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A PRESCRIPTION FOR SAVINGS: REDUCING DRUG COSTS TO MEDICARE

HEARING

BEFORE THE

SPECIAL COMMITTEE ON AGING

UNITED STATES SENATE

ONE HUNDRED TWELFTH CONGRESS

FIRST SESSION

WASHINGTON, DC

JULY 21, 2011

Serial No. 112–7

Printed for the use of the Special Committee on Aging

Available via the World Wide Web: http://www.fdsys.gov

U.S. GOVERNMENT PRINTING OFFICE

68–441 PDF WASHINGTON : 2011

For sale by the Superintendent of Documents, U.S. Government Printing Office
Internet: bookstore.gpo.gov Phone: toll free (866) 512–1800; DC area (202) 512–1800
Fax: (202) 512–2104 Mail: Stop IDCC, Washington, DC 20402–0001

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A PRESCRIPTION FOR SAVINGS: REDUCING DRUG COSTS TO MEDICARE

THURSDAY, JULY 21, 2011

U.S. SENATE,
SPECIAL COMMITTEE ON AGING,
Washington, DC.

The Committee met, pursuant to notice, at 2:08 p.m. in Room SD–106, Dirksen Senate Office Building, Hon. Herb Kohl, Chairman of the Committee, presiding.

Present: Senators Kohl [presiding], Whitehouse, Udall, Manchin, Blumenthal, Corker, and Kirk.

Also Present: Senator Brown.

OPENING STATEMENT OF SENATOR HERB KOHL, CHAIRMAN

The CHAIRMAN. Good afternoon. This hearing will come to order, and we thank you all for being here.

As we all know, rising health care costs are threatening our economy. While the health care reform bill of last year was a start, it certainly has not done enough to address costs at this point. We need to do more, and we need to look at every option as we seek to provide quality care for all Americans at a cost that we can afford.

According to testimony provided by the Special Committee on Aging by the Organization for Economic Cooperation and Development, OECD, in 2009 the average price of pharmaceutical drugs in the U.S. was 30 percent higher than in the other 30 OECD countries. These are the most advanced and developed countries.

Another study found, the McKinsey study, that the difference in price may actually be as high as 50 percent between what we charge for pharmaceutical drugs here in this country versus those other 29 countries.

As I'm sure we can all agree and understand, rising health care costs are hurting America's global competitiveness and are a drag on family wages as potential increases have been used to pay for the rising costs of health care and prescription drugs instead of augmenting the wages of our working families.

In 2010, the American people spent more than $300 billion on prescription drugs, and a third of that was paid for by Medicare and Medicaid. Left unchecked, these costs threaten our country, our economy, and every American family, and we all, I think, would agree that this kind of a condition is not acceptable.

Today's hearing will focus on one aspect of health costs, namely prescription drugs, and provide an opportunity to talk about possible solutions. The committee will also release an investigative re-

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port that indicates that drug companies charge American consumers more because we lack the negotiating power used by other countries.

We already have prescription drug programs in place which do cut costs through negotiation, including the Veteran's Administration and a program in Wisconsin called Senior Care, and we should look, I believe, to emulate those examples.

The 91,000 beneficiaries enrolled in Senior Care in my state cost the Federal government a third of what it would cost for them to be enrolled in Medicare Part D with the same benefits.

By negotiating prices, Senior Care in Wisconsin did save my constituents $80 million in 2010. The VA demands a minimum discount of 24 percent on wholesale drug prices. If Medicare were able to save 24 percent, taxpayers would then save more than $350 billion over 10 years.

We also need to look at giving the government the ability to address sizeable price differences between drugs that are similarly effective. The National Institutes of Health recently sponsored a lengthy comparative clinical trial between two highly effective drugs used to treat macular degeneration, a condition that often causes blindness among seniors. The trial found that both drugs worked equally well in treating this condition. However, one cost $2,000 a dose, while the other cost $50. So we will be hearing testimony today about these two drugs on which Medicare is spending more than a billion dollars a year.

Today we'll be releasing a number of additional cost savings policy options suggested by experts to hugely reduce prescription drug costs. Some of these options would save billions, while others would be more modest. These options include ways to increase transparency and expand discount programs and reduce the financial incentives for doctors to prescribe the most unnecessary or expensive drugs.

This morning, the Judiciary Committee passed on one of these bipartisan proposals which would limit delays in getting generic prescription drugs to consumers. Several of our witnesses will discuss how these and other policies result in lower costs without sacrificing access, choice, or quality of care. I urge my colleagues to be open to considering all of these ideas, and I hope that together we can put additional solutions on the table.

We thank you all again for being here today.

And now we turn to the ranking member of this committee, Senator Bob Corker.

STATEMENT OF SENATOR BOB CORKER

Senator CORKER. Mr. Chairman, thank you for having the hearing, and I want to thank all of our witnesses who are here. I know that we have two panels. We had expected actually numbers of Senators on both sides of the aisle, and in order that we not have a lot of long, drawn-out opening comments, I'd rather hear from the witnesses. I'm not going to make an opening statement.

I will say that I think all of us are concerned about the cost of prescription drugs. Obviously, we may have differing views as to how to solve those, but I think that's the purpose of our hearing

today. I look forward to hearing the witness testimony and thank the chairman for calling the hearing.

The CHAIRMAN. I thank you very much, Senator Corker.

Senator Kirk, do you have a comment or two to make?

Senator KIRK. I do not, Mr. Chairman. I'll be brief, and let's go.

The CHAIRMAN. Thank you very much.

So now we turn to Panel 1 and our one witness. He is Jonathan Blum, the Deputy Administrator and Director of Medicare at the Centers for Medicare and Medicaid Services. Mr. Blum previously served at the Office of Management and Budget, and for the Senate Finance Committee. Mr. Blum is also the former vice president of Avalere Health.

We welcome you back, and we look forward to your testimony. Go right ahead.

STATEMENT OF JONATHAN BLUM, DEPUTY ADMINISTRATOR AND DIRECTOR, CENTER FOR MEDICARE, CENTERS FOR MEDICARE AND MEDICAID SERVICES, U.S. DEPARTMENT OF HEALTH AND HUMAN SERVICES, WASHINGTON, DC

Mr. BLUM. Chairman Kohl, Ranking Member Corker, Senator Kirk, thank you for the opportunity to talk about Medicare's payments for prescription drugs.

All four parts of the Medicare program, Part A, Part B, Part C, and Part D, pay for drugs in some form or fashion. All use different payment systems under different statutory authorities and frameworks.

I'd like to focus today on payments for drugs under our Part B and Part D payment systems, the two payment streams that receive the most policy attention.

All of our payment systems for drugs are similar in one respect. The Medicare program does not reimburse drug manufacturers directly for drugs provided to Medicare beneficiaries. Instead, Medicare pays physicians, hospitals, dialysis facilities, and insurance plans, who in turn purchase drugs or pay a pharmacist for drugs provided to Medicare beneficiaries. That is, we have no direct payment relationship with drug manufacturers.

Under Medicare Part B, the most common payment for drugs is to physicians who provide drugs to their patients. The program also pays outpatient hospital departments for drugs provided during outpatient procedures such as chemotherapy drugs, and dialysis facilities for drugs provided in the context of dialysis care.

The Congress has authorized the Part B program to pay for only certain drugs through Part B. These drugs include drugs administered by a physician or under the supervision of a physician; drugs provided through durable medical equipment such as nebulizers or IV pumps; and drugs that are directed by statute. These include certain drugs provided to dialysis patients, oral cancer drugs, and certain vaccines.

The Part B program covers about 800 drugs total that fall under these three categories. In 2010, CMS spent $12.5 billion for Part B-covered drugs, and the CMS actuaries project that total spending for these drugs will double over the next 10 years.

Today's spending for Part B drugs is highly concentrated in a relatively few number of drugs. Thirteen drugs account for half of the

total spending. About $6.25 billion is comprised of 13 drugs, and the top spending drug is Lucentis, that accounted for 16 percent of total Part B drug spending.

Congress reformed the payment system for these drugs in 2005, or most of these drugs in 2005. Prior to 2005, Medicare's payments for these drugs were based on the so-called average wholesale price, or the sticker price. There were numerous studies finding that payments to physicians under this pricing system far exceeded physicians' own costs to purchase the drugs. This created a payment spread for physicians.

Congress changed the system in 2005. CMS now uses a system based upon the average sales price, or ASP. The ASP is the average of each manufacturer's sales price net of most discounts and rebates and other price concessions. The ASP accounts for most sales from manufacturers to entities in the U.S. who purchase the drug from the manufacturer.

CMS, for the Medicare Part B program, pays physicians who administer these drugs a payment of ASP plus 6 percent.

The Part D prescription drug program works somewhat differently. Private insurance plans compete to provide outpatient drug coverage to beneficiaries who choose to participate in the Part D drug program. CMS contracts with hundreds of drug plans which must meet program requirements.

Virtually all Part D plans build their own drug formularies or lists of preferred and non-preferred drugs. CMS must approve plan formularies, and plans must cover at least two drugs in each therapeutic class, and the formularies must be deemed by CMS not to discriminate.

Today, Part D plans cover more than 6,000 drugs, and the average Part D private plan formulary includes about 1,000 drugs on average. CMS pays Part D plans a fixed monthly payment which is based upon the average premium bid of all participating Part D plans. Medicare also provides other payments to these Part D plans to offset the insurance risk that these plans bear, and Part D plans that enroll low-income beneficiaries receive greater subsidies from the Medicare program.

According to the CMS actuaries, total Part D costs were about $62 billion in 2010, and the CMS actuaries project that total Part D spending will rise to $156 billion by 2020, an average growth rate of about 10 percent per year.

The rising cost of drugs will consume a greater overall share of Medicare spending over the next 10 years. This spending growth will require all of us to work together to ensure that costs remain affordable while maintaining access to necessary treatments.

I'd be happy to answer your questions.

[The prepared statement of Jonathan Blum appears in the Appendix on page 42.]

The CHAIRMAN. Thank you, Mr. Blum. I'm sure you are familiar with the general fact that many of these prescription drugs are available in other countries for much less than what they cost here in the United States. Why do you think this is so?

Mr. BLUM. Well, I think a couple of reasons. One is that the public programs, Medicare and Medicaid, operate our payment systems according to very strict statutory formularies. Different countries

use other payment mechanisms. We have a policy within CMS not to require formularies. Private Part D plans are able to implement formularies. So we have different statutory frameworks than I think other countries can operate under.

The CHAIRMAN. Well, would that suggest that, in a sense, we're shooting ourselves in the foot?

Mr. BLUM. Pardon me. I don't understand the question.

The CHAIRMAN. Would that suggest that we are making mistakes in how we operate our programs here in this country if our goal is to provide the product at the least possible cost?

Mr. BLUM. Well, I think one thing that we observe within both the Part B and the Part D payment systems is that when drugs have competition, meaning they have generic alternatives or they have multiple drugs competing in the same therapeutic class, we see much more pricing pressure. We see less pricing pressure for drugs that don't have competition, that don't have generic substitutes. So I think that's one observation.

And I think to CMS' observation, when we have competition for drugs in particular classes, when we have generic alternatives, we see greater pricing pressure through both the Part B payment system and also the Part D payment system.

The CHAIRMAN. Mr. Blum, as you know, I sent your agency a letter yesterday requesting that Medicare ensure that Avastin is available to all patients who choose to use it as a treatment for macular degeneration. As you note in your testimony, Avastin was recently shown by an NIH trial to be similarly safe and effective to Lucentis. I hope that CMS will bring immediate attention to this matter and make an affirmative national coverage decision for Avastin.

Mr. BLUM. We currently cover both drugs. Both drugs are covered through the Part B program. We note that the majority of physicians that treat this condition choose to use Avastin. Lucentis is an on-label drug, and the Avastin for the condition that you're concerned about is an off-label marketed drug. CMS currently pays for both drugs, and physicians have the option to use both drugs.

But while the majority of physicians use Avastin, a vast majority of the spending is for Lucentis, a higher priced drug through our payment system.

The CHAIRMAN. Why doesn't CMS obtain discounts on the much more expensive drug, Lucentis?

Mr. BLUM. We don't have any authority to do so under our current law. The pricing system is based upon the average sales price, which takes into account more or less the private purchasers of these drugs. I think what is true is that for Lucentis, this is a condition that's particularly focused within the Medicare program that I believe about 75 percent or so of the drug is delivered to Medicare beneficiaries through our fee-for-service program.

But the statutory construct is such that CMS pays based upon the average sales price, but we also note that the Medicare program is by far the largest part of the spending for this particular drug.

The CHAIRMAN. Thank you very much, Mr. Blum.

And now we turn to Senator Corker.

Senator CORKER. Thank you, Mr. Blum, for being here and for your testimony.

You mentioned that Part D plans negotiate rebates, and there's been some legislation put forth by a couple of Senators looking at that ceiling issue and other kinds of things that introduce Medicaid-style drug rebates into Medicare Part D. Is that something you support or do not support?

Mr. BLUM. Well, I think what the President has said is that he is open to all ideas in the context of the debt ceiling discussions. In April, the President put out a framework——

Senator CORKER. I was asking you specifically, since this is what you do, whether you support Medicaid-style rebates or not. I understand what the President may or may not——

Mr. BLUM. Sure. Well, as an official of CMS, I have to support the official position of the administration.

Senator CORKER. So did the President take a position on Medicaid-style rebates?

Mr. BLUM. I think what the President said is that he's open to all ideas in the context to reduce overall costs, both in the Medicare——

Senator CORKER. Just sort of let me move away from the talking points. Do you, as an official that deals with health care issues on the issue of prescription drugs, which is why we're all here, do you or do you not support Medicaid-style rebates for Medicare Part D?

Mr. BLUM. I believe that the Medicare program has proven successful in lowering drug costs through competition. I also believe that there are certain drugs that are provided through the Medicare program that don't have as much competition, and there are more opportunities for us to reduce costs.

Senator CORKER. So I think what you're saying is in the overall Medicare Part D program, you think it's worked pretty well. There may be some isolated cases where you would recommend a different type of approach.

Mr. BLUM. I believe that the Congressional Budget Office has scored a policy that would require Medicaid-level rebates for certain drugs at about $120 billion savings for the next 10 years. The President has said that he's open to all ideas and offered that as one suggestion to reduce overall Medicare spending.

Senator CORKER. Would it make any sense in those areas to maybe have the same type of competitive structure that we have in Medicare Part D now?

Mr. BLUM. In terms of the parts of the program? We know that when we structure competition in parts of the program, like durable medical equipment, that we get lower costs, get better prices for both the beneficiaries and for taxpayers. The Part D program in general has produced much lower Part D premiums than I think our actuaries had predicted when the program was enacted.

But at the same time, in order to get competition, you have to pick winners and losers. In cases where there aren't alternatives or there isn't competition for products or suppliers, it's very difficult to get lower prices through competition. Where you have lots of choice and you have lots of suppliers, like in the durable medical equipment context, or in the Part D plan context where we have 25 or 30 stand-alone drug plans competing in the same market, we

see that competition produces good results. In the cases where we have a single item for a single product, it's very hard to get lower prices through competition.

Senator CORKER. Thank you. I think, again, to restate what you're saying is Medicare Part D works really, really well as far as the competition goes in lowering prices for seniors, but there are some isolated cases where when only one type of drug is available, we might look at some other ways of dealing with that.

Mr. BLUM. The total cost of Part D is certainly lower I think than the actuaries for the Congressional Budget Office estimate. I think part of that is that we have seen much more rapid generic diffusion through the Part D program than I think the actuaries would have said. I can't speak for the actuaries, but I think what they would say is that the main reason we're seeing lower costs than expected is that we have much more generic competition and diffusion than they had predicted back in 2003 when the benefit was enacted.

We also see robust premium competition for Part D plans, and we see beneficiaries gravitate to the plans that offer the most competitive premiums.

So I think to my observation, the number one reason why Part D costs remain low is that we have more generic use through the Part D program than in other payment systems, but we also see very robust premium competition for Part D plans, and we have a very rich market.

Senator CORKER. Thank you. That's quite an endorsement.

Let me ask you, on the Medicaid programs in general where we have a different type of situation, we've seen tremendous cost increases on the prescription drug side of Medicaid, which has a very different type mechanism. Is that not true?

Mr. BLUM. My observation is that when you compare Medicaid paid net prices to other purchasers, that oftentimes Medicaid is a lower price. I don't know the reasons why Medicaid drug spending is growing like you say, but I would guess that most of that growth is due to the fact that we have more beneficiaries in the program, not necessarily higher prices for prescription drugs.

Senator CORKER. I see my time is up, and I thank you again for answering the questions the way you have. I appreciate it.

Mr. BLUM. Thank you.

The CHAIRMAN. We turn now to the Senator from Ohio, Sherrod Brown.

Senator BROWN. Thank you, Mr. Chairman, and Ranking Member Corker. I appreciate especially being here at the request of the chairman because I'm not on this committee, and I appreciate the opportunity to share some information and ask you something.

I first appreciate the chairman's work on Avastin/Lucentis.

I want to bring another issue to you on a progesterone called P17, marketed by KV Pharmaceuticals out of St. Louis as a drug called Makena. I think you know the story, that for several years women, at the cost of $10 to $20 a dose and 20 doses, 20 weeks once a week of a shot they get typically in a hospital or doctor's office. So the cost overall of $200 or $300 for the whole regimen of this P17 progesterone has dramatically cut the rate of low birth weight babies born in this country. Medicaid pays for about 42 per-

cent of the nation's more than 4 million annual births. Twelve percent of live births involve a preterm baby.

