he companies regulated by it. Sixty percent of survey respondents stated the FDA changed its position during the course of a review, up from forty percent just four years ago. Almost half the people responding to the survey felt products were denied in part because of inadequate resources at the agency. And forty-eight percent reported a break in continuity during the review process.

This level of uncertainty is not surprising when taken in context of the current state of the agency. In a town hall forum in San Francisco last week, the Director of the FDA's Center for Devices and Radiologic Health admitted that the average CDRH reviewer had less than 3 years

[1] PwC/BIOCOM; Improving America's Health V, 2010; http://www.pwc.com/us/en/health-industries/publications/improving-americas-health-v.jhtml

of experience. With that level of personnel turnover, it is not surprising review times become elongated as new reviewers are brought up to speed on the history of an application. And sometimes these new reviewers may request information or data on issues never previously raised with the applicant. Often, these requests can require studies costing millions of dollars and a year or more to complete. This situation can start to be addressed through collaborative FDA/stakeholder sponsored science-based training programs and continuing education programs that focus on enhancing agency staff scientific capabilities. Such training will help achieve more consistency throughout the agency.

I want to point out that the FDA has recognized there is a problem and is implementing some measures to address specific issues. However, BIOCOM is concerned that these changes will not go far enough.

I would be remiss if I did not also mention current issues before Congress that add to the uncertainty felt by the industry and keep precious investment dollars on the sidelines or drive investors to other industries.

- The Independent Payment Advisory Board, enacted as part of health care reform, is an unelected body which could institute broad changes to Medicare with no Congressional input or oversight.

- Medicare Part D, which provides seniors with a long-sought drug benefit, and Part B, which provides oncology products for administration in physicians' offices, have been under threat.
- The medical device excise tax, also a part of the 2010 health care reform, will impose a 2.3 percent tax on revenues regardless of profitability. This will be devastating to smaller medical device companies.
- Also, there are those, including the President, who seek to reduce the 12 year data exclusivity period for biologic products. The 12-year period has been supported overwhelmingly in Congress on a bipartisan basis. The possibility that the rules may change may change on these important issues creates a pall of uncertainty that is central to the crisis in which the life sciences industry now finds itself.

Innovation in the life sciences industry is meaningless if it does not reach the patient. BIOCOM welcomes a constructive dialogue with the agency and with Congress in which lawmakers, the industry, and the patient population we all seek to help can come together to insure patient safety and advance the cures and treatments that will reduce overall health costs, create jobs and benefit all Americans while insuring that our citizens have access to them. Thank you for this opportunity, and your time and concern on these issues.

Mr. BURGESS. Thank you for your testimony.

Mr. Larkin, you are recognized for 5 minutes for purposes of an opening statement.

STATEMENT OF KEVIN T. LARKIN

Mr. LARKIN. Good afternoon. I am Kevin Larkin, CEO of TherOx, a venture-capital-supported clinical stage medical device company located in Irvine, California. And I appreciate the opportunity to testify before you today.

As background, I have spent 37 years in the cardiovascular medical device business in items such as heart pacemakers, implantable defibrillators, angioplasty catheters, and stents. As a consumer and as an occasional patient, I absolutely recognize the need for and support reasonable evaluation and regulation roles for FDA. These facilitate, but do not foster, innovation.

My peers and physicians I have worked with over the years are overwhelmingly honest and ethical, and will not knowingly do harm. Unless and until these values are violated, they should be treated as professionals with trust and respect.

I believe also that FDA has huge challenges satisfying its diverse, heterogeneous constituents: the public, physicians, industry, media, and legislators. Most of these groups do not understand the risk-benefit aspect of medical treatments.

Specific to the PMA regulatory path, the advisory panel process is dysfunctional and counterproductive. The good news is it can be fixed fairly easily.

As background, TherOx is a non-revenue-generating, clinical stage company that pioneered a breakthrough heart attack treatment that minimizes heart damage compared to the standard of care.

We successfully completed a controlled, randomized pivotal trial that met both its effectiveness and safety endpoints with headroom. Our trial design was collaboratively worked out with our FDA review team, a group within CDRH.

After we submitted our trial data, our FDA review team on its own initiative awarded us what is called expedited review, a classification that not only indicates prespecified criteria were met, but that the new therapy treats a serious disease state for which there are no better alternatives. Despite this apparent success, an advisory panel did not vote to recommend approval of our therapy.

Here are some observations from that panel meeting. Physician composition of 15 members, three routinely treat heart attack patients and, therefore, know the practices, logistics, adverse event and mortality rates for this serious disease state. These physicians were ignorant, even dismissive of our Bayesian statistical design, which was recommended by our FDA advisory group.

Because they don't routinely treat heart attack patients, they were unfamiliar with our heart measurement assessment, Sestamibi Nuclear Imaging, a well-validated assessment tool. They dismissed it.

They were concerned about a 1.8 percent death rate in the treatment arm of the group. They didn't understand the normal death rate was greater than 3 percent at the time. They just didn't know.

An untrained advisory panel chair accepted a motion for approval without conditions, completely inappropriate for a full PMA product. Minimally, a postapproval study is always a condition for approval.

The panel vote was 9-to-5 not to recommend approval, despite the trial having met its endpoints.

Here is a summary of actions since that meeting.

Jobs were lost. One of my first actions post-panel was to fire half of the employees as a cash conservation maneuver while we attempted to work through an approval path with FDA. The employees let go had done their jobs effectively, helped us deliver a successful trial, and did not deserve this outcome.

Since that panel meeting, heart attack patients, particularly those suffering large, debilitating heart attacks, have no access to our therapy.

The advisory panel is being tweaked slightly. For the most part, it operates as it has in the past.

Venture capital, by far the dominant financing resource for innovative breakthrough medical devices, is increasingly abandoning investments in PMA product research due to uncertainty over FDA approval, even when outcomes meet goals.

No doubt, many of you here today have heard a variety of criticisms about FDA policies, personnel, arbitrariness, follow-up, and more. Unlike many of my peers who run small medical device companies, I have high regard for most of our particulars CDRH members. They are collaborative, helpful yet firm, and importantly, they are responsive. I appreciate that we work together and how each of us tries to work toward deliverables.

As you may have assumed by now, my biggest single criticism is with the advisory board. The good news is that I believe the advisory panel function can be monumentally improved with just a few changes. Here are a couple recommendations.