So compounding pharmacists were making these drugs. Often one in a community or in a major city hospital or whatever were producing these drugs, and women's lives or babies' lives were saved in many cases. Babies were born full course, full term much more often.

KV Pharmaceuticals, a company I'd not heard of before this, went to FDA, got approval for exclusivity for seven years. They raised this $10 to $20 a dose for 20 weeks, $200 to $300, $200 to $400, to $1,500 a dose times 20. Do the math. Under pressure from many of us, they dropped the price to $690. That's still a significant public health problem. Call it greed, call it gaming the system, call it what you want, it's a significant public health problem.

It's also a significant insurance company and Medicaid/taxpayer problem.

We have seen a similar kind of gaming the system on a drug called Colcrys, as you know, treating gout. It used to be 4 cents a pill. After URL Pharma went to get FDA approval, the price went from 4 cents a pill to $5 a pill. Gout is a serious problem for a lot of people in this country.

My question is—oh, one more thing. The FDA—oh, I'm sorry. Yes, the FDA did something that is highly unusual. FDA, when KV Pharmaceuticals sent a cease and desist order to compounding pharmacists all over America, the FDA stepped in and said we will not enforce that cease and desist order, implicitly saying carry on and keep compounding this drug.

Now, there is not a public safety issue here. There's never been any accusation the compounding pharmacies, pharmacists and pharmacies have contaminated this drug, have made it in a way that's not safe for these women, never that I've read any accusation about that.

So my question is what do you do about this? On Colcrys, on Makena, it's such a public health issue, it's such a taxpayer issue where even today, after CMS or—I'm sorry, after FDA stepped in, only three states, according to the American College of Obstetricians and Gynecologists, only three states are solely covering 17P. Five are covering only Makena. Twenty are covering both. So if the physician is not on her toes here, and if the ultimate buyer of this drug or the user of this drug or the hospital is not paying enough attention, they're paying more like $690 a dose instead of the $10 to $20 that compounding pharmacists are still making this for.

What is your role and what is CMS really going to do to make sure the public health isn't at risk and taxpayers aren't paying billions of dollars more, whether it's Colcrys, whether it's Makena, whether it's the next drug that some opportunistic—I won't use the word "greedy" but opportunistic drug company decides to move forward on?

Mr. BLUM. A couple of observations on the examples that you raised. I think it shows that when a drug or a product that doesn't face competition from other products or generics, that they can exercise monopoly pricing power, and that's the incentive to do so.

So I think one thing that CMS and every other part of the public health infrastructure needs to do is to ensure that we create con-

sistent and quick pathways consistent with the law to generics to ensure that we have competition. But outside of——

Senator BROWN. That's not—sorry to interrupt. That's not good enough here because they have seven years of exclusivity. So that's an answer in some cases. I don't think it's an answer to the chairman's issue, and it's certainly not an answer to these two drugs.

Mr. BLUM. I think, in complete frankness, Senator, the authorities that you're suggesting aren't authorities that CMS has today. If Congress would like CMS to exercise those authorities, the law would have to be changed in order for us to do so.

Senator BROWN. You have no role in negotiating drug prices in that narrow window?

Mr. BLUM. No part of my testimony said that our payment systems don't tie to drug manufacturers directly. CMS pays physicians. CMS pays drug plans. CMS pays hospitals, who in turn purchase drugs, and our payment systems are set based upon very tight statutory constructs. So, today, CMS does not have any negotiating authority directly with drug manufacturers.

Senator BROWN. Thank you, Mr. Chairman, for your time. Thank you.

The CHAIRMAN. Thank you very much, Senator Brown.

Now we turn to Senator Kirk.

Senator KIRK. Thank you, Mr. Chairman. Thank you for having me on this committee.

I'd like to raise—I've got a chart here. I'd like to raise an issue with regard to IPAB, the Independent Payment Advisory Board, and the British equivalent, the National Health Service. Their equivalent of IPAB is called the National Institute for Health and Clinical Excellence, called NICE.

And what I'm worried about is NICE is not so nice, generating clinical outcomes significantly worse for patients who are unfortunately under its jurisdiction rather than American seniors, who are under Medicare.

When you look at several of the indicated medicines that are available, you see, for example, in postmenopausal women, Herceptin is indicated and is available under Medicare for treatment. But NICE denies this, and that would total about 46,000 women in the United Kingdom that are not allowed Herceptin because a British bureaucrat has said to all British doctors, under every circumstance, no matter what your clinical judgment regarding this patient is, you may not provide this. Luckily, we still give this freedom to U.S. physicians.

For liver cancer, the indicated treatment may be Nexavar. And in England, the NICE bureaucrats have now denied authority for all British physicians to provide this.

This may be one of the reasons why the United Kingdom now ranks 16th out of 18 EU states in cancer survival in this area. They simply are dying, and part of the reason might be that what is indicated and especially could be provided by a physician under Medicare is not allowed.

In colorectal cancer, we all understand Avastin, and Avastin has been shown as being clinically indicated to cut off the blood supply of a tumor. For Americans suffering from kidney cancer, they may be prescribed with Affinitor. Affinitor is indicated if the other drugs

are not working, and it has also been helpful when you have a transplant. It helps the body accept this.

In the case of British patients, they are denied Affinitor and its benefits.

Probably a bigger disease, leukemia, the cancer of white blood cells, for Spyrocel, this is used if Gleevec is not seen as effective. American physicians under Medicare are allowed to do this. NICE under the NHS then denies all care for this.

And then for lung cancer, Tarceva, which is used for small cell cancer if other chemotherapy fails, and also in pancreatic cancer, denied.

Here's my question. What is going to prevent IPAB from metastasizing—and I use that word directly—into NICE? Because I think for many Americans, we go to England, especially on holidays, and normally an American will not get on a plane and leave the United States for a holiday unless they're in a good health status. And so Americans' personal experience with the NHS is minimal to none.

I lived and worked in Britain for three years, and I can tell you my first experience inside a British hospital was shocking as to its level of physical infrastructure, some hospitals being not improved or expanded since the blitz, and then the denial of care and lack of technical expertise, as opposed to what I saw at Evanston Hospital near my own town.

What actions are you taking to make sure that IPAB can never metastasize into NICE?

Mr. BLUM. I think a couple of things. One is that as the Affordable Care Act structured the IPAB, that it was structured as an independent body from CMS. And I think that the goal, as I understand it, of the legislation was to create mechanisms to ensure that overall costs of the Medicare program and other parts of the health care system remain affordable. And I think we can all agree that the ultimate goal is to ensure that per capita cost growth remains affordable to ensure that the Medicare program remains strong, and for current beneficiaries——

Senator KIRK. Wait, wait. When you lay out that mission, NICE's view is NICE is connected to a bankrupt government. The British government has almost as many debt loads as we do. And so the bureaucrats then use comparative effectiveness research to then support the kind of decisions that I just laid out that then deny care, driving cancer survival rates in Britain to the lowest in the EU.

Mr. BLUM. Sure. Well, my understanding of the legislation is that the IPAB provides recommendations to the Congress. Congress has the right to choose to accept those recommendations or not. The Congress can overrule those recommendations.

Senator KIRK. And then this is regardless of what a physician thinks is indicated for their patient. What if the physician disagrees with what IPAB recommends?

Mr. BLUM. Well, I think a couple of things. One is that the IPAB legislation, consistent with the Medicare framework today, I believe prohibits coverage decisions or any coverage decision from factoring costs to those coverage decisions. So I think the chart that you're suggesting suggests that NIHCE takes into account cost considerations for particular drugs.

The current coverage authority that CMS operates under in the Medicare program does not allow us to consider cost in making coverage decisions, and I believe the IPAB legislation doesn't change that framework that we currently operate under today.

Senator KIRK. Mr. Chairman, I would just say I'm highly worried that before the legislation we didn't even have an IPAB. Now we have an IPAB, and I think its goal inevitably will be to metastasize into what the British have.

Thank you, Mr. Chairman.

The CHAIRMAN. Thank you, Senator Kirk.

Now we turn to Senator Udall.

Senator UDALL. Thank you, Mr. Chairman.

I listened to my colleague from Illinois with interest.

I do understand, Mr. Blum, that if the IPAB recommendations are not acceptable to the Congress, we can simply override them with our own ideas and our own proposals about how to contain costs. Is that correct?

Mr. BLUM. That's my understanding, Senator, yes.

Senator UDALL. That's your understanding? And I also understand that we have had a similar advisory committee attached to CMS, MedPac some have called it, which has made a series of insightful recommendations in retrospect about ways in which to contain costs but also maintain quality of treatment.

Mr. BLUM. Right.

Senator UDALL. Would you agree?

Mr. BLUM. MedPac serves the Congress. They don't serve CMS. So they're an independent agency that provides the Congress recommendations about how to improve Medicare/Medicaid payment policy. They provide recommendations to the Congress each year that the Congress can choose to accept or not.

I think what is different about the IPAB is that, if the Congress does not act upon the recommendations, the Secretary of Health and Human Services would have the authority to implement those recommendations.

But you're absolutely correct that the recommendations go to Congress. Congress can choose to accept or to suggest other ways. And so I see it as a body that serves the Congress to provide recommendations to contain overall per capita spending.

Senator UDALL. And that's the point of the hearing today, and I want to thank Senator Kohl and Senator Corker for convening this.

If I might, Mr. Chairman, I'd like to ask unanimous consent that my initial statement be included in the——

The CHAIRMAN. Without objection.

Senator UDALL [continuing]. In the record.

And if I could, I'd just like to turn to the earlier comments you made about generic drugs. They're obviously a focus of the Medicare Part D, and I know there's some good news on that front.

Would you talk about your sense of how branded drug costs have affected costs at CMS, as opposed to generics?

[The prepared statement of Senator Mark Udall appears in the Appendix on page 114.]

Mr. BLUM. Sure. Well, I think we operate under different statutory frameworks and different competition within different therapeutic frameworks in the Part B program and the Part D program.

What is driving Part B drug spending? These are drugs largely provided through physician offices. The spending is concentrated on a handful of drugs. They're often new drugs coming on the market that I think are in the brand category.

In the Part D program, we continue to see robust generic competition for many of the most commonly prescribed drugs for conditions like diabetes, heart care, et cetera. In the Part D context we see very strong generic competition, less so in the Part B drug context. Many of the drugs that we pay for in Part B are drugs used to treat cancer that are new treatments that still are in their market exclusivity. But on the Part B side, we have a concentration of spending in a handful of drugs due to the newness of the treatments, and due to the popularity of the treatment.

Senator UDALL. On balance, do you see a flattening out of the costs on whatever metric is the most useful on the branded side?

Mr. BLUM. Well, I think——

Senator UDALL. Again, the point of the hearing is how do we maintain quality, how do we encourage the pharmaceutical sector to innovate and take some risks, but how do we get a handle on the enormous cost of providing drugs to Americans.

Mr. BLUM. And I think one observation is that while we need to have strong incentives for manufacturers to bring new markets to market, we see that when drugs do face competition from generics or other treatments, that the prices that are fed through our payment system reflect that competition.

So I think the question is how do you always find the right balance between creating strong incentives to ensure that new markets come to market, and also that competition can happen when it's appropriate for it to happen.

Senator UDALL. Let me turn to outcomes. The Affordable Care Act was focused in part on outcomes. What are you doing to pay for services based on health care outcomes, and how does evidence on health care outcomes affect CMS determinations on what drugs they cover?

Mr. BLUM. Well, a couple of things. One is the Affordable Care Act clearly gives CMS a very strong direction for us to develop the next generation of payment systems for hospitals or for physicians to really focus on the overall value of care rather than the volume of care. We have different authorities that we're implementing, or different programs that we're implementing with this direction.

One is accountable care organizations to ensure that physicians working with all parts of the health care system really focus on the long-term outcomes of the patient rather than a single episode of the payment. We are starting to receive stronger evidence that, when beneficiaries continue to follow drug regimens, when beneficiaries have access to drug benefits, that it saves the program long term. I think these are initial studies. I'm not sure that our actuaries have given them kind of absolute certainty, but there is stronger evidence that, when we focus on the overall preventive care, that we save long-term costs.

And so, hopefully with our direction, and I think the health care system's direction of focusing payment on the outcome of the patient, the value of the care rather than the volume of the care, that physicians will make the best possible choices with their patients to ensure that care is better coordinated, better managed for the long term.

Senator UDALL. Thank you, Mr. Blum.

Mr. BLUM. Thank you.

The CHAIRMAN. Thank you very much, Senator Udall.

Now we turn to Senator Manchin.

Senator MANCHIN. Thank you, Mr. Chairman, appreciate it. Thank you, Mr. Blum.

Basically, with rising costs, especially of Medicaid or Medicare Part D costs, they're estimated to rise about 9.7 percent, I think, for the next nine years. And with the waste, fraud and abuse that we see an awful lot throughout government and throughout basically the programs that we're responsible for, what are you all's intentions and what do you think can be done within your confines in order to remove or eliminate or reduce significantly the waste that's in as far as the billing, overbilling, or wrong prescription?

And rebates, I think, as I had done a little bit of investigation, the Inspector General's findings suggest that the underreporting of drug rebates has led to excess rebate payments of approximately $1.9 billion per year. And do you all, are you looking at that? Do you have a group or a task force to eliminate that?

Mr. BLUM. Sure. I think the Part D program is administered through private Part D plans, private insurance plans, who then pay pharmacists and then sign up beneficiaries and operate the benefit through pharmacy benefit managers.

Part of our strategy to address the concern that you're raising is to ensure that we set very strong requirements for our Part D plans, to have compliance programs in place to share data. So when we see a fraud issue on the fee-for-service side, we share that information with private insurance companies so they can act accordingly.

We're doing a lot more with sophisticated data, data analysis, to highlight and kind of bring to bear when spending is concentrated within a particular physician or a particular pharmacy.

So our strategy I think is twofold. One is to ensure that we incent and require our Part D plans that are providing the benefit on behalf of our beneficiaries to have the strongest compliance programs in place. When we see plans have weak compliance programs, we take action very quickly. But also our strategy is to share information, to share data so it's not just the fee-for-service Medicare program that's responding to a fraud hot spot. We're sharing that information with all of our partners to ensure they can respond as well.

But if we know that folks that are trying to commit fraud, if one spigot gets cut off, they move to another spigot, so our strategy is to make sure we're working in unison to ensure that all the spigots get cut off, to the extent possible.

Senator MANCHIN. With the cost of drugs, prescription versus generics, do you all play a role in your rulemaking as far as what we are to prescribe first, or go to low cost?

Mr. BLUM. We allow Part D plans, consistent with the statutory framework, to establish formularies, to set differential cost-sharing policies, to encourage beneficiaries to use generic drugs or lower-cost drugs relative to higher-cost brands. So those formularies have to run through our checks and balances to ensure that they're fair for our patients, but we provide the incentives, and also we provide the framework for private Part D plans to set those cost-sharing policies to encourage the——

Senator MANCHIN. So you're telling me that basically our prescription prices for our drugs are anywhere from 30 to 50 percent higher than most other nations.

Mr. BLUM [continuing]. We operate within current statutory frameworks, and our payments are consistent with those statutory frameworks.

Senator MANCHIN. Do you have any opinions on that? Do we have the right statutory provisions in place, or do we need to make some adjustments?

Mr. BLUM. I think what——

Senator MANCHIN. To reduce the costs, just to lower the costs so we're able to provide to more people in need.

Mr. BLUM. What I can say, Senator, is that the President has made it very clear that lowering Medicare costs and all costs in the health care system is one of our highest health care challenges, and he has said that all ideas and options are on the table in the context of the overall debt ceiling discussions.

Senator MANCHIN. Would you agree basically that if you would use prescriptions, that you would have a reduced cost if they were available for the same type of treatment where a higher-cost drug is available?

Mr. BLUM. I'm sorry. I don't understand the question.

Senator MANCHIN. Well, basically if you all had a policy or if you want us to change the law that would require you all to use the lowest-cost provider or the lowest-cost drugs for their treatment, then there would be tremendous cost savings.

Mr. BLUM. I think the overall framework that CMS operates under is that the physician and the patient should make those choices together.

Senator MANCHIN. We're paying for it, though.

Mr. BLUM. Absolutely. And so our payment policy should support the physician and the patient to make those best possible choices. We also know that, due to our cost-sharing policies, higher-priced drugs generally have higher-priced copayments attached to them. And so we have to be very sensitive that it's not just the taxpayers who are paying higher prices, but it's out-of-pocket costs that are also impacted as well.

And so our payment framework pays for drugs indirectly, but we also have coverage policies that support physicians and patients making the best possible choices, and hopefully part of that discussion is taking into account the out-of-pocket costs that are being borne by our beneficiaries.

Senator MANCHIN. I'd like to go into it in more depth with you, because I think in these budgetary-constrained times that we have, we should be looking at trying to get the best bang for our buck, and right now it doesn't seem that we're doing that.

Mr. BLUM. I'd be happy to follow up with that, Senator.

Senator MANCHIN. Thank you.

The CHAIRMAN. Thanks a lot, Senator Manchin.

Now we turn to Senator Blumenthal.

Senator BLUMENTHAL. Thank you, Senator Kohl, and thank you for again having a very, very informative and useful hearing. And thank you, Mr. Blum for your testimony here today.

Would you agree with me that the Veterans' Administration has greater leeway or authority to negotiate lower drug prices?

Mr. BLUM. The VA operates I think relative to the Medicare program but with a much tighter what I would call formulary or drug list, and that provides them more negotiating leverage than I think what we operate within the Medicare program. And so the way that the statutory construct for the VA payment system has been constructed is giving the VA the freedom to kind of manage a much tighter formulary, which gives them more negotiating leverage to extract overall lower prices than what the Medicare program would pay.

Senator BLUMENTHAL. And in effect, just cutting through what you just said and putting it in layman's terms, the VA can negotiate lower drug prices by using the Federal Government's bargaining power on its formulary. Is that not correct?

Mr. BLUM. Sure, and I think——

Senator BLUMENTHAL. And under Medicare, that practice, that use of the Federal Government's bargaining power is essentially barred; correct?

Mr. BLUM. Correct.

Senator BLUMENTHAL. And don't we have an obligation to enable taxpayers and seniors to have lower drug prices by using the Federal Government's bargaining power to lower those drug prices?

Mr. BLUM. I think it's fair that we have an obligation at CMS in the Medicare program to ensure that we are managing costs throughout the program to the best of our ability, and we pay for drugs today within the confines of the statute that's been given to us.

Senator BLUMENTHAL. And it really is, in fairness to you, Mr. Blum, the confines of the statute that, in effect, straightjacket you. There's really no other word for it, in my view, straightjacket you from serving the public interest by saving taxpayers and seniors money by using the Federal Government's bargaining power to lower drug prices.

Mr. BLUM. Sure. My observation is that the payment system that we use for Part B drugs is set very clearly by statutory formulas. CMS operates those payment systems, but we cannot influence those payment systems.

Senator BLUMENTHAL. And there's no other way to view it than as a kind of loophole, giveaway, sweetheart deal that raises the cost of drugs at a time when the cost of drugs is already spiraling upward; correct?

Mr. BLUM. I'm sorry. I don't understand the question.

Senator BLUMENTHAL. Well, let me put it a different way. Wouldn't you recommend that the confines of the statute, as you have adroitly put it, be changed so that the public interest could be better served to lower drug prices?

Mr. BLUM. I can't speak to a specific policy recommendation, but what I can say is that if we—the Congress believes that the pricing mechanisms or the pricing outcomes should be different, then I believe that the statutory construct would have to be changed.

Senator BLUMENTHAL. Just to put it in very practical terms, my understanding is that, in FY 2010, the VA in fact spent $3.9 billion in drugs and realized cost savings from negotiations of $700 million. If you were to extrapolate from the current expenditures on Medicare and prescription drugs, there would literally be billions of dollars in savings; correct?

Mr. BLUM. Well, I think you'd also have to extrapolate the coverage that the VA programs provide relative to the Medicare program. And it's my understanding that the VA operates a tighter formulary, if you will, which gives them more leverage. In order to have leverage, you have to say yes to one product and no to another product to create that negotiating clout.

Senator BLUMENTHAL. But even with—and I apologize for interrupting, but my time is about to expire. Let me just make the point, whether the formulary is tight or expansive, negotiations enable lower prices; correct?

Mr. BLUM. When there's competition, yes.

Senator BLUMENTHAL. And the Federal Government, it seems to me, has an obligation to taxpayers and seniors to take advantage of competition where it exists, or enhance it where it should be more robust in order to achieve those savings. I recognize you operate within the confines of the statute, so I am not asking these questions in a way that is meant to be hostile to you personally, but I thank you very much for your testimony today.

Mr. BLUM. Thank you, Senator.

The CHAIRMAN. Just one point before we turn to Senator Whitehouse. Senator Blumenthal, of course, you were on legislation that would authorize Medicare to negotiate directly with the pharmaceuticals. Is that right?

Senator BLUMENTHAL. I am indeed, and thank you for your leadership on that legislation, Mr. Chairman.

The CHAIRMAN. Thank you.

Senator Whitehouse.

Senator WHITEHOUSE. Thank you, Chairman, and thank you for holding this important committee hearing.

Why is it, Mr. Blum, that you aren't prepared to make a policy recommendation to this committee? Is that a restriction related to your position at CMS?

Mr. BLUM. Well, I think I can speak for the administration's position, and what the President has said is that he is open to all ideas. He has suggested some possible ways in April for us to reduce Medicare and Medicaid costs. One suggestion was to think about requiring that the Medicare program receive deeper discounts for certain drugs that are provided through the Medicare program. But that is a statement that I'm prepared to say here today.

Senator WHITEHOUSE. We're often told that government should try to run more like a business. Can you think of any business that doesn't exercise its buying power to achieve price advantages?

Mr. BLUM. I would assume the answer is yes, that businesses have a clear incentive to maximize revenue and lower costs, and part of that would be through negotiations.

Senator WHITEHOUSE. And you can't think of an example to the contrary. I mean, that is the way business behaves; correct?

Mr. BLUM. That is my understanding.

Senator WHITEHOUSE. So it is unusual for government to be, at least with respect to the business model, it is a departure from government operating more like a business to have government be constrained by the statutory confines you talked about and forbidden as it was in the Part D act from exercising its negotiating leverage.

Mr. BLUM. Well, the Part D legislation that was enacted in 2003 provides CMS the leverage to negotiate with Part D plans. That authority was expanded through the Affordable Care Act. The leverage that we have is through our contracting with Part D private plans. But you're correct, we have no authority to negotiate with manufacturers to receive better prices paid or provided to our private Part D plans.

Senator WHITEHOUSE. And does that relate back to the Medicare Modernization Act of 2003 and its so-called noninterference provision?

Mr. BLUM. The noninterference provision prohibits Medicare CMS from interfering with private negotiations, with private health plans, with pharmaceutical companies, hospitals, any other service that is being purchased by the private plan. Our authority is to contract with the plan, but by and large we cannot interfere with the negotiations with the plan and their other providers or suppliers. We have authority to make sure that the benefits are consistent with the program's requirements, but the prices that are contracted with the plan and the manufacturer are outside of CMS' purview.

Senator WHITEHOUSE. Setting aside the merits or the policy recommendations just for a moment, the National Committee to Preserve Social Security and Medicare has estimated that nearly $240 billion could be saved in the Medicare program over 10 years if the Secretary were authorized to negotiate drug prices. As an estimate, do you have any comment on its accuracy?

Mr. BLUM. I've seen different estimates on such authority, but I had not personally seen that estimate that you cite.

Senator WHITEHOUSE. What estimate do you have or do you credit that is out there that would reflect the potential savings from such a change in law?

Mr. BLUM. I believe the Congressional Budget Office may have scored a similar policy. I don't recall the results, but I believe it was lower than the numbers that you have cited.

Senator WHITEHOUSE. One of the things that I hear from Rhode Island seniors pretty often is that they have signed on to a Part D plan, and once they were signed on, the formulary then changed and the drug or drugs that they're using and dependent on, or the reason they signed on to that Part D plan, are suddenly either no longer available or require a different and higher copay. In any event, they signed up for one thing, and in midterm they got dealt another set of conditions that they had never agreed to.

Can you tell me what role CMS can play to ensure that Part D plans have to—can't make these midterm changes and that they can only become effective after a period has expired that would allow the senior to make a different set of choices, and that you basically get what you signed up for and you're guaranteed to get what you signed up for until you can find something different?

Mr. BLUM. Sure. What CMS requires, I believe, and this may not be 100 percent accurate, that when that drug plan changes a drug that's provided on the formulary midyear, the plan is required to notify the patient, to provide a transition fill to ensure that the beneficiary can go back to their physician to get a new prescription.

But I—but drug prices change throughout the year, and some manufacturers have the freedom to raise and lower prices throughout the year. So I would be hesitant, in the interest of ensuring that prices remain low and affordable, both for the program and for the taxpayers, to take away Part D plans' ability to change formularies for different circumstances, whether the drug is deemed not effective, whether the price goes up and the Part D plan needs to respond to keep premiums affordable.

So our policies require that the plans provide notice and transition, but at the same time I would be hesitant, personally, given that we want to make sure that Part D plans have the freedom to respond to different circumstances.

Senator WHITEHOUSE. Thank you, Chairman.

The CHAIRMAN. Thank you very much, Senator Whitehouse.

Mr. Blum, we thank you for being here. You've been very helpful, very informative. We're looking forward to continuing our work with you.

Mr. BLUM. Thank you very much, Senator.

Senator CORKER. Mr. Chairman, could I make one comment?

The CHAIRMAN. Go ahead.

Senator CORKER. I want to thank you for coming, too. And I think in spite of the push by many of my colleagues on the other side of the aisle to push you into direct negotiations on Medicare Part D, I think what you said is that it has been very, very successful at keeping prices low because of the tremendous amount of competition that exists, and that there may be a need in some isolated cases where only one type of drug is available to look at a different type of arrangement.

But in Medicare Part D, generally speaking, the costs are far lower than ever imagined, seniors have far more choices than they ever would have, including the VA I think as you mentioned, and from your perspective this competitive nature of Medicare Part D has been very, very successful, and messing with it in any way would likely lead to some unintended negative consequences. So I thank you very much for being here and appreciate your testimony.

The CHAIRMAN. Thank you.

We'll turn now to our second panel, if you would approach the witness stand.

First we'll be hearing from Dr. Philip Rosenfeld, Professor of Ophthalmology at the University of Miami. Dr. Rosenfeld has worked on many clinical trials involving innovative treatments for eye diseases.

Next we'll be hearing from Dr. Anthony Adamis, who serves as a Global Head of Ophthalmology at Genentech. Dr. Adamis was formerly a professor at Harvard Medical School and is cofounder of Eyetech Pharmaceuticals.

Next we'll be hearing from Dr. Sean Tunis, who is founder and director of the Center for Medical Technology Policy.

After that we'll be hearing from Lisa Swirsky. She is a senior policy analyst for Consumers Union Health.

Finally, we'll be hearing from Dr. Scott Gottlieb. Dr. Gottlieb is a resident fellow at the American Enterprise Institute.

We welcome you all. We're looking forward to what you have to say, and we'll now start with you, Dr. Rosenfeld.

STATEMENT OF PHILIP ROSENFELD, M.D., Ph.D., PROFESSOR OF OPHTHALMOLOGY, BASCOM PALMER EYE INSTITUTE, MIAMI, FL

Dr. ROSENFELD. Chairman Kohl, Ranking Member Corker and other distinguished members of the committee, thank you for inviting me today to testify on this important topic. I'm Dr. Philip Rosenfeld, Professor of Ophthalmology at the Bascom Palmer Eye Institute of the University of Miami Miller School of Medicine. This statement represents my own opinion and not those of the University of Miami or the Bascom Palmer Eye Institute.

I bring to the discussion today a real-world perspective of the forces influencing the choice between two commonly used drugs for the treatment of wet macular degeneration. By studying this example, I believe we can better understand how the current incentives in our health care system promote the use of the most costly alternatives.

These drugs are being used to treat wet macular degeneration, a leading cause of irreversible blindness among the elderly worldwide. When I say wet macular degeneration, I'm talking about the abnormal growth of blood vessels in the back of the eye. These blood vessels leak, they bleed, and they accelerate vision loss.

Genentech performed groundbreaking scientific research that led to the discovery of two fabulous drugs, Avastin and Lucentis. Both drugs block the factor that causes the blood vessels to grow. Both drugs are derived from the same mouse monoclonal antibody. Avastin is a full-length antibody; Lucentis is a fragment of that antibody. Avastin is infused through an arm vein every two weeks in cancer patients; Lucentis is injected into the eye as often as every month. Avastin was FDA approved for colon cancer therapy in February of 2004; Lucentis for eye injections for wet macular degeneration in June of 2006.

I don't have time to go into the background of why I first injected Avastin into an eye, but it was clinically and scientifically justified. This off-label injection was successful and led to the international use of Avastin for a wide range of eye diseases. The rapid spread in 2005 was fueled by the availability of Avastin, its apparent efficacy and safety, its low cost, and the fact that Lucentis was not yet available, though everyone was seeking Lucentis. Even after Lucentis was approved, though, Avastin continued to be used as the low-cost alternative to Lucentis.

When a pharmacy follows strict USP—that's United States Pharmacopeia—guidelines, Avastin can be prepared for $20 to $40. Lucentis costs $2,000 a dose. Since 2005, 1,500 scientific papers have appeared in peer-reviewed journals exploring the safety and efficacy of Avastin. However, definitive data was not available until the CATT trial results were published in the New England Journal of Medicine in May of this year. Dr. Dan Martin, chairman of the Cole Eye Institute of the Cleveland Clinic and chairman of this National Eye Institute-sponsored multicenter clinical trial, is here in the audience today.

In this two-year study, injections of Avastin and Lucentis were compared in 1,200 wet AMD patients. After one year, the Lucentis injections given monthly were comparable to the Avastin injections given monthly. When Lucentis was given as needed, it was comparable to Avastin given as needed. Overall, the two treatments seemed equivalent.

There were no apparent expected adverse event differences between the two drugs. However, Avastin was associated with an increase in unexpected adverse events that are not thought to be drug-related. However, these adverse events are closely being studied in the second year of the trial.

To understand how these drugs are used in the United States, I collaborated with Ross Brechner at Medicare, and we found that 60 percent of physicians in 2008 used Avastin, 40 percent used Lucentis. This is looking at 100 percent database from Part B Medicare. We saved Medicare approximately $800 million in 2008 alone by the use of Avastin.

So what determines why clinicians use one drug or the other? We have found there are several incentives in the system that promote Lucentis use. First, Medicare promotes the use by the 6 percent average sales price reimbursement to physicians. Not only does Medicare cover the cost of the drug, but they also add 6 percent of the average sales price. That's $115 every month for a $2,000 investment. That investment is returned every month. So overall, the physician makes a 70 percent return on that initial $2,000 on Lucentis.

In addition, CMS decreased the reimbursement for Avastin from $50 to $7 in a hospital-based setting. This was part of a bigger reduction that was blocked by a number of my colleagues, specialty societies, and government officials. However, the $7 reimbursement in a hospital-based setting is still in effect. In addition, in what's called a disproportionate share hospital, the 340B discount program allows Lucentis to be purchased at $1,600 and get reimbursed at $2,000. That makes a $400 profit for each injection of Lucentis. This profit or this rebate should go to Medicare.

Finally, Genentech has two incentive programs. One is a rebate program reported by Andy Pollack of the New York Times, November 2010. It's very lucrative to clinical practices. It's based on volume use and increase in usage of Lucentis. This rebate should not go to the clinician. It should be going to Medicare.

And Genentech also allows the direct purchase using a credit card, allows cash back up to 2 percent to the physician. This rebate should go to Medicare and not the clinician. A transaction fee should be charged by Genentech.

So while these historical details surrounding the use of Avastin and Lucentis are unique in the annals of medicine, the financial incentives driving the use of expensive drugs and procedures are not. These incentives and disincentives should be eliminated.

And finally, I inject over 4,000 eyes per year. I use about half Lucentis, half Avastin, and as a clinician I don't want Medicare telling me which drug to use, but I don't want my patients worrying that my decision to inject their eyes is being influenced by these incentives. The choice between drugs should be based between the physician and the patient based on efficacy, safety, and cost. It is noteworthy that, despite all of these financial incentives, most ophthalmologists use Avastin. This suggests that most ophthalmologists really do care about the cost of health care.

Thank you again for this invitation, and for your attention. Thank you.

[The prepared statement of Dr. Philip Rosenfeld appears in the Appendix on page 53.]

The CHAIRMAN. Yes. We'll turn now to Dr. Adamis.

Before I do, did I hear you say you inject half of your patients with one and half of your patients with the other?

Dr. ROSENFELD. When I look at my 4,000 eyes that are injected, I use on average half Avastin in those eyes and half Lucentis, and the decision is based between discussions with my patient and their decision after all the options are presented whether they want one or the other drug.

The CHAIRMAN. You let your patient make that decision?

Dr. ROSENFELD. Once they're given all that information. I strongly believe that full disclosure is required for a patient to understand the reasons why a needle is going to be stuck into their eye.

The CHAIRMAN. I understand. You seem to be indicating your independent opinion is that the drugs are similar.

Dr. ROSENFELD. That's my clinical opinion.

The CHAIRMAN. Okay. Thank you very much.

Dr. Adamis.

STATEMENT OF ANTHONY ADAMIS, M.D., VICE PRESIDENT, GLOBAL HEAD OF OPHTHALMOLOGY, GENENTECH, INC., SOUTH SAN FRANCISCO, CA

Dr. ADAMIS. Chairman Kohl, Ranking Member Corker, honorable members of the committee, thank you for inviting me here today. I ask my full written testimony be submitted for the record.

My name is Tony Adamis. I'm the Vice President, Global Head of Ophthalmology at Genentech. I'm an ophthalmologist and vascular biologist by training. Prior to joining Genentech in 2009, I served in other positions in the biotech industry, as well as 11 years at the Harvard Medical School where I treated patients and conducted research.

Genentech is based in South San Francisco and as part of the Roche group currently employs over 30,000 people in the United States. Our commitment to innovation is unparalleled within the industry, with more than 100 projects in clinical development. In 2009 alone, Genentech Roche spent $9.1 billion on R&D, an amount greater than any other company in the world.

Genentech's mission is to develop innovative medicines to treat serious diseases. One of the most impactful medicines we've ever developed is Lucentis. Before Lucentis was available, wet AMD was the leading cause of blindness in older Americans. The average patient lost central vision until the ability to read, recognize faces and drive was lost.

In addition to the personal suffering and loss of independence, the total annual cost to the U.S. GDP was estimated to be $5.4 billion. Everything changed with the development of Lucentis. For the first time, the average patient with wet AMD recovered vision. When the results were first presented at a major medical meeting, Lucentis was publicly compared to the discovery of penicillin.