Reconcile the ridiculously extreme conflict of interest restriction that increasingly staffs advisory panels with nonspecialists in the field being evaluated. Strike a balance between accessing expertise with reasonable, commonsense restrictions. Require advisory panel members to pre-read the prepared material and attest to having done so, or be disqualified.

If the FDA adopts and recommends trial design efficiencies, such as Bayesian statistics, uneven randomization, and other maneuvers, panel members have to be educated and oriented to such concepts or be considered not qualified. Panel members who make false or misleading statements must be corrected immediately by either the FDA or the sponsor to prevent negatively influencing other members. And instruct panel members to evaluate the trial and the results being presented, not redesign it on the fly. Train panel chair and other members on panel logistics, voting procedures, risk-benefit interpretation, and other issues critical to effective panel function.

Having gone through what I call a good example of a bad example, I believe a few changes like these can preserve the important independent advisory function intended for panels, yet dramatically improve the effectiveness and the assistance of those panels in FDA's decisions to approve or not and under what conditions.

The big picture: This is about maintaining our overwhelming U.S. leadership in medical research and innovation. It is about U.S. citizens being able to access the latest, most effective new treatments. It is about preserving venture capital's dominant role in financing daring new breakthrough treatments. And it is about adding, not reducing, U.S. jobs in this exciting medical device field.

I appreciate having had the opportunity to testify today. I offer to be of assistance in any way I can.

[The prepared statement of Mr. Larkin follows:]

KEVIN T. LARKIN
PRESIDENT AND CHIEF EXECUTIVE OFFICER OF THEROX, INC.

WRITTEN STATEMENT OF PROPOSED TESTIMONY BEFORE THE
COMMITTEE ON ENERGY AND COMMERCE
SEPTEMBER 26, 2011, SUBCOMMITTEE ON HEALTH
ENTITLED "IMPACT OF MEDICAL DEVICE AND DRUG REGULATION ON INNOVATION, JOBS AND PATIENTS: A LOCAL PERSPECTIVE."

SUMMARY

Despite there currently being significant criticism directed at FDA, its procedures, personnel, and decisions relative to medical device approval, I believe most of it is fixable without extraordinary measures.

Breaking the medical device regulatory process into component parts allows one to make improvements to individual parts, which ultimately improves the process in total.

My area of focus in this testimony is the Advisory Panel process in particular. After years of it being allowed to "drift" off course, compounded by extreme restrictions on panel qualifications and poor procedural orientation, it has become dysfunctional and counter-productive.

I don't believe it should be scrapped.

I do believe a few changes, reasonable and implementable, can significantly improve the appropriateness and fairness of FDA decisions following Advisory Panel recommendations.

Restoring fairness and competence to the process is important for many reasons. It's about maintaining our overwhelming US leadership in medical research and innovation. It's about US citizens being able to access the latest, most effective new treatments. It's about preserving venture capital's dominant role in financing daring new break-through treatments and keeping the critical funding pipeline flowing to companies. It's about adding, not reducing, US jobs in this exciting medical device field.

PROPOSED TESTIMONY

Good morning. I'm Kevin Larkin, CEO of TherOx, a venture capital supported, clinical stage medical device company located in Irvine, California. I appreciate the opportunity to testify before you today.

My profile and views are as follows:

- 37 years in cardiovascular medical devices including heart pacemakers, implantable defibrillators, angioplasty catheters, stents, and other devices treating heart disease.
- As a consumer and occasional patient I absolutely recognize the need for and support reasonable evaluation and regulation roles for FDA. These can facilitate but not foster innovation.
- My peers and physicians I have worked with over the years are overwhelmingly honest and ethical and will not knowingly do harm. Unless and until these values are violated, they should be treated as professionals with trust and respect. Violators should be weeded out.
- I believe FDA has huge challenges satisfying its diverse, heterogeneous constituents – public, physicians, industry, media and legislators. Most of these groups do not understand the risk-benefit aspect of medical treatments.
- Specific to the PMA regulatory path, the Advisory Panel process is dysfunctional and counter-productive. The good news is it can be fixed with a few tweaks.

TherOx is a non-revenue generating, clinical stage company that has pioneered a break-through heart attack treatment that minimizes heart damage compared to today's standard of care.

We successfully completed a controlled, randomized, pivotal trial that met both its effectiveness and safety endpoints with headroom. Our trial design was collaboratively worked out with our FDA review team, a group within CDRH.

After we submitted our trial data, our FDA review team, on its own initiative, awarded us Expedited Review, a classification that not only indicates pre-specified criteria were met, but also the new therapy treats a serious disease state for which there are no better alternatives. Despite this apparent success, an advisory panel did not vote to recommend approval of the therapy. Here are a few observations from the panel meeting:

- Physician composition – of 15 members, 3 routinely treat heart attack patients and therefore know current practices, logistics, and adverse event and mortality rates for this serious disease state. Our panel included a mix of surgeons, electrophysiologists, general cardiologists, as well as several interventional cardiologists (those who routinely treat heart attack patients).

- These physicians were ignorant, even dismissive of our Bayesian statistical design which was recommended by our FDA review team.

- Because they don't routinely treat heart attack patients they were unfamiliar with our heart damage measurement – Sestamibi Nuclear Imaging, a well validated heart assessment tool. They dismissed it.

- They were concerned about 1.8% death rate in the TherOx treatment group. They didn't understand the normal rate was >3% at the time. They didn't know.

- Based on questions and comments, several had obviously not read the panel reading materials as they should have.

- Panel members made clinically incorrect, misleading statements during the period when the company (sponsor) is supposed to remain silent.

- An untrained Advisory Panel Chair accepted a motion for approval without conditions – completely inappropriate for a full PMA product – a post approval study is always a condition for approval.

The panel vote was 9-5 not to recommend approval despite the trial having met its endpoints. Here is a summary of actions since the panel meeting:

- Jobs were lost. One of my first actions post panel was to fire half of our employees as a cash conservation maneuver while we attempted to work through an approval path with FDA. The employees "let go" had done their jobs effectively, helped us deliver a successful trial, and did not deserve this outcome.

- Since that panel meeting, heart attack patients, particularly those suffering large debilitating ones, have no access to our therapy.

- Physicians familiar with the trial and outcomes have been frustrated by not having access to another "tool" they need to more effectively treat patients, particularly those suffering from large, debilitating heart attacks that lead to cascading, worsening symptoms post recovery.

- The Advisory Panel process is being tweaked slightly but for the most part operates as it has in the past.