Since then, Lucentis has reduced the rate of legal blindness by 72 percent. As a result, wet AMD may no longer be the leading cause of blindness in older Americans. Subsequent investments in Lucentis trials by Genentech have demonstrated sustained gains in vision in two additional serious diseases, retinal vein occlusion and diabetic macular edema. So to date, Lucentis has exhibited heretofore unseen efficacy in three of the major causes of blindness in the United States.

Drug development is lengthy, expensive, and risky. Drugs entering clinical development have a 92 percent failure rate. Lucentis was one of the 8 percent that succeeded. The price of Lucentis therefore funds not only its own development but also the 92 percent that fail and our future successes. Eleven years and almost $1.4 billion have been spent on the development of Lucentis, involving over 18 clinical trials and 7,100 patients in the United States, and over 10,000 around the world.

In 1989, Napoleone Ferrara discovered vascular endothelial growth factor, or VEGF, at Genentech. His research showed that blocking VEGF might prove useful in the treatment of cancer, a line of research that eventually resulted in Avastin. Around the same time, my colleagues and I working with Dr. Ferrara determined that VEGF was also a potential target for eye disease. Dr. Ferrara, however, was concerned that Avastin may not be ideal for the eye, so his team set out to create something better. That drug became Lucentis.

For his work on Lucentis, Dr. Ferrara was awarded the Lasker Prize in 2010. Seventy-six Lasker laureates have gone on to win a Nobel Prize in medicine.

There are four scientific reasons why Lucentis was created. Today I will focus on one of them, systemic safety. When drugs are administered to the eye, they often find their way into the bloodstream. When that happens, side effects are more likely.

Avastin was designed to last a long time in the bloodstream so that it can have sustained activity against tumors. Lucentis, however, was designed to exit the bloodstream very quickly.

VEGF-blocking drugs can result in rare but serious side effects. When an interim safety analysis in 2007 revealed a potential stroke risk with the use of Lucentis, Genentech sent a letter to doctors, notified the FDA, updated the package insert, and presented the data to the medical community.

Today, there's a growing body of data that suggests off-label Avastin may pose a greater risk than Lucentis. Two large Medicare

claim studies, one from Duke and a second from Johns Hopkins, both identified a potentially greater risk of stroke and death when using Avastin in wet AMD. The CATT trial also showed a safety difference. A 29 percent increased risk of serious side effects was seen with Avastin, with over 80 percent requiring hospitalization. Genentech's internal analysis indicates that part of the increased risk is consistent with VEGF blockade in the blood stream.

These data are not yet conclusive. However, it is notable that the three largest studies to date have shown statistically significant safety risks with the use of Avastin in wet AMD. As the data emerge, we agree with the American Academy of Ophthalmology and the written testimony of the American Society of Retinal Specialists that a treatment plan must be selected by an ophthalmologist and a patient, considering important benefit/risk information that empowers them to make evidence-based decisions.

Genentech is also committed to ensuring that no patient goes without treatment due to financial barriers. Since 1985, we have donated $2.3 billion in free medicine to uninsured patients and more than $550 million to various independent nonprofit organizations for copay assistance.

We're committed to working with the Congress, public health agencies, CMS and the FDA to ensure the safety and effectiveness of our products. Today, innovation continues at Genentech as we seek to improve Lucentis and develop additional breakthrough medicines. This work depends in part on the success of Lucentis.

Thank you for the opportunity to provide my views today, and I look forward to your questions.

[The prepared statement of Dr. Anthony Adamis appears in the Appendix on page 61.]

The CHAIRMAN. Thank you very much, Dr. Adamis.

Now we'll hear from Dr. Tunis.

STATEMENT OF SEAN TUNIS, M.D., MSC, FOUNDER AND DIRECTOR, CENTER FOR MEDICAL TECHNOLOGY POLICY, BALTIMORE, MD

Dr. TUNIS. Mr. Chairman, Senator Corker, thanks for the invitation to appear before the committee today. My name is Sean Tunis. I'm the founder and CEO of the Center for Medical Technology Policy, which is an independent nonprofit that works to improve the quality and relevance of clinical research. I was previously chief medical officer at the Centers for Medicare and Medicaid Services, and I was there at the time when some of the predecessor treatments to Avastin and Lucentis for macular degeneration were introduced.

I just wanted to mention what hasn't been mentioned today, that prior to Avastin or Lucentis, the treatments that were available for macular degeneration only slowed the rate of degradation of vision. None of them actually reversed it, and yet Medicare was paying $2,000 to $3,000 a dose for those drugs. So Genentech deserves some credit for having developed the first two effective treatments for macular degeneration that actually improve vision.

The Medicare program can almost certainly spend less on drugs without any negative impact on health outcomes for Medicare beneficiaries. In my view, there are at least three important strate-

gies that can be pursued to achieve that, at least one of which has been discussed today.

First, Medicare should have the authority to link drug prices more directly to health outcomes. Secondly, Medicare should implement additional policies to promote high-priority clinical research such as the CATT trial, which has provided invaluable information that would support any sort of additional clinically sensitive policies that Medicare might introduce. And third, Medicare should develop a systematic policy approach to promoting drug innovation. The agency is certainly tremendously impactful on biomedical innovation, and there's a number of potential tools that the agency could use to promote innovation. Drug pricing is only one potential tool and probably not the most efficient tool for promoting innovation.

I recognize that the approaches to reducing drug spending that we're going to talk about today are not going to save the Medicare program from bankruptcy. These are going to require more fundamental payment reforms and systems innovations. However, I think it's still worth pursuing policy interventions that can save $100 million a year or $500 million a year, et cetera, even though by themselves, obviously, much more significant cost savings are going to need to be pursued.

So one relatively straightforward approach to reducing Medicare spending on drugs without negatively affecting patient outcomes would be to restore the agency's authority to pay the same price for drugs that produce similar benefits and harms. Medicare's regional contractors have been adjusting prices based on clinical effectiveness evidence for more than 15 years through their authority called least costly alternative.

The policy rationale is that Medicare, beneficiaries, and taxpayers should not pay more for a service or a drug when a similar drug can be used to treat the same condition and produce the same outcome at lower cost. There is no statutory provision giving specific authority or prohibiting the application of least costly alternative. CMS has considered its reasonable and necessary statutory authority to provide the needed legislative basis for this approach.

However, a recent court decision has constrained Medicare's ability to use LCA determinations, and therefore restoring that authority legislatively would restore Medicare's ability to adjust the prices of drugs to reflect their clinical outcomes. And as John Blum said earlier today, Medicare is moving towards a policy approach that links payment to outcomes, and there's no reason that that should not also apply to the outcomes for specific technologies, not just the outcomes that providers, hospitals, and others achieve.

The CATT trial underscores the importance for Medicare of having the capacity to rapidly identify, design, and implement trials on questions of substantial importance to the Medicare program. Senator Kohl was actually instrumental in addressing the challenges with handling copays for patients enrolled in the CATT trial and helped to craft language addressing this problem in the Medicare Improvements for Patients and Providers Act of 2008. It is my understanding that that statutory language has not been the basis for developing implementing instructions for Medicare, and therefore it remains as difficult as it was before to address those problems.

So one step that Medicare could take would be to develop implementing instructions for the language that you develop, Senator Kohl, in order to facilitate future trials like the CATT trial, which are still quite difficult to do.

Medicare could also promote critical research by making more systematic use of coverage with evidence development. Coverage with evidence development is a policy tool that links coverage of a drug or device or procedure with a requirement that patients receiving the service are enrolled in prospective clinical studies that would inform future decisions.

Medicare has the authority to implement coverage with evidence development, but because it's a vague statutory authority, the agency is reluctant to use that approach, and therefore their ability to support the costs of new clinical interventions in the context of clinical trials is extremely limited, and giving them explicit statutory authority to do so would substantially improve their ability to generate the kind of evidence that would give not only the Medicare program but patients and clinicians more of the kind of information they need to make good judgments based on clinical effectiveness.

And last, I see my time has expired, but I just wanted to make the point that Medicare does have an important influence on biomedical innovation just by the virtue of the huge role that it plays on the use of devices and other biomedical services globally. So it's impossible for them to avoid having an impact on innovation, and I think it would be extremely valuable for the Medicare program to take a comprehensive approach to looking at the relationship between various medical policies and biomedical intervention, think about the range of policy mechanisms through which innovation could be promoted, including potentially differential drug prices. But again, it seems that it would be useful to systematically look at ways that this could be done rather than defaulting to a singular approach of drug pricing.

So again, I thank you for the opportunity to share some ideas with the committee today.

[The prepared statement of Dr. Sean Tunis appears in the Appendix on page 69.]

The CHAIRMAN. Thank you very much, Dr. Tunis.

Ms. Swirsky.

STATEMENT OF LISA SWIRSKY, SENIOR POLICY ANALYST, CONSUMERS UNION, WASHINGTON, DC

Ms. SWIRSKY. Good afternoon. Consumers Union is the nonprofit publisher of Consumer Reports magazine. It has a long history of advocating for improving health care and lowering costs of drugs for consumers. So I very much appreciate the opportunity to testify in front of the committee today.

Our popular Best Buy Drugs report reaches 100,000 readers per month and provides rigorous evidence-based comparative effectiveness information on a range of commonly used drugs through our website. It's available through our website at www.consumerreportshealth.org. We're proud to say that we make that available free and that we do not accept any advertising.

Best Buy Drugs reports rely on credible systematic reviews of available clinical evidence conducted by expert researchers. We use price information from a leading health care data and analytics company. The value added that we think we bring to the table is that our editors and writers then translate this very complicated clinical evidence for our readers into consumer-friendly language and format, which is the hallmark of our publications.

To earn a Best Buy Drug designation, a drug must generally be at least as effective and safe as other medications in its class and less expensive. If the data show that the brand name drug is notably safer or works better than a lower cost medicine, that drug gets the Best Buy designation, and I think that's important to stress.

We have done a lot of work in the area of statins. Consumer Reports has found that for cholesterol lowering drugs, one of the most common medications, lower cost generics are just as effective and safe as more expensive brands. If you are taking this type of medicine for preventive reasons and you have not yet had a heart attack, the generic lovastatin is as effective, just as safe, and considerably less expensive than the brand Lipitor. A daily dose of Lipitor will cost an individual without insurance about $112 a month, compared to $4 a month for lovastatin.

Diabetes medication is another area where our organization has found low-cost alternatives to be effective and safe, and actually in this instance even safer. An older diabetes drug, generic metformin, is our Best Buy recommendation. It clocks in at about $4 a month and is a bargain compared to the pricey drug Actos, which would cost consumers $280 a month. Metformin is also the safest. Newer medications Actos and Avandia both carry a higher risk of increased heart failure. It's worth noting that FDA restricted Avandia's use, proving that you don't always get what you pay for when it comes to drugs.

We have found similar findings when it comes to pain medications, which you can read more about in our prepared testimony.

These real-life examples show how effective and safe generic drugs are and how they can save consumers precious dollars, and purchasers by the way. Our organization strongly believes that Congress should pursue policies that improve access to generic drugs, including passing Senator Kohl's and Senator Grassley's bill to end collusion between brand and generic companies to delay generic competition. CBO and the FTC have found that these paid-for delay agreements cost Americans billions of dollars.

In addition to promoting generics, Congress should do more about the safety and efficacy of drugs, including reforming Medicare and Medicaid payment processes to make use of available evidence that lower-cost drugs are as effective as more expensive drugs. We agree with Dr. Tunis that Congress should consider legislation to authorize CMS to reinstate the least costly alternative policy.

Congress may also create incentives to ensure that Part D formularies and state Medicaid formularies carry the generic as a preferred drug when there's strong evidence of comparability. Of course, it goes without saying that doctors must always have the ability to specify a brand alternative if that's in the best interest of the patient.

Finally, Congress should act to improve the way pharmaceutical companies convey safety and efficacy information to consumers so that they can better understand and use available clinical evidence to make better choices about their treatments. Consumers Union looks forward to working with Congress to improve the way patient safety and efficacy information is presented to consumers so that they can make better informed decisions about their choices. We believe in a lot of instances when consumers are provided and armed with good information, they will often choose the lower-cost option. A lot of times, that's just not what they're getting from the marketing.

In conclusion, I wanted to thank the committee for hearing me out, and we look forward to working with the committee.

[The prepared statement of Ms. Lisa Swirsky appears in the Appendix on page 79.]

The CHAIRMAN. Thank you very much, Ms. Swirsky.

Now Dr. Gottlieb.

STATEMENT OF SCOTT GOTTLIEB, M.D., RESIDENT FELLOW, AMERICAN ENTERPRISE INSTITUTE, WASHINGTON, DC

Dr. GOTTLIEB. Thank you, Chairman Kohl, Ranking Member Corker. Thank you for the opportunity to testify before this committee.

Over the past decade, the drug space stands apart from other segments of the healthcare industry in terms of how much the underlying business model has changed. The life sciences sector has undergone a fundamental transformation to focus on delivering more value and more basic innovation to consumers.

Industry pipelines have also had more new compounds in late-stage development than at any time before. More of these new drugs are aimed at fundamentally new targets, and more address unmet needs in medicine, including many orphan diseases.

But despite recent progress, challenges remain. There are still consumers priced out of health care. The cost of developing drugs is rising sharply, and new biotech company formation has fallen off. Too many diseases remain poorly treated.

So we must craft policies that provide proper incentives for new technology while making sure we are getting more value for programs like Medicare.

Any discussion of policies that have worked to bring more price competition to the prescription drug market and lower overall spending has to begin with Medicare's Part D prescription drug program. Competition between more than 1,000 drug plans has resulted in costs that are substantially less than what was first envisioned, wider use of generic medicines and deep discounts on branded drugs.

Now, I know there is discussion around imposing mandatory rebates in the Part D program. These are a form of price controls that distort commercial forces. Mandatory rebates create a strong incentive for companies to launch drugs at higher prices in anticipation of the payments that they will have to provide. These rebates also discourage additional discounting.

Moreover, as more beneficiaries come under these kinds of tacit price control regimes, it will erode the ability of health plans to use

competitive negotiations to move their market share and improve profit margins. This in turn will reduce their incentive to try and drive hard bargains with drug companies.

I know members of this committee have also considered proposals to give the staff of Medicare least costly alternative authority. There is nothing inherently wrong with a payer carefully judging the clinical data supporting the use of a particular medical product or service to determine what it will reimburse, but Medicare is no ordinary payer. Its decisions are widely followed. As such, Medicare has an outsized impact on what the U.S. patients will have access to.

If Medicare were to make clinical judgments about new technology at the time of their launch, it would also undermine the way innovation unfolds in the life sciences. In many cases, much of the innovation takes place post-market as new technology is introduced and demonstrate additional benefits from real-world use. Demanding early life cycle demonstrations of value, however measured, skews heavily against this sort of postmarket innovation.

We should also consider how past treatments we now view as profound advances would have fared under an LCA policy, and we should also consider how such a construct would affect future investment decisions.

Policies that encourage more price competition and more clinical competition between similar drugs can help drive more value for beneficiaries while encouraging more opportunities for new innovation. This gets me to the idea of merging Medicare's drug and medical benefits, folding Part B into Part D. There is good clinical and economic rationale for providing drugs under a single unified program. Many private plans have already merged their drug and medical benefits. Folding Part B into Part D could provide substantial savings to Medicare. The savings would be a result of greater therapeutic substitution between oral and injectable drugs, and more price competition between similar agents.

Now, moving Part B into Part D is enormously complex and full of potential damaging unintended consequences. It would need to be considered carefully. It is also worth noting that if doing it only invites more temptation to import price controls into the resultant drug program, that will erode competitive forces that ultimately drive value.

Moreover, not all the savings would actually accrue to Medicare. Some of it would need to be used to help offset the rise in premiums and out-of-pocket costs incurred by beneficiaries. Medicare would also have to create new codes to compensate doctors directly at a fair and sustainable rate for the cost of infusing drugs in their offices.

In conclusion, the drugs that are in late-stage development and have recently been launched are more promising than at any time in recent memory. Yet the model that has made life science successes possible is fragile. The decisions that we make about how we regulate these products and pay for their cost have direct effects on whether these endeavors get undertaken in the first place.

Thank you.

[The prepared statement of Dr. Scott Gottlieb appears in the Appendix on page 83.]

The CHAIRMAN. Thank you very much, Dr. Gottlieb.

We'll start now by asking everybody on the panel to respond to the question of where do we have the greatest opportunities, in your opinion, in Medicare to help lower the costs?

We'll start with you, Dr. Rosenfeld.

Dr. ROSENFELD. Well, I think in my testimony today I've outlined some important measures that Medicare can take. Medicare can address the 6 percent average sales price payment to physicians. It's really preposterous, if we think about it, that we should be paid a percentage of the cost of the drug. It develops a codependency between the clinician and the pharmaceutical industry.

Moreover, we should look at this disproportionate share hospital discount and ask why that isn't passed through to Medicare. And we should look at these unusual decreases in reimbursement that are still in place that incentivize the use of Lucentis in hospital-based settings.

Moreover, if drug companies offer rebates for increased use and the rate of increased use to physicians, it implies that physicians are not injecting the patients they should be and they have to be incentivized to do that. I doubt that's the case. Those rebates are really focusing on increased utilization by the physician, and if there is a rebate, it should be passed on to Medicare, and there should be a limit on how physicians purchase drugs with credit cards, and there should be an added transaction fee or that cost should be rebated directly to Medicare.

The CHAIRMAN. Thank you so much.

Dr. Adamis.