- Venture Capital, by far the dominant financing resource for innovative, break-through medical devices, is increasingly abandoning investments in PMA product research due to:
    - Uncertainty of FDA approval even when outcomes meet goals.
    - The panel conclusions often being inconsistent with FDA's position and dysfunctional as a process, negatively influencing the result.

No doubt many of you here today have heard a variety of criticisms about FDA policies, personnel, arbitrariness, follow-up, and more. Unlike many of my peers who run small medical device companies, I have high regard for most of our particular CDRH review team. They are collaborative, helpful yet firm, and importantly – responsive. I appreciate that we work together and how each of us tries to work toward deliverables. As you may have assumed by now, my single largest criticism is with the Advisory Panel.

The good news is I believe the Advisory Panel function is one that can be monumentally improved with a few changes, all fairly obvious and easy to implement.

Here are a few recommendations:

- Reconcile the ridiculously extreme conflict-of-interest restriction that increasingly staffs advisory panels with non-specialists in the field being evaluated. Strike a balance in accessing needed expertise with reasonable, common sense restrictions on conflicts of interest.
- Require Advisory Panel members to pre-read the prepared panel materials and attest to having done so. Disqualification from panel duty is the consequence for failure to have read it.

- If FDA adopts and recommends trial design efficiencies to a company (Bayesian statistics, Sestamibi Imaging, 3:1 uneven randomization, etc.) panel members must be educated, oriented to such concepts or not be considered "qualified".

- Panel members making false, misleading statements must be corrected immediately by either FDA or the sponsor to prevent negatively influencing other members.

- Instruct panel members to evaluate the trial and results being presented, not re-design it after the fact.

- Train panel Chair as well as members on panel logistics, voting procedure, risk-benefit interpretation, and other issues critical to effective panel function.

Having gone through a "good example of a bad example" I believe a few changes like these can preserve the important independent advisory function intended for panels yet dramatically improve their effectiveness and assistance in FDA's decisions to approve treatments or not, and under what conditions.

This is about maintaining our overwhelming US leadership in medical research and innovation. It's about US citizens being able to access the latest, most effective new treatments. It's about preserving venture capital's dominant role in financing daring new break-through treatments. It's about adding, not reducing, US jobs in this exciting medical device field.

I appreciate having had the opportunity to testify before you today. I also offer to be of assistance as you act on improving parts of the regulatory approval process.

Mr. BURGESS. I want to thank all of the panelists for their testimony this morning. It certainly has been enlightening.

It is usually our custom to go to questions from the Members of Congress at this point. We usually start with the person who has traveled the farthest and has the most seniority.

[Laughter.]

If it is all right with you, Mr. Bilbray, I will go first.

I recognize myself for 5 minutes for the purpose of questions.

Mr. Larkin, I have heard it from others on the panel as well, this issue of the advisory panels. And I have to tell you, in June of 2007, when we did the reauthorization for the FDA, in particular the prescription drug user fee, the medical device user fee, and it was the first time I saw the language that was going to be restrictive that said, no one with any potential for conflict of interest was going to be seated on the panel.

The Institute of Medicine, and I recognize there always are some disagreements with the Institute of Medicine, but they themselves said no more than 40 percent should have some identifiable conflicts. But they recognized the utility of having people on the panel who actually understood the drug or device under question.

And when we had that conveyed in the committee, we had the markup on the bill, and I put up an amendment out there twice, once in subcommittee and then a full committee, and it was always voted down on a party-line vote. So clearly, there were political overtones as to why you mustn't have any type of conflict of interest on the panel.

But particularly in a very small universe, I was thinking at the time about pediatric oncology; certainly, your area fits that bill. But in a very small universe, the number of people who actually know something is going to be a pretty small number of people. And if you exclude all of them, then you are knocking all the experts off of the panel before you started. You might as well have OB–GYNs on the panel as pediatric oncologists.

So I certainly welcome the fact that I have heard I think from several of you today, and I promise you this is something I am going to get fixed when we reauthorize both the prescription drug user fee on the medical device user fee in this Congress.

It was wrong the way it was approached last time. I was young, well, younger. I didn't appreciate how things worked. It was my first term on the committee in the minority. And I didn't really understand how things were working, when the chairman, then-Chairman Dingell, offered to work with me and help me get this fixed, it turned out to be pretty much an empty promise. This time, we will get this done.

Do any of you have any things you would like to offer on the conflict of interest stuff, because that is a fight that I intend to continue into this year?

Yes, Doctor?

Mr. GOLLAHER. We have talked to the FDA, including the commissioner, about this. And I think the FDA themselves support significant changes. So you are not completely swimming upstream. They realize that there are weaknesses that they can bring to bear, both on the drug and device side.

We talked with the commissioner about transparency. And the rule, generally, in looking at IRBs, for example, institutional review boards, within clinical medicine is conflicts are unavoidable. So clear illumination, as was said before, about the nature of the conflict is essential.

But I think what we have seen is going very far the wrong way to try to exclude them, and that causes more harm than good.

Mr. LARKIN. I think, sir, David is correct. The pendulum, to use that expression again, has swung too far.

To me, it is sort of a cascade. You know the media gets the public stirred up about not necessarily a real problem but a whiff of a problem, a potential drug or device related problem that has yet to be demonstrated. And then once that is going, the public gets upset about it, rightfully. And then, I have to say, sometimes a few legislators kind of join that bandwagon. And at that point, it is very hard for someone to stand up and say, wait a second, let's have a commonsense, practical approach to conflict of interest.

It is as if, at that point, the public has said nobody who has anything to do with this specialty can be involved in this decision. And of course, that is not right.

The more you know about the field, the more you know about devices or new approaches, particularly the more important you are as an element in that decision. I think you can be vetted, but I think we have to get back to some more reasonable—and the other thing I would say is, my point about believing that the vast majority of physicians and professionals in the medical space are honest and ethical is important. As soon as someone violates that, you kick them out.

Mr. BURGESS. Yes, and that is critical.

Mr. Panetta, you talked about the cutting-edge stuff now moving and instead of it being United States-oriented, it would be oriented overseas in Europe or perhaps even Asia. Right before his death, I had an opportunity to meet Dr. DeBakey. He got the congressional gold coin, and we spent an afternoon talking. And he reminded me that in the 1930s, when he graduated from medical school, that he was immediately required to go to Europe to get the credential to be a researcher, because he wanted to do research, and you couldn't be a researcher of any renown if only trained in the United States.

So he was a graduate of medical school, I think the year Elvis was born. And then things all changed after the Second World War. He attributes a lot of it to the funding of the National Institutes of Health, but there are obviously other reasons as well.