Dr. ADAMIS. It's beyond my area of expertise to make recommendations how we could lower costs for Medicare, but whatever is chosen by the committee and by Congress, we should make sure that it preserves a physician's ability to choose which medication they feel is most appropriate for a patient. I think that's very important.

And then the second piece of it, because this is my job, is to make sure that the incentives are still there to develop new therapies. We're working on drugs that have to be dosed just twice a year as opposed to injections every month, and we're working on drugs that work better than Lucentis, hopefully. I want to make sure those incentives stay in place so that we can consider those programs.

The CHAIRMAN. Thank you.

Dr. Tunis.

Dr. TUNIS. Yes, I think the several ideas offered in my testimony were some of the notions that I had in terms of what might, both in the short term and long term, lead to reductions in prices, and that included least costly alternative, giving the CMS, taking a more proactive role in promoting the kinds of studies like the CATT trial, and also looking at ways in which they could specifically incentivize high-value innovation.

The only thing I would add is you heard numerous times from John Blum how constrained they are with their existing statutory authority to do anything around negotiating drug prices or responding to or setting drug prices in any way to reflect the clinical benefits of a drug. I suspect if John was sitting here in a few years,

no longer working for the administration, he would have told you more explicitly what kind of authorities that they would like, but I imagine they would be things like least costly alternative authority and perhaps something like the ability to vary patient cost-sharing according to the value of a drug.

So a drug that's highly cost effective would have a very low copay so that patients would be inclined to prefer that for economic reasons, and drugs that are extremely expensive and produce very small incremental benefits would have higher copays so that the patients would have to take on more of the incremental costs, and that way the patients and clinicians are still free to make choices. It's just not all of the financial responsibility for the difference falls on the taxpayer; some of it falls on the patients as well.

Currently they have no ability to vary cost-sharing based on some judgment about the clinical effectiveness and costs of a drug.

The CHAIRMAN. Thank you very much, Dr. Tunis.

Ms. Swirsky.

Ms. SWIRSKY. I just would kind of highlight some of the same things that Dr. Tunis said about least costly alternative policy as being a ripe avenue. I also would agree with earlier comments about changing incentives in the Medicare program so that physicians aren't incentivized to promote or prescribe a higher-cost drug.

I'd also kind of reiterate or maybe go back to some of the examples we weren't able to use in my testimony because of time. But I think a lot of our examples of statins, proton pump inhibitors, which are basically heartburn medications which we can use very inexpensive generics as opposed to the expensive version of Nexium and so forth, all of these things are used by seniors, many seniors on a daily basis and I think are all really ripe for policies that promote their use.

The CHAIRMAN. Thank you, Ms. Swirsky.

Dr. Gottlieb.

Dr. GOTTLIEB. Thank you. I think the problems with Medicare are long-term problems, and they need long-term solutions with respect to changes in the structure of how Medicare pays for services. I think a lot of what we've done in recent years in terms of just across-the-board cuts or targeting individual products or freezing market basket rate increases doesn't tackle the long-term underlying problems in the Medicare program. In some cases, I think it makes true, fundamental reform more difficult.

I think Medicare looking for ways to try to tie what it pays for and how it reimburses to notions of value and looking at outcomes are the kinds of payment reforms we need to pursue. I think most of the spending, if you look at it, and most of the waste is probably on the services side and not on the technology side. I think it becomes much more, much easier to target the technologies and the introduction of technologies because you typically have one product and one sponsor, as opposed to trying to tackle reimbursement that affects hundreds, if not thousands of providers across the country. So that becomes politically much more difficult, even though that I think is what we need to ultimately address.

On the drug side, ultimately I think we need to concede the Part D plan is working. The competitive structures in Part D are bringing down the rate of inflation on small molecule spending and driv-

ing higher generic drug utilization. If we accept that, then most of the growth, if there is growth, is on the Part B side, and Mr. Blum testified that most of that is confined to just a handful of drugs. And quite frankly, those are oncology products. I think as a political matter it's going to be hard to really address some of the utilization in oncology. I think it will be hard to tackle that.

If I put forward one competitive market-based reform that I think could work to try to drive some higher-value utilization of drugs, it would be moving B to D. It would address the injectable drugs, but this would be a very hard reform, frankly, to implement.

The CHAIRMAN. Dr. Rosenfeld, as you know now in his prepared testimony, Dr. Adamis made reference to a study funded by his company, Genentech, which shows safety concerns for Avastin. I think you're in some disagreement with that, but do you want to talk a little bit about that?

Dr. ROSENFELD. At our annual research meeting, which is called ARVO, held in Ft. Lauderdale at the end of April, beginning of May, Dr. Gower presented data which had been much publicized before the presentation which has not been submitted for publication as far as I know, and it certainly isn't peer reviewed yet as far as I know, that there was increased risk of hemorrhagic stroke with Avastin.

When I attended that presentation, that's not what I heard. What I heard, and it's a common problem with Medicare databases—and I want to thank Dr. Ross Brechner from Medicare because he's taught me a lot about looking at Medicare databases and all the confounding variables that one needs to be concerned with, because the Medicare databases show you what doctors claim happened to their patient, but you know nothing about the patient.

So what Dr. Gower presented was that the overall stroke rate in the United States for Medicare beneficiaries was 0.4 percent. The stroke rate among patients getting Avastin was 0.4 percent, the same, but it was lower for the Lucentis patients, which was 0.26 percent, a difference of .15 percent.

Now, for those of us working with the Medicare databases, and in particular knowing the distribution of how Avastin is used and how Lucentis is used, those patients with Medicare and full secondary insurance are more likely to get Lucentis than Avastin. If you don't have secondary insurance and you have to pay out of pocket, you get Avastin. These are less wealthy patients and generally less healthy patients.

So looking at Dr. Gower's data, the Lucentis rate was lower than the average Medicare patient, while the Avastin rate was the same, suggesting either Lucentis protects against stroke, which seems unlikely, or it's a different population. And that, in fact, is what we're finding in our analysis of the Medicare database. There are so many confounding variables. You have to adjust particularly for wealth and concomitant diseases.

Now, the other report that was talked about was a report out of Duke, and contrary to what we heard today—in fact, I brought the paper along. And when these confounding variables are addressed, the concluding paragraph is as follows: "In conclusion, we found no evidence of increased risks of mortality, myocardial infarction,

bleeding or stroke among Medicare beneficiaries who received intravitreous Lucentis or Avastin for wet AMD."

So when authorities who deal with Medicare databases analyze the data, they understand that you have to do this analysis, this adjustment for confounding variables. It's an ongoing problem and something that we're acutely aware of in our ongoing analysis of the Medicare database.

So to answer your question, I do not believe there's any data at this point in time to suggest an increased risk with Avastin, though it needs to be monitored and studied further.

The CHAIRMAN. Dr. Adamis, your company has paid for a research study that purports to show safety concerns with the use of Avastin for macular degeneration. But the National Institutes of Health has provided the committee with a written statement that indicates they may not agree with these findings. Other experts consulted by the committee also believe that there are shortcomings in the methodology of the study that you all conducted. Is Genentech willing to address these criticisms?

Dr. ADAMIS. Of course. These were Medicare claims database studies. The first was the one that was sponsored by us at Johns Hopkins. It was an unrestricted grant, which means that Dr. Gower had full control over the data and the conclusions that she made. We had that study done because, to date, prior to CATT, there was zero data on the safety of Avastin versus Lucentis in large populations.

So if, for instance, the drug increases the risk of stroke by 1 percent, you can't learn that unless you study tens of thousands of patients. You don't have the power in the small number of patients studied in some other trials to detect that difference. And so the only way you can do that is with a Medicare claims database study.

And Medicare claims database studies are not conclusive. I agree with that. However, it was the best way to look to see if there is a safety signal. What was surprising to us was, when Dr. Gower completed her analysis, she in fact found the same signal that was found in the Duke study, and that was an increased risk of both stroke and death.

So when two studies that are very large—the Duke study looked at the 2006 patient Medicare claims database, and the Gower study looked at 2008 and 2009—show you the exact same signal, although not conclusive, you don't ignore it.

And then it was surprising when the CATT data came out; and although CATT was a 1,200 patient trial, it wasn't powered to detect those 1 percent differences. Nonetheless it showed this 29 percent increased risk of serious side effects, with 80 percent of them landing the patient in the hospital.

So now you have three large studies showing this, none of them definitive, but I don't think we can ignore them.

The CHAIRMAN. Okay. All right, Dr. Tunis, many people believe that more needs to be done to give the government the tools to address rising drug costs. In your time at CMS as chief medical officer, Dr. Tunis, what did you find were the biggest policy barriers to lowering drug costs and preserving high quality?

Dr. TUNIS. Again, I think this comes back to the statutory formulas that determine what Medicare, what price Medicare has to

pay for a new drug that are completely insensitive to how much incremental clinical benefit it provides. And one example that's fairly well known was the example of the drug Aranesp for anemia that was caused by cancer chemotherapy, which was a new version of a previous and very closely related to another drug called Procrit, which treated the same problem, anemia from cancer chemotherapy.

Because of the pricing systems in place at the time, Procrit would have been a substantially cheaper approach and achieve the same clinical outcomes as patients treated with Aranesp. But because Medicare was forced by statutory formula to pay 95 percent of average wholesale price for Aranesp, the Medicare program would end up spending $150 to $200 million more per year with no additional clinical benefit, no better treatment of anemia than if the program used only Procrit.

The agency, CMS, had no statutory authority to do anything about that, other than pay 95 percent of average wholesale price. What happened at the time was they used an authority in the outpatient payment system called the equitable adjustment authority to try to come up with a price for Aranesp that was what was appropriate based on getting the same clinical results. That was actually put in place. But then because of that approach and the Medicare Modernization Act, there was language put in that actually prohibited Medicare from ever doing that again.

And so it seems to me that whether it's the least costly alternative or some other version of statutory flexibility to set prices of drugs in some way sensitive to how much additional benefit, if any, is provided, would be a very important way for Medicare to be able to spend less on drugs and not harm Medicare beneficiaries in any way at all. It wouldn't be rationing. It would just be paying the least that the program could pay for a given level of clinical benefit.

The CHAIRMAN. All right. Before I turn it over to Senator Corker, Ms. Swirsky, Consumers Union recently advocated to instill a fiduciary responsibility on the pharmacy benefit managers, the PBMs. It would require them to work for employers and insurers rather than drug companies. So why did you take that position, and how important do you think it is?

Ms. SWIRSKY. I'm not sure where that information came from. We have not done anything on PBM since I've been there. It may be that that was something done prior to my coming on board. I've been at the organization for about a year. But I will be happy to find out and to make that——

The CHAIRMAN. Are you familiar with the issue?

Ms. SWIRSKY. A little bit, but I don't—we have not done anything on that recently.

The CHAIRMAN. All right.

Senator Corker.

Senator CORKER. Thank you, Mr. Chairman.

It's interesting sitting up here and listening to testimony, and Dr. Rosenfeld and Dr. Adamis seem like very good folks who have a very strong disagreement, yet the testimony from both of you is very credible.

The issue that Dr. Rosenfeld brought up regarding the rebates and the fact that physicians shouldn't be paid those rebates on some of the drugs he was mentioning that you make as a company, what is your response to that?

Dr. ADAMIS. When I was practicing medicine, you would prescribe a medicine based on what you thought was best for your patient, and you never thought about what it meant for your practice or your hospital, and I think that's the way it should be done around the country.

There are these rebates, and this can't be looked at in a vacuum because I have colleagues in South Carolina who don't prescribe Lucentis because there's a $140 tax on it when you use it, so it's a money loser every time you give it.

So as I said at the outset, if we could set the system up so we're not incenting or disincenting people one way or the other, and that really you approach the issue with equipoise and a doctor can just be free to choose based on the evidence, the scientific evidence, I think that would be the best of all worlds.

Senator CORKER. And so this is really—what you're leading to is that this is something that the Federal Government has set up regarding the rebates, not something your specific company is doing relative to trying to drive this product.

Dr. ADAMIS. No. I didn't mean to convey that. So there are rebates that the company provides, but they're——

Senator CORKER. But let me just say, if it's not to—if physicians should make decisions based on what's good for their patient, and you say the physicians really don't make decisions based on their own economic benefit, then why do you guys do that?

Dr. ADAMIS. So the rebates actually lower the cost of the drug to Medicare, because what happens is you're required to report that to Federal authorities, and that goes into a formula—and this is getting beyond my area of expertise—that calculates that average sales price. So the average sales price actually moves down, and the amount that Medicare reimburses moves down. So the rebates actually lead to a lower cost of the drug.

Senator CORKER. But this is not something that you're required to do; is that correct? By the Federal Government.

Dr. ADAMIS. No, but it's something that is pretty routine in the industry. But we're not required to do it.

Senator CORKER. Wouldn't it also lower the price to Medicare if you just charged a lower price without the rebate to the physician?

Dr. ADAMIS. Correct, it would.

Senator CORKER. I mean, again, you sounded pretty credible on the front end, but what you're telling me right now is not particularly credible.

Dr. ADAMIS. In what sense, sir?

Senator CORKER. Well, if the purpose in doing this is to lower the cost of the drug to the end user or the end payer, and you could get there the same way by just charging 6 percent less or giving a rebate to the physician who, I guess, keeps that, it seems to me that you are, in fact, paying that rebate to drive physicians to use your drug.

Dr. ADAMIS. I can't say that that is the purpose behind it. The way it's structured—and I'm getting out of my area of expertise be-

cause I don't set these programs up, and I don't work in the commercial area of the organization. But it's for high-volume users of the drug. They're the ones who are eligible for the rebate.

Senator CORKER. So they make even more money by prescribing your drug, and yet your testimony was that their compensation isn't what drives them to use your drug.

Dr. ADAMIS. I think the rebate, in a vacuum, if you looked at it that way, could be viewed as an incentive. But as I said, there are some jurisdictions, South Carolina being one of them, where actually even with the rebate it's a money loser. So in a perfect world it would be great if docs had an opportunity to prescribe what they want without any incentives.

Senator CORKER. But in a perfect world, you could do that yourself, right? I mean, in the world we live in today, which is not perfect——

Dr. ADAMIS. I can't control, our company can't control South Carolina's taxes.

Senator CORKER. But let's say in Tennessee, where I live——

Dr. ADAMIS. I don't know, sir.

Senator CORKER. I mean, am I missing something here? Am I——

Dr. ADAMIS. I'm just getting into an area that is not my area of expertise. I'm the scientist in the company. I work in development, and I came primarily to discuss CATT and the differences between Lucentis and Avastin.

Senator CORKER. And I really didn't come to chase the rebate issue. I just heard you mention it. So am I missing something, Dr. Rosenfeld?

Dr. ROSENFELD. Well, Dr. Adamis is correct. In South Carolina, it's a unique situation where there's a state tax on drug revenues, even Medicare Part B drug revenues. But in the rest of the country, the rebate is focused on high-volume users with the intent to increase their use even more.

Senator CORKER. So that a physician like you would prescribe that drug more than another drug because you'd make more money?

Dr. ROSENFELD. Well, I'm not in the rebate program, but I know several large-volume practices that make a lucrative sum every month from the rebate program.

Senator CORKER. I wasn't really planning to chase that, but it was a comment made, and I appreciate the discussion.

So, Mr. Gottlieb, on the idea of combining Parts B and D in Medicare and some of the difficulties you mentioned that might come with that, you threw it out there. It's a pretty big idea. What are some of the immediate issues you think that might be problematic with that type of a combination?

Dr. GOTTLIEB. I think the discussion around rebates gets us some of both the attraction of doing it and the complexities of doing it. I mean, part of why the rebates exist in the market at all, or even the spread on ASP, is to help compensate physicians for the cost of delivering drugs.

And I think the existence of these rebates in the market, it's hard to look at them in isolation around a particular product be-

cause the existence of rebates market-wide, and they exist marketwide, creates terrible distortions in the market.

If you look at what's going on in the 340B program, which was mentioned here, that has resulted in terrible gaming where the 340B hospitals are buying up local oncology practices for the purposes of capturing the spread on the drugs that they're able to acquire at a lower price and then reselling them at a higher price. And so they're capturing that revenue. So you're seeing oncology practices consolidating around 340B hospitals, which is probably the last thing we want to see as a public health matter.

So I think the existence of these things in the market across the board is creating bad distortions.

In terms of just moving Part B into Part D, I think it would be enormously complex but doable, and we've talked about it in the past. I worked at Medicare and Medicaid in the 2004–2005 timeframe, and it was something that was talked about, and talked about even in the context of MMA. Premiums would go up, out-of-pocket costs would go up for beneficiaries, so you'd have to offset that. You'd have to figure out a way to pay physicians directly for the true costs of the administration of the drugs. Somehow you'd have to, if you still do it under a buy and build model, where the physicians acquire the drug, somehow you'd have to offset the cost of that acquisition for the physician. There are probably financial arrangements that could do that, but you'd have to also pay for the infrastructure for the physician to be able to do that. Those are just some of the complexities that would ensue.

You'd also have the reality that for certain products that are truly breakthrough products for which there is no competition, under the Part D scheme you'd probably potentially see prices go up. I don't think that's necessarily a bad thing because I think it would reflect the fact that they're able to take price increases because they represent true innovation in the marketplace. But in other cases where there might be oral drugs that compete with injectable drugs, you might see more utilization of the oral agents, which would invariably provide cost savings to Medicare because it would be cheaper to deliver.