But it seems like such a shame to undo that now, as we are poised to do. But that is obviously something that concerns you, as well.

Mr. PANETTA. Absolutely, Congressman.

It used to be, too, that the majority of drugs were developed in Europe. And we saw, fortunately, the pendulum shifting as drug companies came to the U.S. to take advantage of the work that is done by the NIH, to take advantage of the skilled workforce here, and certainly to take advantage of the reimbursement that is supported here that doesn't come anywhere close in Europe to what companies are able to ——

Mr. BURGESS. And the academic community, we must mention them, since we are in their facility today.

Mr. PANETTA. Absolutely. And here in San Diego, if it weren't for the academic community, we wouldn't have a biotechnology industry. It came out of the academic community.

Just to get back to the advisory panel situation, the one comment I would add, if I could, on that situation is that many of my members tell me that there is definitely a disconnect between the advisory panels and a lot of the work that is done leading to advisory panel meetings, in terms of an understanding of the earlier work that has been done. And we have had several companies here that have gone all the way through the process and invested hundreds of millions of dollars only to be redirected back to something that came up as an issue early on in the process and could have easily been tackled then. And this creates even more delay and uncertainty at the end of the process. So there is definitely a discontinuity there.

Mr. BURGESS. Thank you.

Mr. Bilbray will be recognized for 5 minutes.

Mr. BILBRAY. Yes, Joe, let me follow up on that. So you are talking about the staffers who review the original applications are not available as resources for the review bodies?

Mr. PANETTA. Many times, Congressman Bilbray, the staffers are no longer there, because, as I said, there is a 3-year average turnover. Many times the products are reassigned. Staffers move from one division to another. And many times the record is just not clear within the agency as to decisions that were made early on, and there is no consistency in that decisionmaking.

Mr. BILBRAY. So we have a real problem with institutional memory starting with that nobody can follow this through. I mean, how many people know the frustration, a good example, is you go to get your passport and somebody says oh, yes, you do this and this. You go back to the same window or you go back for your birth certificate, and they say, well, I wasn't here, I didn't tell you that. And I have a new set of rules.

So I think this institutional memory, I just can picture, the fact of, coming from local government, of a planning commission not being able to have as a resource the staffer who actually processed the application. I mean, that is a huge resource there. And so, you are literally flying blind.

Kevin, my question, though, is this conflict issue, it really is broad. I mean, it is almost going to a concept—and I guess it is something that I would ask both sides of the aisle to perceive—this would be like saying that you wouldn't allow any school board member to be either a parent with a child in the school system or to be a teacher. You know how that would be received around this country.

But that conflict I think is a real concern I have, because it is almost as if that there is a willingness to accept ignorance to avoid any appearance of prejudice. And I guess that is where we get into it on that. It is a big concern that you can't sanitize the system to the point where nothing can grow. And, hopefully, we will be able to address that kind of conflict.

Let me just open up on one thing here. My concern is that, anybody here, if we do nothing, if we don't address the issue of the desperate need for venture capital for research, if we don't change the system where we allow experts—and let me just say a moment about that. You think cardiovascular, you know, you have a pretty broad perspective with some of this kind of stuff that goes on. I mean, I think we can talk about—what about gene therapy?

If you don't allow somebody was some expertise in the field, literally, you are flying blind. You would rather say, why even have a review body if they are going to be basically blind to the whole facts and science out there?

But let me just open up, if we do nothing at all, if we keep on the projection that we are looking now, you know how guys love to do their scales. OK, here is our projection, here is where we are going, where are we in 10 years?

Mr. GOLLAHER. Sir, just one way to think about this is the concept of regulatory competition. The FDA doesn't view itself as having international competitors, but it does.

In fact, the Europeans have designed their drug and their device regulatory systems with the explicit intention of building up their industries. And so it is really the opposite of the way that we have thought traditionally about regulation.

Mr. BILBRAY. So it sounds like they actually approach this like they did the shipbuilding industry, where they were going to participate in helping to get to that outcome, that they were going to be partners with the researchers and the private team to get to the outcome. Where we used an approach of, "That is your problem. We are just here to make sure you don't do something we don't want to do. We don't. We have no obligation to help you do the things we do want."

Mr. GOLLAHER. Well, it is interesting. If you look at the European system, and it is different for drugs than it is for devices. But in both cases, the regulatory system has a sponsor that relates to the company that is going for approval. It is called a rapporteur in the medicines agency, and it is called a notified body in the device industry.

But these are people who are basically working with the industry sponsor to shepherd the product approval through. And it is not an adversarial but rather a collaborative process.

Mr. BILBRAY. Doctor?

Mr. MENTO. Yes.

Mr. BILBRAY. I hear that we are looking at having our medical research facilities go the way of our shipbuilding. And it all ends up being basically something we used to talk about, where Americans used to be employed, used to being involved with that. If we don't change the system, is our medical research going the same way as our shipbuilding did?

Mr. MENTO. I think it is actually worse than that. I think, I mean you heard today, and the concept is that in small biotech companies that are willing to take the risk, so they are going to go out there with a first class drug. Well, the problem with first in class drugs are, there is no regulatory pathway.

You are talking about pathways that exist with endpoints. I am talking about drugs that we are developing in liver fibrosis; there are no endpoints. No one has ever done a drug in that space.

So when you think about uncertainty associated with trusting that the FDA is going to be capable enough to have continuity over a 10-year process it is going to take to develop a brand-new drug in a space that hasn't been developed before, it is incredibly difficult to bring money in. And if the money doesn't come in, you are going to have more—I mean, I have a distinguished member of the venture community next to me. But I can tell you that the people I talk to, they would rather have I have a second or third generation drug where they know the pathway is, where they can at least identify if it is the next antihypertensive, people know how to measure blood pressure. They don't know how to measure the progression of fibrosis in the liver in a way that will determine whether or not the clinical outcome for those patients is going to be better or worse.

And, in particular, in our case, the epidemic—everybody talks about obesity. We are talking about the epidemic of obesity, and one of the things that people are probably not aware of—you probably are, as a physician.

Mr. BILBRAY. He is aware of everything.

[Laughter.]

Mr. MENTO. The inflammation of liver associated with obesity is rampant and increasing, not just in the States but elsewhere. So it is not just that we are developing drugs for, say, in the U.S. elsewhere. The diseases that we have, because of the westernization of some of these other locations, are becoming more prominent there.

They are going to be first in line, because they are going to be able to have the scientists there, have the funding for that to treat the disease there. They may not even get to the United States.

So I think it is worse than the shipbuilding industry.

Mr. PANETTA. May I add to that, Congressman?

Mr. BURGESS. Of course.

Mr. PANETTA. Last year, I had I had the—Mr. Chairman, last year I had the opportunity to go to Taizhou in China, where the Chinese are building China Medical City.

China Medical City is a remarkable effort to begin with, because it is a ground-up—from the ground up effort to build research facilities, universities, incubators. But what impressed me the most was in the center of China Medical City was an SFDA building, a Chinese FDA building.

And when I asked about it, I was told this building is here so that the SFDA folks can work directly with the researchers and the folks in the incubators from beginning to end and make sure that there is collaboration in the development of products. What a unique concept.

Mr. BURGESS. The gentleman's time has expired. We are, obviously, going to go another round for questions.

Dr. Stevenson, I would like to ask you if I could, the concept of the shipbuilding having already left the country. Has the financing for the shipbuilding, has that ship also already sailed?

Ms. STEVENSON. It is hovering, I would say. I am not so sure sailed.

But the point about the fragile ecosystem I think is really, really important, because I can only invest in companies when I have a fund to invest. I get that from my limited partners, who are, as I mentioned, the pension funds. In our case, we have these small funds. A lot of ours are family offices and high-net-worth individuals.

Well, people are only—they are making those investments in order to get returns. So if I can't demonstrate consistently over time that they are getting a reasonable return, they are simply going to go elsewhere. I mean it is——

Mr. BURGESS. There are too many iPhones being made.

Ms. STEVENSON. Well, yes. Why not go where you are going to get a guaranteed return, as opposed to investing in innovation. Good to be a good citizen, but that is not why people are making these investments.

And there is a really big lag. You asked what would happen if it all kind of stopped today. Well, it takes a long time to go grow an idea. It takes a long time to grow the management team. I mean, nobody does life science management that hasn't been doing something allied for 15 or 20 years. Well, those people are going to go elsewhere.

I mean, I had like 20 years of college and operating experience and all this stuff before I started doing that. That is not unusual for a venture capitalist.

So the life sciences folks will go find other things to do. And it is very hard then to restart this ecosystem, once it has started to die off, like in any other ecosystem.

Mr. CASEY. And to put a point on that, you asked a question, Congressman. I mean, if things track the way they are today, the medical device industry will be a memory in the United States, point 1. Point 2 is patients are going to be paying a lot more for devices, and that money, just like oil, is going to go overseas to either China, South Korea, or over into Europe. And to be honest with you, the economy is going to have sacrificed, in our estimation, between 200,000 and 1 million jobs on literally looking at how you begin to foster an industry and the next generation of health-care information technology.

A lot of discussion here is a little bit more about bio and pharma. I will just tell you that if—you mention gene sequencing. When you look at wireless data, the prevalence of data is just going to explode in health care. And there is an entire new industry that is waiting to be formed on data analysis, data capture, data manipulation, over time.

And if we have a regulatory environment today that is basically saying, well, hang on a second. The minute you move off the device and a drug into something that combines information with a device, that creates a whole world of regulatory uncertainty. That industry is going to be picked up and moved.

I will tell you, currently today we are 3 years behind Japan. I would tell you we are about 18 months behind what we are seeing going on in Europe. And the Chinese who had no interest in that a year ago are now throwing around a ton of money looking at the space.

So it is an industry that should be American. It is needed for the economy; it is needed for jobs. And we are just going to watch that just sail away.

Mr. BURGESS. Dr. Stevenson, I know you have to leave.

Is there any parting advice that you want to give Mr. Bilbray and myself?

Ms. STEVENSON. Well, to use the analogy I used with our risk, manageable and unmanageable, to manage what is obvious here that needs to be managed, the conflict-of-interest issues.

We can live with an FDA that is hard. We want safe, effective products. That is what we are investing in. We just simply need to be able to see the process and then depend on that process. So when we make our forecast, when we make our investments, we get to where—we have forecasts. We can get on that amount of capital and that amount of effort.

That is really all anybody is asking for. And it existed at one point in time. And we believe it should exist again.

Mr. BURGESS. I agree with you.

I yield back to Mr. Bilbray.

Mr. BILBRAY. Yes, let me just say, Doctor, we don't talk enough about it, because it is not sexy. It is heart-wrenching. But thank you for representing the venture capital industry, because as those of us that have been involved in the environmental movement, you are the krill of medical miracles.

Ms. STEVENSON. Is that good or bad? I am not familiar with the term.

[Laughter.]

Mr. BILBRAY. The entire ecosystem depends on you being healthy.

Ms. STEVENSON. Oh, OK.

Mr. BILBRAY. But you are out of sight, out of mind.

Ms. STEVENSON. And sometimes maligned, I would also ——

Mr. BILBRAY. There was once a very bad movie that Charlton Heston did called "Soylent Green." And when the krill dies, we start eating each other. In other words, it is not sustainable without you.

And you are out of sight, out of mind. You let people take you for granted, and trying to get both sides to understand that we need that public research to create the sea grain for you. But we have got to allow you to get the capital to be able to create those startups that the big guys use to feed the system of the next generation.

And without you, I really worry that we are going to be asking ourselves in 10 years, where are all those great medical breakthroughs? Where are the things like a vaccine for cervical cancer, so the politicians can debate about should it be applied or not.

[Laughter.]

Those type of miracles I think we take for granted too much, and I think we are too jaded.

But you are the foundation on that, and I want to thank you for that.

Ms. STEVENSON. Thank you.

Mr. BILBRAY. My time has——

Mr. BURGESS. No, your time has not expired. You encroached on my time, which is perfectly fine.

Go ahead and reset the time. We will give it to Mr. Bilbray.

Mr. BILBRAY. I want to bring that up, because there is a lot of stuff like the medical device issue.

And, Don, my family went through the tragedy of crib death. And then went through the tragedy of living with an infant monitor with false alarms and everything else out there. A big concern is where we would have been if we didn't have that device and how if that wasn't made available—and, sure, there was a lot of false alarms. But let me tell you something, after you have to pick up a cold, dead baby, that false alarm doesn't bother you at all, especially when you could pick up a nice warm baby while you are terrified.

And I guess that is my frustration of taking a look at things like aspirin. Hundreds of people die every year in this country from aspirin. And I wait for the time of the FDA explaining to me how aspirin stays on the market and admitting because, probably of any drug out there, it has probably saved more lives than anything else.