The last thing to keep in mind is that we're also entering an era right now where there's going to be multiple drugs on the market to attack a particular target. So if you have a target like VEGF or other kinds, CD20, CD30, there might be multiple agents that all attack the same target. You want the decision about using a particular agent to be driven by the clinical circumstances and what's best for the patient, not which scheme it's in. And right now you have examples where a decision might be driven by what scheme it's in, Part B versus Part D.

Senator CORKER. So those are a lot of complications. They seem like vague ones. As to the idea of combining B and D, it's a real idea, or is that just a throw-out idea?

Dr. GOTTLIEB. I think it's a real idea, and it's an idea we've talked about in the past. The reason I caveat it with all the complications is——

Senator CORKER. Sounds like one of these advertisements for drugs on television.

Dr. GOTTLIEB. What concerns me is that once it gets into a political context, some of these things that need to be addressed might not be adequately addressed.

So, for example, where is going to be the assurance that physicians are compensated for the cost of truly delivering the drugs in their office? We've seen under the physician payment scheme that physician costs have increased and payments have stayed stagnant, so their effective income has been eroded.

So those are the kinds of things that worry me, that once this kind of idea gets in the political context, the things that will truly make it successful and competitive won't be adequately addressed. What's the assurance that a future Congress two years from now isn't going to want to impose price controls in the Part D scheme to address the previously Part B drugs? There is no assurance there. And so you would want to see those things addressed in the legislation.

Senator CORKER. Dr. Tunis, you mentioned—you may have adequately discussed the notion of prescribing drugs based on outcomes, or paying for them based on outcomes, and we hear that a lot. You know, there was a lot of debate about that during the discussions regarding health care reform, and those of us who do what we do up here never were able to grasp a real way of making that happen with other providers. I know that CMS is working towards that end now, but do you want to expand any more on that notion? It's hard for me as a layman to understand how you really make that happen, especially as it relates to prescribing drugs.

Dr. TUNIS. So there's complicated versions of it, I guess, but in some ways the simplest——

Senator CORKER. And those probably won't work on us.

Dr. TUNIS. Right. I won't give you that one.

But the simplest version again I think comes back to some notion of reference pricing, least costly alternative, or what we did back in Medicare with the anemia drugs, which was called functional equivalence at the time, which is—but I'll give the example of what led to the court case that took away Medicare's ability to use this least costly alternative approach.

It was for two drugs to treat asthma in children and adults. But basically there was one drug that was a generic drug that was 10 cents to inhale it, and then there was another drug that was developed which was very closely chemically related that was $1.10, so 10 times the price. And when you actually looked at the clinical studies to see how much they opened up the lung airways, how much they freed up people's ability to breathe, it was close to identical. There was no clear information to suggest that there was a clinical reason for either a patient or a physician to want to use the more expensive one.

And so under those circumstances, after very careful review, the Medicare contractors tried to apply the least costly alternative approach, and basically to pay essentially 10 cents and you pick your drug. We'll pay 10 cents whether you use the really expensive one or the cheap one based on the fact that there's no clinical difference, and that was taken to court. And the reason, as I understand it, that the court decided that Medicare could not do that was because the Medicare Modernization Act told Medicare you

have to pay ASP plus 6 percent, and ASP plus 6 percent for the more expensive drug was 10 times the price.

So, you know, that's why in my testimony it seems that at a minimum, in situations where there's a pretty good level of confidence that you get the same clinical benefits for 10 cents or for $1.10 per treatment, there's no reason that the Medicare program should pay $1.10. The only argument that I've ever heard about why they should is that that extra money can then be circulated back to support innovation, or it creates an incentive for pharmaceutical companies to invest in new treatments, and that's why I added the last part of my testimony, which was, well, that certainly does encourage innovation. If you spend more money for things, people are going to want to invest more money to create them. But if it's not creating any more value for the Medicare beneficiaries, that doesn't seem like the best way to incentivize innovation necessarily.

I don't know if that cleared it up at all, but that's the——

Senator CORKER. It seems like that a competitive market and keeping things competitive, much as has been alluded to in earlier testimony regarding Medicare Part D, would actually drive towards that end anyway, would it not?

Dr. TUNIS. I think because in Medicare Part D the prescription drug plans have a lot more freedom to use pricing tools to manage their benefits. That's exactly how they get the costs down. When you have drugs that are paid for under Part B and under the statutory constraints or lack of statutory flexibility is where you get these what seem like unjustifiable price differences, I don't think that would happen if those were products——

Senator CORKER. In Medicare Part D.

Dr. TUNIS. Yes, exactly.

Senator CORKER. So we had another Senator talk about some of his concerns about IPAB and just decisions that can be made by groups of unelected folks, if you will.

But using the same line of thinking, if you were a patient who needed chemotherapy of some type and, to use the same analogy, there was only a 20 percent chance that it was going to be effective for you but it was the only thing left, the only chance left, if you will, for some type of treatment that might work on that type of disease, how would you employ the kind of thing you're talking about?

Dr. TUNIS. What I'm talking about wouldn't really come into play there just because we're talking about two choices that are clearly equivalent. In the case where you're talking about something that's better, a drug that's better by some measure and lots more expensive, so it comes up questions about is it actually worth the money, for example, the recent Medicare decision to pay for Provenge to treat prostate cancer is a good example, where it's $90,000 or $100,000 for a treatment that maybe on average extends life for three to four months, and that's the kind of situation where really nobody wants to touch it. And the fact that the National Institute of Clinical Excellence in the U.K. might well decide that that's not a cost-effective use of collective resources doesn't seem to me like a place that you all want to venture into or I would really want to venture into.

When you really look at it in terms of all of the downward pressure that there's going to be on finding some way to constrain spending in the Medicare program over the next 10 years, it's hard to believe we're going to be able to do all of that without getting into some sensitive areas of not being able to provide everything that doctors and patients might decide is what's in their best interests. But I'm not able to provide a lot of solutions today.

On that kind of example, the one thing I will say is, to some degree, all of the different options that are out there for trying to constrain spending are just a matter of who you decide is going to take on their shoulders the weighing of costs against benefits. And when the government does it, it's government bureaucrats interfering with clinicians and patients. When you put financial incentives on doctors to be cost conscious, then essentially—there's no polite way of saying it—doctors don't want to ration care for their patients. And if you put more cost sharing on patients by higher deductible plans or adjusting benefit designs, you're just asking patients to ration their own care.

So it's not a matter of whether or not you ration. It's just who you decide is going to be the rationer.

Senator CORKER. Mr. Chairman, I want to thank you for the hearing. It's amazing to me. I know that most of these witnesses were chosen by the majority, which is the way things work around here. But it's amazing to me that in every case, it seems like they believe that competitive market forces like we have in Medicare Part D are what drive choice and drive better decisions, and that rebates or things that are distortive like government getting involved in setting rebate levels and making choices really foul the process up tremendously.

So I thank you for this very clear signal that everything we need to do, from these witnesses anyway, that everything we need to do needs to move us towards much, much greater competition and innovation, and I couldn't agree with that more, and I thank you for providing these outstanding witnesses.

The CHAIRMAN. Well, I too think, Senator Corker, that we've had a great hearing and shed much light on the question of prescription drugs, their costs, and what we might do to alleviate those costs on behalf of consumers. I think that my conclusions might be somewhat different than Senator Corker's, but that's why we're here and that's why we debate and hopefully move the ball forward.

But you've all done a great job of shedding light on the issue, and we thank you for coming.

[Whereupon, at 4:14 p.m., the hearing was adjourned.]

APPENDIX

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STATEMENT OF

JONATHAN BLUM

DEPUTY ADMINISTRATOR AND

DIRECTOR, CENTER FOR MEDICARE

CENTERS FOR MEDICARE & MEDICAID SERVICES

ON

SUSTAINING THE MEDICARE PROGRAM THROUGH LOWER COSTS

BEFORE THE

UNITED STATES SENATE SPECIAL COMMITTEE ON AGING

JULY 21, 2011

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U.S. Senate Special Committee on Aging

Hearing on Sustaining the Medicare Program through Lower Costs

Jonathan Blum

July 21, 2011

Chairman Kohl, Ranking Member Corker, and distinguished members of the Special Committee, thank you for the opportunity to discuss the Medicare Program with you, and more specifically, the laws guiding how Medicare pays for prescription medications. The Administration is committed to protecting and strengthening Medicare, and reducing health care costs in the Medicare program, which will provide care to approximately 50 million Americans in 2012. Paying appropriately for prescription drugs is an important part of that commitment.

The Affordable Care Act reforms the health care delivery system, reduces health care costs, and extended the solvency of Medicare. The Affordable Care Act also builds a stronger Medicare program by improving access and coverage of life-saving prescription drugs, through lower prescription drug costs for beneficiaries.

Medicare pays for prescription drugs in many ways: drugs provided as part of inpatient or outpatient hospital care are provided under Part A and Part B, respectively, physician-administered drugs are generally paid under Part B and other prescription drugs are paid under Part D.

Medicare Prescription Drug Benefit: Part D

The Medicare Part D pharmacy drug benefit program was established under the Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (MMA) P.L. 108-173. Part D is designed to provide beneficiaries with drug coverage through private prescription drug plans. Under Medicare Part D, private insurers contract directly with CMS to provide prescription drugs, certain vaccines, insulin and certain medical supplies associated with the injection of insulin. CMS pays plans per enrollee, and the plans compete for enrollees on the basis of premiums and coverage. Medicare subsidizes about 75 percent of the average cost for basic

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coverage, with beneficiaries who choose to enroll in the voluntary Part D benefit paying the balance through monthly plan premiums. Beneficiaries also have the option to choose "enhanced plans" that have higher premiums and more generous coverage than basic plans.

Each plan year, beneficiaries decide whether they want to remain in their current plan or enroll in a different plan that best meets their unique health and specific prescription drug needs. The Administration values giving beneficiaries that choice, and in recent years, CMS has worked to simplify the plan selection process for beneficiaries so that the differences between health plan and prescription drug coverage choices are easily understandable to the Medicare population. Beginning in 2011, CMS adopted its meaningful differences policy whereby CMS will approve a bid submitted by a Medicare Advantage (MA) organization or Part D sponsor only if the plan's benefits or cost sharing are substantially different from those of other plans offered by the organization or sponsor in the area. In making this determination for Part D, CMS takes in to account key plan characteristics such as premiums, cost-sharing, formulary structure, or benefits offered. These policies are in place to ensure that CMS is providing a meaningful choice to beneficiaries.

The 2011 deductible is $310 and average monthly premiums for standard Part D basic coverage is $30.72, an increase of less than $2.00 from 2010. In addition, even with changes to simplify the plan selection process, there are at least 29 Part D plans available in each Region. For low-income beneficiaries, varying degrees of cost-sharing are available with co-payments ranging from $0 to $6.30 and low to no monthly premiums. Beneficiary premiums in the Part D program are stable even though the costs of prescription drugs have increased by 4.2 percent in the last year.[1] Part D plan sponsors have been able to manage benefit costs through the widespread use of generic drugs, refinements of drug formularies, and utilization management.

The average Part D premium for basic coverage in 2006 was $23.42 and is currently $30.72, an increase of less than $8.00 since the inception of the Part D program. For 2012, there will be about 32 million beneficiaries enrolled in Medicare Part D, including about 11 million low-

[1] Bureau of Labor Statistics, Consumer Price Index, Unadjusted % change June 2010 to June 2011 for prescription drugs: http://www.bls.gov/cpi/cpid1106.pdf

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income subsidy beneficiaries. About 66 percent of those with Part D coverage are enrolled in stand-alone Part D prescription drug coverage and 34 percent are enrolled in a Medicare Advantage Prescription Drug Plan (Medicare Part C). Overall, approximately 87 percent of all Medicare beneficiaries receive prescription drug coverage through Medicare Part D, employer-sponsored retiree health plans, or other creditable coverage. Beginning in 2011, Part D premiums have been adjusted for beneficiaries whose modified adjusted gross income exceeds thresholds established for the Part B income-related premiums.

CMS pays Part D sponsors based on their bids to provide basic prescription drug coverage to beneficiaries. Part D plan sponsors are required to cover two drugs in every drug class and all drugs in six "protected" classes. These "protected" classes are: Antiretroviral; Anti-Cancer; Immunosuppressives; Antipsychotics, Antidepressants; and Anticonvulsants. In order to ensure that beneficiaries have access to a full array of medications to manage their health care conditions, each year CMS reviews Part D formularies for adequacy to ensure plans' pharmacy benefit packages are not being discriminatory. Further, a coverage determination can be requested by a beneficiary, by an appointed representative, or by the prescribing physician on behalf of the Medicare beneficiary. If a beneficiary does not agree with the initial coverage determination made by the Part D plan sponsor, there is a formal process through which the beneficiary has the right to appeal the coverage determination of a non-formulary drug, request an exception to a plan's tiered cost sharing structure, and request an exception to the application of a cost utilization management tool.

Part D: Actual Costs

According to the Medicare Trustees, total 2010 benefit costs ($61.7 billion) were almost identical to those projected last year, and were about 6 percent lower than the projection from the 2009 report. Further, overall net Medicare costs for the Part D program in 2011 are approximately 40 percent lower than what was initially projected upon enactment

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Part D: Management Tools for Lowering Costs

Most Part D plans use drug formularies to assist in the overall management of drug costs. Plans negotiate rebates (price concessions) with brand-name drug manufacturers in exchange for preferential formulary or "tier placement" that reduces the copayment for the beneficiary.

Part D plans are able to manage drug costs using utilization management techniques such as prior-authorization; "step therapy," which encourages lower-cost therapeutic alternatives by requiring beneficiaries to try generics or lower-cost alternative drugs first; and quantity limits, with appropriate appeals process in place to protect beneficiaries. Plans also manage drug costs by encouraging beneficiaries to use in-network pharmacies and mail order programs.

Part D formularies include generic drugs. Part D plans promote generic drug use by placing them on the first or second tier that contains minimal or zero cost sharing. As a result, generic utilization in Part D far exceeds private insurers' generic utilization rates and has grown over time. In 2006, the first year of Part D, generic utilization was 60 percent, as a percentage of drug fills, and in 2009 generic utilization had risen to 72 percent.[2]

Part D: Affordable Care Act Improvements

As a result of new provisions in the Affordable Care Act, people with Medicare have already received relief from the cost of their prescription medications. For 2010, nearly 4 million eligible seniors and people with disabilities who reached the donut hole received help through a one-time, tax-free $250 rebate check to help reimburse them for out-of-pocket drug costs. And beginning this year (2011), applicable beneficiaries have been automatically receiving a 50 percent discount on covered brand name drugs in the Part D coverage gap, or "donut hole." Almost half a million individuals enrolled in Medicare's prescription drug benefit who have reached the donut hole have saved an average of $545 each, for total savings to beneficiaries of more than $260 million, so far this year. People with Medicare Part D will pay a smaller share of their prescription drug costs in the coverage gap every year from now until 2020, when the coverage gap will be closed.

[2] http://www.cms.gov/Dashboard/DBRD/list.asp#TopOfPage

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Medicare Drug Benefit: Part B

Medicare Part B coverage is defined by statute and includes a limited number of prescription drugs under three basic categories:

- **Drugs that are administered by a physician or under physician supervision.** Most Part B drugs are paid under this "incident to" provision, where the physician buys the drug, administers it and then bills for it. These are typically injectable drugs that are administered in a physician's office, for example injectable prostate cancer drugs; drugs used to treat cancer and side effects of cancer medications; injectable drugs used to treat rheumatoid arthritis; and drugs used to treat age-related macular degeneration, the most common cause of blindness among older Americans. Part B also covers these types of drugs when furnished in a hospital outpatient setting.

- **Drugs administered through durable medical equipment (DME) such as nebulizers or IV pumps.** For example, inhalation drugs, such as albuterol sulfate and ipratropium bromide, are frequently administered through a nebulizer, when used in a home setting.

- **Specific drugs covered by statute.** The list of Part B drugs covered by statue includes a variety of items that are administered in specified settings, including: certain drugs used for the treatment of end-stage renal disease (ESRD) furnished by dialysis facilities; certain oral anticancer drugs; certain oral antiemetic drugs; drugs for beneficiaries with a Medicare covered organ transplant; influenza, pneumococcal, and hepatitis B vaccines; as well as intravenous immune globulin G (IVIG) used to treat primary immunodeficiency in the homecare setting.

Part B generally does not cover drugs that are self-administered.

Part B: Payment for Drugs

The Medicare Prescription Drug, Improvement, and Modernization Act of 2003 (MMA) P.L.108-173, the statute that created Part D, substantially revised Medicare payments for Part B drugs. Prior to the MMA, payment for many Part B drugs was calculated from the Average

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Wholesale Price (AWP). The MMA authorized the Average Sales Price (ASP) methodology which calculates payments from manufacturers' reported sales prices.

Numerous reports by the MedPAC, HHS Office of Inspector General (OIG) and the Government Accountability Office (GAO) indicated that Medicare's AWP based payment was significantly higher than physician acquisition costs for these drugs.[3] The difference between Medicare's payment and acquisition costs is referred to as "spread". We believe that physicians used this spread in order to cross subsidize other expenses.

The MMA revised the system, changing Medicare's payment both for Part B drugs and their administration. The MMA specifies that Medicare's payment for most Part B drugs be 106 percent of the volume weighted average of manufacturers' Average Sales Price (ASP). The ASP-based payment rates, commonly known as "ASP + 6," became effective January 1, 2005.

Part B: Payments through ASP

The ASP is the average of each manufacturer's sales price, net of most discounts, rebates and other price concessions. The ASP accounts for all sales from a manufacturer to all entities within the nation who purchase the drug from the manufacturer. Certain low price sales are not included in the manufacturers' ASP. Manufacturers report their ASPs to CMS on a quarterly basis. CMS takes each manufacturer's reported ASP for each National Drug Code (NDC) that is assigned to a billing code and weights it by the volume of sales of all NDCs assigned to the billing code and then determines the ASP-based payment rate for each billing code.