But aspirin, as far as I know, do you think aspirin, anyone of you guys, do you think aspirin could get through the system today?

[A chorus of noes.]

Mr. BILBRAY. And how many people, how many thousands of Americans and people around the world, are alive today because of aspirin? So how many other aspirins are being held up in a system and how many other people are dying because we are not approving the proper triage on that?

And I would open it up. If there any specifics that you guys can see, the frustration you run into.

Mr. CASEY. Congressman, I want to pick up on your Sudden Infant Death Syndrome as illustrative of what is going to happen.

I mean, basically you are talking about a baby monitor that you can hear and whatnot. The technology is rapidly getting to the point where it is eminently affordable that you could put a device under a mattress that is going to basically measure whether the child is breathing with a pretty fair degree of precision. And then that can be either pushed to a doctor, a caregiver, a parent. It could get pushed anywhere, because the data is basically easily moved.

The challenge and the reason we are talking about de novo is, the minute you take a relatively straightforward device and put a wireless component to it that involves data transmission analytics, you enter into this very strange world where, well, hang on a second, are we actually looking at a device? Are we looking at a device plus an analytical system? And you get in this very strange do-loop.

And one of the reasons, and it is a shame that Dr. Stevenson isn't here, venture capital is walking away from that, saying, oh, hang on a second, that is wireless.

You know that is going to get into a bad place with the FDA. And you have totally ramped up the regulatory risks.

So the technology that should actually already be 10 years old is waiting. And just pick that or, for us, congestive heart failure. $10

million disease, in terms of what the government pays out. It is billed $30 billion, but it is a $10 billion disease. There are relatively straightforward wireless medical devices that can make a transformative difference that can really ramp down costs, increase patient satisfaction, and reduce readmissions.

Then again, it gets caught in this lack of predicate device, which is one of the reasons we have been pushing so hard on this de novo pathway.

Mr. MENTO. If I may add something in the context of a word that we heard—I don't know, I was going to count—probably 100 times: innovation.

Funny you brought up gene therapy. The first biotech company that I was recruited to in San Diego back in 1992 was a gene therapy company. And in those days, in the '90s, the investment in this industry was truly all about innovation. You heard from the venture community that limited partners were flowing in there because there was the perception that the science was going to dictate and lead the way. And when we started in gene therapy, the FDA not only had no one at the FDA that knew anything about gene therapy, because it was such a new field, but there wasn't that fear that there wasn't going to be a pathway that could be generated, because the feeling was that science was going to win out, both in the companies to develop innovative products, and through the FDA, that ultimately the process would be defined well enough that that the venture community would not go away.

That is completely the opposite now. I mean, there is a fear, an absolute fear, in innovation, and you heard a bit of it here, in the venture community, not so much the science isn't good, but there is no predictability and no trust that a pathway will ever be developed.

And even when you have a job that goes all the way through the process, you have had your agreements, you add the issue we had talked about relative to the scientific advisory committee meeting, and not having people on there that are even capable of understanding the process. We are in dire straits.

If it doesn't get fixed, we may not see its 4 or 5 years, I am more concerned not about the drugs that are currently in phase 3. It is the pipeline that is not going to be there 5 or 6 years from now, where you are not going to have to worry about regulating innovative drugs, because they are not going to be any there, because there wasn't any financing.

And it is not just the venture community. Look at the large pharmaceutical companies. They are eliminating their R&D organizations, because they can't even manage or feel comfortable in managing if that is a good investment on our part.

I mean, shipbuilding is close, but I don't think not even close to being as dire, what we are in for in the future.

And when I talk to my colleagues within the industry, there is depression, because the science is better now. It is better than it was 20 years ago. We can develop—it is remarkable the kinds of things that we can do. And you know, with electronics, also with gene sequencing and marker analysis.

But there is no way that we can convince investors that there will be a pathway, that the FDA is going to get up to speed, and

that even if they are that that pathway is going to be predictable enough with a trust level that if it really does meet its endpoints, it is actually going to get through, and not be stifled by some people that are in the process at the end-stage that don't even know what they're talking about.

So I don't know how you fix that.

Mr. BILBRAY. OK, Doctor, you raise a whole new concern that we need to follow up on and get into. And in all fairness, you are right. It took decades to lose the shipbuilding or the auto industry. It will take months to lose the research facilities.

But the prejudice, the prejudice of people that claim to be based in science but bringing prejudice in, and the gene issue is a good example, where a top oncologist here at U.C. tells me flat out, we are this close, this close. If there was any opportunity, this close to the breakthrough, but it has been stopped dead in its tracks.

And resources and everything else are moving off, and to see somebody whose entire life is devoted to trying to save life, a frustration of seeing a system that basically takes prejudice, and discriminates against a certain scientific approach, based on ignorance, and to claim they are somehow being informed decision-makers just I think is one of those things that we need to talk more about and look at. And that is one thing our committee has to look at is the prejudice.

We just got this big scandal that is coming down that the doctor and I are working on of a certain kind of energy technology. And the prejudice led them to major mistakes that cost the taxpayers a half billion dollars.

The trouble is, with your field, we are seeing bureaucratic prejudices that are standing in the way of not just the creation of jobs, but the saving of lives. And that sure makes what went on up in San Francisco look second rate.

Mr. BURGESS. Let's go each one last round.

Mr. Panetta, I wanted to ask you a question. You referenced the period of exclusivity. And I thought, for all the bad stuff that is in the Affordable Care Act, I thought at least the period of exclusivity had been put to rest. It seemed to be pretty bipartisan, bicameral support for the 12 years that was I think included in the amendment that passed through our committee. And I think similar language was taken up by the Senate.

Then the President the other day said we are going to roll that back, I think he said to 7 years.

Mr. PANETTA. Seven years, correct. And if you recall, Congresswoman Eshoo took the President on directly on this issue as the Affordable Care Act was in the process of being passed. And this was probably a good year and a half ago that the President proposed to reduce it to 7 years period. We thought that had been put to rest, but of course it is in his latest deficit reduction plan again.

Mr. BURGESS. Yes, I mean, it almost defies gravity that you are going to be able to recoup the investment cost in such a short period of time and have a product on the market that anybody can actually afford. Otherwise, your price is so high that it becomes something that is not attainable.

Mr. PANETTA. Absolutely, and there was plenty of economics behind the proposal for 12 years. Of course, it was debated by the En-

ergy and Commerce Committee, and we were able to back up the need for 12 years.

It provided some sense of confidence and security to the investment community. And to propose to move it back to 7 years brings that insecurity back into the equation.

Mr. BURGESS. And just so you can take some comfort, I don't see any abiding interest from either side of the political dais, and in our committee, to reengage that fight. There was one person who was pushing it, as I recall, and the vote was literally that lopsided in July of 2009, when that came up in committee.

Mr. Casey, on the novel device aspect, you have obviously referenced one that sounded pretty important to me. I assume there are others out there in the pipeline that you all are looking at.

Mr. CASEY. There are a ton. I mean, if you look at this space again, we commit to things as to how do we dramatically lower the cost of health care by creating infrastructure independent, or how do we create monitoring that to accelerate the development of novel technologies.

If you look at the combined weight of like an Apple or Verizon, AT&T, they all want to jump in the health-care space, but then they jump and they look and say, oh my God, there is an uncertain regulatory body there that actually is actively discouraging innovation by virtue of not investing in understanding the science and not creating a pathway.

We just see a significant amount of problems where we——

Mr. BURGESS. Now would Google be subject to that 2.9 percent medical device tax on the iPhone?

Mr. CASEY. No, it is very interesting. Apple, as an example, for which there are now over 13,000 medical apps, you actually sign a release if you are going to develop a medical app with Apple that declares that you will not make the Apple iPhone a medical device, as a way of shielding them from the 2.9 percent tax.

And it is interesting that one of the most innovative companies in the world is so concerned about being regulated that they just basically sit there and put up a big wall.

But if you look at the five most costly diseases—and I am talking to a physician. I don't mean to—but if you look at congestive heart failure, if you look at COPD, all of these, you run down asthma, you run down pregnancy, in terms of cost per individual that has that. These are all conditions that there are devices that can be made to transmit well-understood biometrics from point A to point B, and create—Congressman Bilbray, whether it is a false alarm or any kind of alarm, just move data and get it into a rich data analytic field, where you can learn long term what is the actual pathology of the disease and other things.

But if you look at the amount of money that is being spent on wireless devices in COPD right now, it is nonexistent. Congestive heart failure, a little bit.

Mr. BURGESS. Yes, are forced expiratory volume transmitted wirelessly on a daily basis to predict the compliance——

Mr. CASEY. Lack of compliance.

Mr. BURGESS. Or lack of compliance. Same with the daily weight on a CHF patient. Relatively low-tech ideas that go high-tech on the wireless side. But it could just be a game-changer, as far as the

practice of everyday clinical medicine, where you are able to anticipate your patient's problems.

One of the things I liked about that was you can set that so that the investigation is done at 2 o'clock in the afternoon, not in 2 o'clock in the morning, which is when we typically see the patient come in to the emergency room, because they have been worried because all day they have had this pain or this lack of activity, or something that has gotten their attention. So the ability to manage your clinical practice becomes huge.

Mr. CASEY. And you set the alarms. If you say, I have a diabetic obese—gestational diabetes obese patient, I actually might want to see more data than less. Where if I see somebody who you think is relatively low risk, you set the parameters, because this is the revolution that we are talking about.

And the fact that the country invested so much money in creating basically ubiquitous communication technology, and we are not leveraging it against the 20 percent of spending that is called health care I think is really kind of of a national——

Mr. BURGESS. Maybe the critical point was——

Mr. BILBRAY. Doctor——

Mr. BURGESS. Wait, wait, I just have one cautionary tale.

Three or 4 years ago, I went out to Seattle with Newt Gingrich, who was giving a talk out there. And he asked me to talk on electronic health records. And we got the tour through Microsoft, and all of the smart people at Microsoft were there with us. And we went through the Microsoft house. The house is named Grace. You walk up to the door and Grace recognizes you. You don't have to activate Grace. She recognizes that you are coming up.

She turns on your favorite light, warms up your favorite chair, all this stuff. You go into the kitchen and Grace has some menu suggestions for you. And Grace might know if you are a diabetic or prediabetic.

The thing that concerned me about that house was if the refrigerator talked to the bathroom scale, it might put the ice cream on lockdown for that night.

[Laughter.]

That is one of the things that—the cautionary tale I would have about wireless run amok.

Mr. Bilbray?

Mr. BILBRAY. I almost was ready to make a marriage proposal to Grace. By the time——

[Laughter.]

Until she cut off the ice cream.

Speaking of that is how in government we can weave these webs that come back to get us.

As far as I know, there is a mandate in this new health-care bill of the private sector has to go to electronic recordkeeping and data processing.

But is there any protection against the tax that is in the bill of coming down on that technology that is mandated?

Mr. CASEY. No protection.

Mr. BILBRAY. So while they are mandated on one side, and, oh, by the way, veterans and military have been trying to do this for 10 years. We are going to have the private sector do it in 4.

But now they say they want to do it, but at the same time basically have this cloud hanging over that there may be this big tax on you. If you do what the Federal Government wants, we are going to be coming hard with a big heavy tax. Is that correct?

Mr. CASEY. It is circular logic. And I will just push the EMR debate. We are not huge advocates of EMRs per se, but even EMRs, there is a carveout with the FDA.

Now the FDA is saying, well, excuse me, EMRs have a carveout, but if you put in your clinical decision support algorithm in your EMR, well, that is now under review. And then if you put in your medical device collection capacity in your EMR, now that is going to be regulated.

So the interesting issue is rather than increasing the functionality of this data-driven electronic medical record world that people want to live in, the FDA is now encroaching on what was something Congress had actually stepped up and said there should be a carveout to protect that, to get the innovation we actually need to change health-care paradigms.

Mr. BILBRAY. So maybe we want to start by, basically, getting both sides to agree that we will not implement the device if it falls into the category of a mandate for the data.

Mr. CASEY. That's a good start. A very wise Congressman I thought introduced something about that.

Mr. BILBRAY. Let me just say, we are about—do you have any more questions?

Mr. BURGESS. No, I have some things that I must do at the end, so go ahead with your closing observations.

Mr. BILBRAY. OK.

You now, let me just make an observation as a local, and I want to thank the chairman for being here, and I want to thank the witnesses.

I think the American people are looking around and saying, why can't Washington work in a bipartisan effort? And I apologize, both sides of the committee wanted to see this happen, and, hopefully, they will be able to review this. And I appreciate the fact of the bipartisan effort.

This is the kind of thing that Americans really want to see a bipartisan effort on. The fact is, being from San Diego, it is sort of interesting that today we have had two visitors that have come. One everybody knows it is visiting. Another most people in this county of 3 million-plus won't even notice it. One from the West and from the ocean, and the other one from the East from the sky.