The statute requires that the Medicare payment amounts are updated each quarter based on the most recent data available, which is data from the second previous quarter. For example, Medicare ASP payments for the quarter beginning July 1 are based on sales of the drugs from January through March. Sales data must be reported to CMS no later than 30 days after the end of the quarter. After CMS receives manufacturers' submissions, CMS compiles the data and within a few weeks, calculates the rates, checks potentially erroneous data submissions with manufacturers, makes corrections, publishes the rates, and makes them available to the Medicare

[3] http://www.medpac.gov/publications%5Ccongressional_reports%5CJune03_Ch9.pdf

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claims processing contractors' systems. Unlike most Medicare price files which are updated on an annual basis, the ASP files are updated quarterly. This allows the Medicare payment rate for Part B drugs to more accurately reflect the most current market conditions.

Part B: Drug Coverage

In accordance with Section 1862(a)(1)(A) of the Social Security Act, CMS makes coverage decisions based upon whether a treatment is "reasonable and necessary ." We do not consider cost as part of this decision. Payment for reasonable and necessary uses of drugs can include both FDA approved uses, as well as uses that are supported by evidence but not evaluated and approved by the FDA.

Part B: Spending

For 2010, the preliminary estimate of Medicare allowed charges for the approximately 800 drugs paid for by Medicare Part B contractors that are administered incident to a physician's service or in conjunction with DME was $12.5 billion. Most of the current spending for these Part B drugs is concentrated in less than 10 percent of the approximately 800 covered drugs. For example, of the $12.5 billion spent in 2010, 13 drugs accounted for 50 percent of spending, 34 drugs accounted for 75 percent of spending, and 70 drugs accounted for 90 percent of spending. From 2005 to 2010, spending on Part B drugs increased approximately 24 percent.

In 2010, the top two drugs, ranibizumab (Lucentis) and rituximab (Rituxan), accounted for an estimated 16 percent of spending for these Part B drugs.

Most of the highly utilized Part B drugs do not have generic equivalents. When generic equivalents become available, payment amounts can and often do drop significantly (see Generic Impact table). A number of recent biological approvals and drugs in the pipeline for approval will affect Part B spending in the near future. High cost items, such as Provenge and Jevtana (products used to treat advanced prostate cancer), and Benlysta (a biological used to treat lupus), are expected to add to Part B spending.

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Lucentis and Avastin

According to the National Eye Institute (NEI), a component of the National Institutes for Health (NIH), Age-Related Macular Degeneration (AMD) is the leading cause of severe vision loss in people aged 60 and older. "Wet" AMD occurs when new, but abnormal, leaky blood vessels form under the central part of the retina, the macula. Leakage of blood and fluid raises the macula and destroys central vision.[4] Nearly 2 million American have a visual impairment as a result of this disease, and more than 7 million are at increased risk for vision loss.[5]

Lucentis was approved by the Food and Drug Administration (FDA) in 2006. The clinical trials, upon which FDA based its approval, showed that Lucentis stabilizes and in some cases improves vision for people with "wet" AMD.[6] Previous treatments for "wet" AMD, including the drug Macugen and an expensive laser procedure, did not improve a patient's vision.

Lucentis' manufacturer, Genentech Inc., also produces Avastin, a drug approved by the FDA in 2004 for the treatment of advanced colorectal cancer. Avastin is closely related to Lucentis and is used off-label to treat AMD. Similar to Lucentis, Avastin works by blocking formation of abnormal blood vessels.[7] Off-label prescribing of FDA-approved drugs is a common practice among physicians, and once FDA has approved a drug for one use, physicians can choose to prescribe the drug for another (unapproved) use for his or her patients. Lucentis and Avastin are injected directly into the vitreous (the gel-like filling inside the eye) in a physician's office, but there is a significant price differential as Lucentis costs up to $2,000 per injection and Avastin can be less than $50 per dose, depending upon how it is billed by the physician. A two year course of therapy with Lucentis may cost as much as $48,000.

While Avastin is not FDA approved for ophthalmic use, the recent, one-year published results of the NEI-sponsored randomized clinical trial comparing Lucentis and Avastin in over 1,100 patients, did not detect a statistically significant difference in the effectiveness of these two drugs

[4] http://www.nei.nih.gov/health/maculardegen/armd_facts.asp#more
[5] http://www.nei.nih.gov/news/pressreleases/022208.asp
[6] ibid
[7] ibid

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in the treatment of AMD.[8] Medicare will cover Lucentis injections as in-office procedures, but unless a person carries secondary insurance the co-payment may be as high as $400 per treatment. Medicare also covers treatments with Avastin through off-label prescribing, as described above, with a copayment of less than $20.

Conclusion

CMS continues to operate the Part B and D programs consistent with the statute and governing regulations. Prescription drugs, both those covered by Medicare Part B and Part D, account for a large portion of Medicare spending, and we are working to make sure that we are paying appropriately for drugs. We plan to continue monitoring payment for and access to Part B drugs. CMS and other agencies within HHS are continuing to work with all interested parties to ensure that patients receive appropriate and high quality care. We look forward to continuing to work with Congress on our ongoing efforts to preserve and protect Medicare for future generations. I look forward to answering any questions you may have.

[8] http://www.nei.nih.gov/news/pressreleases/042811.asp

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Effect of Generic Competition on ASP Pricing for High Volume Part B Drugs

Although many of the high dollar volume drugs and biologicals that are paid under Medicare part B are single source (branded) and their ASPs are not influenced by generic competition, when generic competition occurs, the decrease in ASP can be substantial. The table below illustrates the actual decreases in ASP for several "top 50" ASP drugs, four quarters after the introduction of a generic counterpart.

Drug	Common Use	Allowed Charges Year Prior to Generic Counterpart	Change in Price (by quarter) compared to Allowed Charges Prior to Introduction of Generic Counterpart				Comment
			1st qtr price change (%)	2nd qtr price change (%)	3rd qtr price change (%)	4th qtr price change (%)	
ELOXATIN	Treat colorectal cancer	$326 million	-22%	-30%	-54%	-52%	ASP has rebounded to baseline due to withdrawal of generics (patent litigation agreement)
CAMPTOSAR	Treat colorectal cancer	$124 million	-41%	-71%	-83%	-86%	ASP has continued to fall; currently less than 10% of baseline
CELLCEPT	Oral immunosuppressant	$180 million	-28%	-24%	-45%	-61%	
PROGRAF	Oral immunosuppressant	$256 million	-10%	-18%	-18%	-19%	ASP has continued to fall; currently 30% of baseline
PULMICORT RESPULES	Nebulized asthma treatment	$267 million	-24%	-33%	-23%	-35%	

- The "Allowed Charges" column reflects the impact of both price and utilization on Medicare spending, while the "Change in Price" columns reflect changes to price only.
- The price drop associated with Camptosar is unusually high. No definitive cause for this price drop has been identified, but may be linked to the number of generics in the marketplace.
- Cellcept and Prograf payments are made to pharmacies.
- Pulmicort is dispensed by pharmacies/DME suppliers.
- No biologicals are on this list. Price decreases for biologicals/biosimilars, once they become available on the market, may be of lesser magnitude because manufacturing facilities for biologicals are believed to be much more expensive to build and operate than facilities that make chemical-based drugs.

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A Prescription for Savings: Reducing Drug Costs to Medicare
Thursday, July 21, 2011
Dirksen 106

Testimony before the Senate Special Committee on Aging
Philip J. Rosenfeld, MD PhD
Professor of Ophthalmology
Bascom Palmer Eye Institute
University of Miami Miller School of Medicine

Chairman Kohl, Ranking Member Corker, and other members of the Committee, thank you for inviting me today to testify on this important topic. I'm Dr. Philip Rosenfeld, Professor of Ophthalmology at the Bascom Palmer Eye Institute of the University of Miami Miller School of Medicine. I joined the faculty 15 years ago after completing both my MD and PhD degrees at the Johns Hopkins School of Medicine in 1988. I then completed a research fellowship and ophthalmology residency program at the Massachusetts Eye and Ear Infirmary of Harvard Medical School followed by a vitreoretinal surgical fellowship at the Bascom Palmer Eye Institute where I stayed on as a faculty member. I am a molecular biologist, a geneticist, and a retinal specialist, but I am not a specialist in healthcare policy. I do bring to the discussion a real-world perspective on the forces that influence the choice between two commonly used drugs, with very different costs, for the treatment of wet age-related macular degeneration (AMD) and how changes in the way clinicians are compensated when using these drugs could significantly reduce the cost to Medicare for all physician-administered drugs. These statements are my own opinion, and not those of the University of Miami or Bascom Palmer Eye Institute

Wet Age-Related Macular Degeneration
AMD is the leading cause of irreversible blindness worldwide among the elderly. It's a disease that usually causes slow, progressive vision loss after the age of 60. Patients rarely go totally blind, but they eventually lose their central vision and become legally blind. Central vision is the vision we need for reading, driving, recognizing faces, and performing even the simplest visual tasks. The disease is always in both eyes. We know the disease runs in families, and many of the genes that cause the disease have been identified. We also know that poor nutrition and smoking accelerate the disease, while a healthy lifestyle can slow the disease. What's important for our discussion today is that this disease starts off slowly as dry AMD, and then it can convert to wet AMD. The term "wet" refers to the abnormal growth of blood vessels in the back of the eye that leak and bleed. This leakage and bleeding accelerates the onset of blindness.

Avastin and Lucentis
Based on the groundbreaking scientific research performed at Genentech and elsewhere, we now know the factor responsible for the growth of abnormal blood vessels in wet AMD. It's the same factor responsible for blood vessel growth into

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cancers, allowing them to grow even larger and metastasize. Genentech developed two fabulous drugs that block this growth factor and prevent the formation of these abnormal blood vessels. These two drugs are known as Avastin and Lucentis. Both drugs are derived from the same mouse monoclonal antibody specifically developed by Genentech to block the growth factor. Avastin is a full-length form of this antibody, originally developed for colon cancer treatment, and Lucentis is a smaller piece of this antibody known as an antigen-binding fragment, which was developed to treat wet AMD. Avastin is infused through an arm vein every 2 weeks, while Lucentis can be injected every month into the eye. Avastin was FDA-approved for cancer treatment in February 2004, while Lucentis was approved for the treatment of wet AMD in June 2006.

Based on my extensive review of the scientific literature, my scientific training, and my extensive experience using Lucentis in clinical trials, I approached Genentech and asked to use Avastin to treat wet AMD due to its molecular similarity to Lucentis. This was 3 years before the FDA approved Lucentis. Genentech did not provide assistance, so in 2004, I initiated a small clinical study exploring the use of systemic Avastin for wet AMD.[1,2] Encouraged by the success of systemic Avastin, I sought additional support from Genentech for a larger study. When that support failed to materialize in May 2005, we reached out to colleagues all over the country to organize a multicenter clinical trial. While designing this larger trial, I realized an injection of Avastin into the eye should be theoretically equal to an injection of Lucentis into the eye. When injecting Avastin into the eye, I used 1/500th the amount of drug used in a systemic (arm) infusion of Avastin. As a result, an eye injection was both safer and cheaper than an arm infusion of Avastin because during an arm infusion, the entire body is exposed to the drug at high levels. When Avastin is injected into an eye, the drug is taken directly from its vial and placed into a syringe just like Lucentis.

Initially, this off-label use of Avastin was offered to patients as salvage therapy in patients who had failed standard-of-care treatment at the time and there was no hope of avoiding blindness.[3-5] This was before Lucentis was available. The treatment was successful, and one patient led to many more patients. Our success in treating patients with intraocular Avastin for wet AMD spread nationally and then globally leading to the use of Avastin eye injections in a wide-range of retinal diseases. This rapid spread of Avastin in 2005 was fueled by the availability of Avastin worldwide, its apparent efficacy, its low cost, and the fact that Lucentis was not yet approved, though the preliminary results with Lucentis had been promising. When a pharmacy follows strict sterile guidelines, as mandated by the United States Pharmacopeia, a syringe of Avastin should cost between $20 and $40. In contrast, a syringe of Lucentis now costs about $2000. As a result, even after the approval of Lucentis in June 2006[6-8], Avastin continued to be the preferred drug for the treatment of eye diseases and is viewed worldwide as the affordable, low-cost alternative to Lucentis. In the U.S., the Medicare allowable payment per dose is about $2000 for Lucentis and $50 for Avastin.

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Comparison of Age-Related Macular Degeneration Treatment Trial (CATT)
Over the past 6 years, controversy has swirled around the off-label use of Avastin for eye diseases, and this controversy escalated once Lucentis was approved in 2006. Since 2005, over 1500 scientific papers have been published in peer-reviewed journals exploring the safety and efficacy of Avastin in the eye. Despite all this published information and the clinical perception that Avastin and Lucentis were similar with respect to safety and efficacy, we did not have definitive data comparing Avastin and Lucentis in a large clinical trial. Survey after survey showed that the majority of patients in the U.S. were being treated with Avastin even in the absence of this definitive data. All that changed when the results of the Comparison of Age-Related Macular Degeneration Treatment Trial (CATT) were published in May 2011 in the New England Journal of Medicine.[9, 10] Dr. Daniel Martin, chairman at the Cole Eye Institute of the Cleveland Clinic, chaired this National Eye Institute-sponsored multicenter clinical trial. This study compared injections of Avastin and Lucentis in 1200 patients with wet AMD. After one year, monthly injections with Avastin were shown to be equivalent to monthly injections with Lucentis, and "as-needed" injections with Avastin were shown to be equivalent to "as-needed" injections with Lucentis. The study now continues for a second year. Contrary to popular expectations, I do not think these results will change the use of Avastin and Lucentis in the U.S. My opinion is based on research performed in collaboration with Dr. Ross Brechner, a lead medical officer and ophthalmologist at the Centers for Medicare and Medicaid Services (CMS). I believe most clinicians have decided to use one drug versus the other based on existing financial incentives and disincentives.

Avastin and Lucentis in the United States
In 2009, Dr. Brechner and I initiated a study to identify the use of Avastin and Lucentis among Medicare fee-for-service beneficiaries with wet AMD.[11] We reviewed the 100% Medicare Part B claims file from 2008 and found that approximately 60% of Medicare beneficiaries received Avastin while 40% received Lucentis. These numbers were virtually identical to the annual surveys previously performed by the American Society of Retina Specialists (ASRS). During 2008, we found a total of 841,782 eye injections were performed. CMS paid $20,290,952 for the 60% given Avastin and $536,642,692 for the 40% given Lucentis. While Avastin accounted for 60% of the injection volume, it was responsible for only 3.6% of the drug payments associated with treatment. The use of Avastin saved CMS over $800 million in 2008 alone. This research has been published, and we have continued our investigation by examining the 2009 database. In 2009, the total number of injections increased to about 1 million, with Avastin maintaining its 60% share of the market.

Dr. Brechner and I also explored the use of each drug throughout the country, and we found enormous variability on a state-by-state basis. In the western U.S., Avastin was used more frequently while in four central plains states (Nebraska,

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Kansas, North Dakota, and Iowa) Lucentis was used more frequently. In the South, only Florida and Tennessee used mostly Lucentis, while in the Northeast, the states of Pennsylvania, New Jersey, Vermont, Connecticut, and Maine used mostly Lucentis. Overall, 11 states use Lucentis more than Avastin, while the remaining 39 states used Avastin more often. Given the fact that CMS reimbursed for both Avastin and Lucentis in wet AMD, how can we explain the variability seen throughout the U.S.?

The use of one drug versus the other didn't seem to depend on the state's financial prosperity, its political party affiliation, nor the urban versus rural distribution of its population. If it was solely a question of ethics behind the use of on-label versus off-label drugs, then why would there be such variability throughout the U.S.? By exploring the differences in the use patterns of these drugs, we wanted to take this unique opportunity to better understand the forces influencing clinicians to choose between similar treatments with very different costs.

How do clinicians choose between Avastin and Lucentis?
Among the fee-for-service Medicare population, the choice between Avastin and Lucentis should be between the physician and the patient. If we assume that all clinicians and patients are informed about these drugs, in particular the differences between on-label and off-label treatment, and if we assume that the clinician has equal access to both drugs, then we wanted to identify the forces influencing the decision. The most obvious influence is cost, and cost becomes a very important issue when the patient does not have secondary or Medigap insurance coverage to pay the 20% balance not covered by Medicare. Not surprisingly, when we examined the CMS database, we found a correlation between the use of Avastin and the absence of this Medigap coverage. After all, the co-pay for Lucentis would be about $400 while the co-pay for Avastin would be $10, and these injections are performed as often as every month. However, this group of patients represented only a minority of patients receiving Avastin. The majority of patients had secondary insurance, so the cost should not have mattered to the patient, yet they still received Avastin. While the cost didn't matter to the patient, perhaps the cost mattered to the physician. In some practices, the cost of offering Lucentis was a financial hardship, while in other practices, the use of Lucentis turned out to be a financial windfall. As a result, we became increasingly interested in the forces that incentivized clinicians to choose Lucentis.