And those of you when you leave here today, you can look out, you can see some very blue water. But then you can see some very ugly red water out there. And it is a thing called red tide.

And the red tide to me is really a great analogy for what we have seen happen in the FDA. Red tide is something that goes into an environment and, basically, makes a very healthy environment toxic. Red tide will poison the basic building blocks of life in the ocean, and that is what we have out here.

I see that as being an analogy for what we are seeing happen with FDA, that people will wonder why the environment has become so, so toxic for innovation and not just job creation but health creation. And we need to do something about it to make sure that

red tide moves off into another neighborhood and leaves the research facilities alone, so you guys can grow and feed the system that we depend on so much.

The other visitor was the President has come to visit us today, and for a lot of reasons, but I think that one that I hope that somebody talks to the President about here is how important medical research is for the creation of jobs in San Diego, that California has been historically the great incubator of medical breakthroughs.

And I hope the President is confronted with the fact that, do not take us for granted here. Everybody in Washington talks about innovation, research, you know, the great potential that this is the backbone of America's economic and social future. And I hope somebody goes over there and reminds the President that he is in a city that desperately needs him to take a lead. Because everything we talked about today can be done tomorrow by the executive branch. They can make the institutional and cultural changes in FDA to make this possible.

But let me just say this, I hope that there is a possibility that maybe some young lady can walk up to the President and not say do this for me, because we all talk about what—Bill Walton says he was a benefactor and he was somebody that benefited by this kind of research and this kind of medical device opportunity.

But I will tell you something, more important than a young lady walking up and saying do this for me, would be a young lady walking with her child and say, the only thing worse than a Bill Walton or somebody and somebody in pain and suffering and needing medical breakthroughs is watching a child. That is a challenge that I wish that President could be confronted with. "Mr. President, if you don't do it for me, do it for my children and do it for my grandchildren."

And so, hopefully, all of us can kind of leave here and say, our grandchildren won't remember if we are Democrat or Republican, but we are going to remember did we save the industry that could have saved their lives? Did we rise to the challenge to not only make sure we have a strong economy, but that we have healthy grandchildren?

And thank you very much for being here today.

Mr. BURGESS. And I think that concludes our testimony and time for questions.

I do want to thank all of you for being with us today. It has been an informative discussion. It is the committee's practice to allow members to submit written questions to witnesses. And I would ask that you would reply to those questions, if offered.

And again, I want to thank you for the time, the time you took in preparing the testimony, the time you took coming here to testify to the committee.

Remind the members that they should submit their questions to the committee clerk by Monday, October 10.

And with that, I will adjourn the subcommittee. Thank you all.

[Whereupon, at 1:17 p.m., the subcommittee was adjourned.]

[Material submitted for inclusion in the record follows:]

Henry A Waxman

Statement for the Record
Rep. Henry A. Waxman
Ranking Member, Committee on Energy and Commerce
Subcommittee on Health Field Hearing
"Impact of Medical Device and Drug Regulation on Innovation, Jobs and Patients: A Local Perspective"
September 26, 2011

In light of Mr. Panetta's reference in his testimony to a statement of mine regarding FDA, I submit the attached letter to clarify the record.

Congress of the United States
Washington, DC 20515

August 4, 2010

The Honorable Kathleen Sebelius
Secretary
U.S. Department of Health and Human Services
200 Independence Ave, SW
Washington, DC 20201

Dear Madam Secretary:

The demands placed on the Food and Drug Administration (FDA) in recent years have never been greater. Continuing innovation has led to a growth in both volume and complexity of FDA regulated products. Advances in the science of early detection of risky products have made available new ways for FDA to prevent problems from occurring. Globalization has prompted an upsurge in the number of imported FDA-regulated products. Congress has also ordered FDA to take on new responsibilities—over 125 new statutory requirements in the past two decades alone.

There have been several high-profile illustrations of the fact that the FDA is not always able to successfully meet these demands. The heparin contamination and the many food safety outbreaks are some of the more notorious examples. Unfortunately, FDA has become an agency that has been reduced to "management by crisis," continually forced to address the most recent crisis instead of preventing the next one.

There is no doubt that the chronic lack of resources at FDA has been a primary cause of these regulatory failures. A series of expert reports from the likes of the Government Accountability Office, the Institute of Medicine, and even the FDA's own Science Board, have documented the serious funding deficits facing the FDA. These experts all agree that the FDA's current resource levels are wholly inadequate to allow the Agency to keep pace with the ever-increasing number of demands on the Agency. After years of inadequate budgets, FDA finally received real increases in fiscal years 2008, 2009, and 2010, which enabled FDA to restore the staffing losses incurred since the early 1990s. However the FY 2011 budget for FDA barely kept up with inflation, and the agency is faced with far more problems and challenges than it had in 1994.

We recognize that there is little that you, as Secretary, can do to stem the influx of demands placed on the Agency.

There is, however, much that you can do to strengthen the Agency so that it is prepared to meet these challenges: it is incumbent on you to request—and to demand—that FDA receive the resources it needs to do its job and to do it well. Congress needs to hear from you, as the leader of this critically important Agency, what the FDA requires to allow it to protect American consumers from unsafe foods, drugs, and medical devices.

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We have seen in recent months how regulatory failures can be catastrophic, both in impacts on our citizens and economic costs to our nation. Three recent foodborne disease outbreaks alone, for example, have sickened or killed scores of our citizens and been estimated to cost over a billion dollars. These are graphic illustrations of what will continue to happen if we do not give FDA the resources to set modern food standards and inspect the facilities that manufacture our food. We also are concerned that such problems could cause the public to view FDA as a failed agency – not because the agency isn't doing its best, but because FDA simply doesn't have the resources to be fully effective.

Therefore, we urge you to ensure that your FY 2012 budget request will reflect the urgency of the findings of our Nation's experts by setting forth a funding level that will allow the Agency to fulfill its vitally important public health mission. Your team at FDA is demonstrating a strong commitment to public health, but we must support them with the necessary resources to be successful.

Sincerely,

Henry A. Waxman
Chairman
House Committee on Energy
and Commerce

Tom Harkin
Chairman
Senate Committee on Health,
Education, Labor & Pensions

John D. Dingell
Chairman Emeritus
House Committee on Energy
and Commerce

Frank Pallone
Chairman
Subcommittee on Health
House Committee on Energy
and Commerce

Bart Stupak
Chairman
Subcommittee on Oversight
and Investigations
House Committee on Energy
and Commerce

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