CMS Incentivizes Clinicians to Choose the Most Costly Alternative
Medicare Part B reimburses for physician-administered drugs such as Avastin and Lucentis. This is different than prescription drugs, which are reimbursed by Medicare Part D. In Medicare Part B, physicians receive a payment from Medicare that covers the cost of the drug when they administer the drug. In addition, Medicare supplements the payment by adding an additional payment equal to 6% of the average sales price (ASP) of the drug. For Lucentis, the

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average sales price is about $1950, so Medicare pays the clinician an additional $115. Does it make sense to base the physician's payment on their choice of drugs, with a higher priced drug resulting in a higher payment? The current 6% payment of $115 is a tempting incentive, almost like a sales commission, and almost equals the entire $125 reimbursement for performing the injection. If Medicare considers this 6% payment as an interest payment to the clinician for the up-front purchase of Lucentis, then this $115 would translate into an annual uncompounded interest rate return of at least 70%, given the fact that most clinicians are paid by CMS and the secondary insurance within one month of injecting the Lucentis and that initial $2000 investment keeps getting a 6% return every month when the Lucentis is purchased for retreatment. Given this kind of return on an investment, I'm sure many of our patients would be willing to purchase the drug monthly if they were guaranteed at least a 70% annual return.

CMS also incentivized the use of Lucentis by dramatically decreasing the reimbursement for Avastin in hospital-based practices from $50 to $7. In the summer of 2009, CMS announced that they were planning to reduce the allowable payment for Avastin from $50 to $7. This decision was met with outrage within the ophthalmologic community. A concerted effort by our lawmakers, other government officials, and our professional societies successfully reversed this policy decision. However, this decision to reverse the payment decrease only applied to private practices. For hospital-based practices, such as our own practice in Miami, the reimbursement for Avastin remains at $7. As a result, this decision by CMS had unintended consequences even though the decrease in reimbursement was no longer in effect for private practices. Our evaluation of the 2009 CMS database suggested that the threat of decreasing the Avastin reimbursement to all practices resulted in the increased use of Lucentis nationally.

Another policy that could increase the use of Lucentis in a hospital-based practice is the Disproportionate Share Hospital (DSH) status of the hospital and the 340B drug discount program. Hospitals are granted this designation based on a disproportionate amount of indigent care provided at their facility. The drug discount program allows for the purchase of drugs at a discount of up to 20%. Consequently, if the hospital purchases Lucentis with a 20% discount for about $1600, then it would get reimbursed $2000 by CMS and the secondary insurance. As a result, the hospital would get $400 every time a clinician injects Lucentis. This payment could be another attractive incentive to use Lucentis, and raises the question why the cost savings aren't passed through to CMS?

The Genentech Rebate Program

On November 4, 2010, Andrew Pollack of the New York Times wrote about a rebate program for clinicians using Lucentis (http://www.nytimes.com/2010/11/04/business/04eye.html?_r=1&hp=&pagewanted=all). The article suggested that practices were given rebates based on their bulk use of Lucentis and the rate of increase in their use of Lucentis. While the

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details of the program remained vague, Pollack reported that the volume rebates combined with the increased usage rebates could net a practice as much as $58,000 per quarter. Following the release of this information, clinicians and our professional societies engaged in discussions about the ethics of such a rebate program. However, for the purposes of this discussion, if this rebate program does exist, then why should clinicians get this rebate rather than CMS? After all, isn't CMS paying for the drug?

Credit Card Purchases of Lucentis
Retina practices can purchase Lucentis directly from Genentech using a credit card. By using one of the American Express small business credit cards, clinicians can make an additional 1-2% in cash-back incentives, or they can accumulate points using cards from one of the other companies. Given the fact that credit card companies charge transaction fees and these transaction fees are not being added to the cost of the drug, it would appear that the drug is being sold at a discount. Shouldn't CMS be the one receiving this discount?

Total Lucentis Incentives
The current system has many attractive incentives that encourage the use of Lucentis. When added up, a busy clinical practice can make 6% on the ASP, another 1-2% by using a credit card, and an unknown amount if the rebate program exists. For a private practice, these incentives amount to a healthy return on a $2000 investment every month. In a DSH designated hospital-based practice, the incentives are even greater. With these financial rewards, why aren't all clinicians using Lucentis?

Important Lessons from the Lucentis/Avastin Controversy
I'm sure many of my colleagues who use Lucentis will be annoyed with me for openly discussing the Lucentis incentives, but I believe the clinical decision to use a drug should be based on comparative efficacy, safety, and cost. The decision should not be biased by financial incentives. Many of my colleagues feel these incentives are justified based on the dramatic and inappropriate reductions in reimbursements to clinicians from CMS over the past year. CMS decreased the injection reimbursement from $200 to $125, a 38% decrease, and decreased the reimbursement for routine imaging of both eyes from $104 to $48, a 54% decrease. While I too am outraged by these reimbursement cuts, I would argue that reimbursement to physicians should be a separate issue that needs to be addressed and should not be confused with these drug incentives. However, it will be interesting to look at Lucentis use in 2011 and see once again if these Medicare cuts to physicians resulted in unintended consequences. I would not be surprised if we see an upsurge in the use of Lucentis as clinicians feel they need additional revenue to cover the significant cuts in reimbursement for injections and imaging in patients undergoing treatment.

While the historical details of the Avastin/Lucentis controversy may be unique in the annals of medicine, the financial incentives driving the use of expensive

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drugs and procedures are not unique. The Avastin/Lucentis controversy demonstrates some very salient points that should not be ignored when formulating healthcare policy. First and foremost, it is not wise to pay a percentage of the drug costs on top of the cost of the drug. CMS should pay a flat fee for the clerical efforts associated with the purchase, storage, and invoicing of a drug. However, a flat fee alone will not be enough to influence practice patterns as long as other incentives are unchanged. Second, CMS should receive the 20% discount when drugs are purchased by hospitals with a DSH designation. Third, if the rebate program exists, CMS should be entitled to the rebates. Fourth, CMS should consider the purchase of a drug with a credit card as a form of a rebate to the clinician. Lastly, CMS should immediately increase the payment for Avastin from $7 back to $50 for hospital-based practices.

As a clinician, I don't want CMS telling me which drug to use, and I don't want patients worrying that the decision to inject their eye is being influenced by financial incentives. By addressing the financial incentives that currently promote the use of the most costly alternative, CMS could level the playing field and allow physicians and patients to focus on efficacy, safety, and cost when deciding between drugs. It is noteworthy to acknowledge, that despite all the financial incentives to use Lucentis, most ophthalmologists have chosen to use Avastin suggesting that most ophthalmologists are trying to control the cost of healthcare. While my suggestions alone won't halt the escalating cost of healthcare, they do represent necessary changes in the way physician-administered drugs should be reimbursed by Medicare Part B. Removal of these incentives would save CMS billions of dollars.

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Statement of Anthony P. Adamis, M.D.
Vice President and Global Head of Ophthalmology
Genentech, Inc.

United States Senate
Special Committee on Aging

July 21, 2011

Chairman Kohl, Ranking Member Corker and honorable members of the Committee, thank you for inviting me to testify here today. I respectfully request that my full written statement be submitted for the record.

My name is Tony Adamis and I am the Vice President and Global Head of Ophthalmology at Genentech. I am an ophthalmologist and vascular biologist by training. Prior to joining Genentech in 2009, I served in other positions in the biopharmaceutical industry, as well as eleven years on the full time faculty of the Harvard Medical School. At Harvard, I led a laboratory studying the mechanisms of eye diseases, including age-related macular degeneration and diabetic retinopathy.

My role here as part of the committee's look at drug pricing, particularly in medicines used by older Americans, is to discuss Lucentis (ranibizumab injection), the Genentech medicine approved for the treatment of a number of eye diseases worldwide. This discussion comes in light of recent studies that compare the use of FDA-approved Lucentis with the off-label use of Genentech's cancer drug Avastin (bevacizumab injection) in an eye disease called wet Age-Related Macular Degeneration (wet AMD). The results of these studies demonstrate that Lucentis improves vision with fewer ocular injections than Avastin[1], and that Avastin may be associated with an increased risk of serious systemic side effects[1-3]. For these and other reasons I shall review today, Genentech believes that physicians should be able to prescribe the medicine that they and their patients determine is most appropriate.

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As we all know, pricing is not a simple issue. To help the committee members better understand the many factors that contribute to the price of Lucentis, I will review the story behind the creation of Lucentis and how it differs from Avastin, the costs associated with the development of Lucentis, and the enormous impact the medication has had on patients suffering from eye disease. All of this I hope will create an understanding that pricing is part of a larger development model that results in breakthrough treatments for patients.

GENENTECH MISSION

Genentech was founded around the discovery of recombinant DNA technology and was the first company to develop medicines from living cells. In so doing, Genentech launched the biotechnology industry 35 years ago. In 2009, Genentech became a member of the global Roche group. Genentech is headquartered in South San Francisco, and as part of the Roche group currently employs over 30,000 people in the United States. By employing Americans in high-skill, high-wage jobs, Genentech/Roche impacts not only patients, but also the recovering US economy.

Genentech's commitment to future innovation is unparalleled within the industry, with more than 100 projects in clinical development. In 2009, Genentech/Roche spent $9.1 billion on research and development, an amount greater than any other company in the world, including Microsoft, Toyota and Apple.[4] Genentech's R&D investments are directed towards developing innovative medicines to treat serious diseases. One of the most impactful medicines we have ever developed is Lucentis.

CLINICAL IMPACT

Age Related Macular Degeneration (AMD)

Before Lucentis was developed, "wet" age-related macular degeneration (wet AMD), was the leading cause of blindness in Americans over the age of 50.[5] Wet AMD occurs when abnormal blood vessels grow beneath the light-sensing tissue of the eye, the retina. When these vessels hemorrhage and leak, vision is degraded. As

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recently as 2005, the average wet AMD patient progressively lost central vision over several years, until the ability to read, recognize faces and drive was diminished or lost. In addition to the personal suffering and loss of independence, the total annual cost to the U.S. gross domestic product due to lost wages was estimated to be $5.4 billion.[6]

That situation changed with the development of Lucentis. Genentech's Phase III trials demonstrated that the average patient treated with Lucentis *recovered* vision – a result never before seen in wet AMD. Vision improved after the first dose and reached peak levels approximately 4-6 months later. When these results were first announced in the summer of 2005, the President of American Society of Retinal Specialists publicly compared Lucentis to the discovery of penicillin. FDA approval was obtained in 2006, and the impact in the United States since then has been sizable. A recent publication estimates that the rate of legal blindness in wet AMD patients treated with Lucentis has been reduced by 72 percent.[7] As a result, wet AMD is likely no longer the leading cause of blindness in Americans over the age of 50.

Retinal Vein Occlusion (RVO)

Genentech also developed Lucentis for a second major cause of vision loss and blindness, retinal vein occlusion. The disease typically has a sudden onset and in the worst cases can lead to total blindness. Genentech's Phase III clinical trials demonstrated that Lucentis dramatically reduced the rate of vision loss and produced sizable visual gains. FDA approval was obtained in 2010.

Diabetic Macular Edema (DME)

Diabetic retinopathy is the leading cause of legal blindness in working age Americans, representing a large unmet medical need with significant economic consequences. The most common complication of diabetic retinopathy is diabetic macular edema, or DME. Since 1985, DME, has been treated with laser therapy, a procedure that clinical trials showed slowed the rate of vision loss.[8] Our recent

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Phase III data show that Lucentis produces significant vision gains in these patients. The beneficial effects were evident after the first treatment and have lasted two years. Three-year data will be available next spring. Approximately 75,000 new cases of DME are diagnosed each year in the United States.

Surveys have shown that people over the age of forty have a significant fear of going blind, and that blindness is feared more than premature death.[9] Lucentis has prevented blindness and restored vision in countless patients. I believe it is not an overstatement to say that it has changed the course of many lives.

THE COST OF DEVELOPING LUCENTIS

Drug development is lengthy, expensive and risky. Data show that drugs entering clinical development have a 92 percent failure rate.[10] Lucentis was one of the 8 percent that succeeded. The price of Lucentis funds not only its own development, but also the 92 percent failure rate and future successes.

Lucentis has gone through rigorous Phase III development programs that have clearly established a favorable risk/benefit profile. To date, 11 years and over $1.1 billion have been spent on completed clinical trials with Lucentis, involving more than 7100 patients around the world. This sum does not include ongoing development trials, investigator sponsored trials, the 11 years of research prior to human testing, our extensive safety monitoring, or the establishment of expensive manufacturing and analytical sites around the world that produce Lucentis and assure its quality. In short, the Lucentis development program has been one of the most expensive in Genentech's history.

LUCENTIS HISTORY

The Lucentis story begins in 1989, when a Genentech scientist discovered vascular endothelial growth factor, or VEGF. Dr. Napoleone Ferrara characterized the DNA

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coding for a novel, naturally occurring protein and demonstrated that it made blood vessels grow.[11] By the mid-1990's, his research showed that blocking VEGF may prove useful in the treatment of cancer, where blood vessel growth is required for tumor growth. Around the same time, and working in close collaboration with Dr. Ferrara, my colleagues and I at the Harvard Medical School conducted a series of experiments identifying VEGF as an important target for eye disease.[12]

While Dr. Ferrara was in the early stages of developing a VEGF antibody for cancer, the medicine that eventually became Avastin, a separate research program was started to design an anti-VEGF drug specifically for the eye. The latter medicine became Lucentis. There were attributes of the Avastin molecule that suggested it wouldn't be ideal for use in the eye, so Dr. Ferrara's team set out to create something better. In 2010, Dr. Ferrara was awarded the prestigious Lasker-DeBakey Prize in recognition for his work on Lucentis. Seventy-six Lasker laureates have gone on to win the Nobel Prize.

There are four scientific reasons why Lucentis was created. They involve drug potency, tissue penetration, ocular safety, and systemic safety. Today, because of the time limitations, I will focus on systemic safety.

LUCENTIS SAFETY

When drugs are administered to the eye, they often find their way into a patient's blood stream. When this happens, side effects are more likely. When administered to the eye, Avastin and Lucentis both enter the blood stream. Avastin was designed to treat cancer, therefore a long residence time in the blood stream was desired, so that it could have sustained activity against tumors in the body. The opposite, however, was desired for Lucentis. It was designed to exit the blood stream very quickly (hours instead of weeks) in order to reduce the risk of systemic side effects.

In the past decade, studies have shown that anti-VEGF drugs in the blood stream can result in rare, but serious, side effects. Genentech continuously monitors the safety of its drugs and takes action when potential risks are identified. When an interim

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safety analysis in 2007 revealed a potential risk of stroke with the use of Lucentis in wet AMD, Genentech promptly sent a letter to health care providers, notified the FDA, updated the package insert, and presented the data to the scientific community.

Today, there is a growing body of data that suggests off-label Avastin may pose greater risks than Lucentis when used to treat wet AMD. Two large Medicare claims studies, one from Duke University[2], and a second from Johns Hopkins[3], both identified a potentially greater risk of stroke and death when using Avastin vs. Lucentis in wet AMD. Of note, Genentech funded the latter study through an unrestricted grant. Separate studies have shown that when administered to the eye, Avastin can block VEGF in the blood stream for up to 28 days.[13]

The NIH-supported CAT Trial also showed a safety signal.[1] This was the first large randomized, controlled trial to examine the safety and efficacy of Avastin in wet AMD. These types of trials are considered the highest level of evidence in medicine. The CAT Trail reported a statistically significant 29 percent increased risk of serious systemic side effects with Avastin vs. Lucentis, with over 80 percent of the side effects requiring hospitalization. Genentech's internal analysis of the CAT Trial data revealed that part of the increased risk with Avastin was consistent with VEGF inhibition in the bloodstream.

These data are not yet conclusive. However to date, it is notable that the three largest studies comparing the safety of Avastin to Lucentis have shown statistically significant safety risks with the use of Avastin in wet AMD. The combined evidence suggests that the use of Avastin in the eye may be associated with an increased risk of serious systemic side effects.

PHYSICIAN CHOICE AND ACCESS TO TREATMENT

As the data emerge on the safety and efficacy of Avastin and Lucentis in wet AMD, Genentech believes that physicians should be free to prescribe the medicine that they determine is most appropriate for an individual patient. We agree with the

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American Academy of Ophthalmology that a treatment plan must be selected by the ophthalmologist and the patient, considering important risk/benefit information that empowers them to make evidenced-based decisions.

Genentech is committed to ensuring that no patient goes without treatment due to financial barriers. Since 1985, when its first product was approved, Genentech has donated approximately $2.3 billion in free medicine to uninsured patients. Since 2005, Genentech has donated more than $550 million to various independent, non-profit organizations for co-pay assistance. In 2010 alone, Genentech Access Solutions helped more than 107,000 patients with coverage and reimbursement issues.

Genentech offers comprehensive patient access programs for Lucentis. They include LUCENTIS Access Solutions® and the Genentech Access To Care Foundation. These programs provide assistance with reimbursement and coverage issues, and offer a variety of other services, including free medicine, to eligible patients.

CONCLUSION

I hope this testimony is helpful in your consideration of these important issues. Genentech is committed to working with the Congress, public health agencies, CMS and the FDA to ensure the safety and effectiveness of our products and fair payment systems that recognize innovation, and provide patients with access to needed medicines.

Today, innovation continues at Genentech, as we seek to improve Lucentis and to develop additional breakthrough medicines for blinding diseases. This work depends, in part, on the success of Lucentis.

U.S. companies are being called upon to innovate. Genentech was in fact founded with a mission of innovation, and has succeeded in treating some of most serious sight-threatening illnesses affecting Americans.

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Thank you for the opportunity to provide my views today, and I look forward to addressing your questions.

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A Prescription for Savings: Reducing Drug Costs to Medicare

Special Committee on Aging
United States Senate

Testimony of:

Sean Tunis MD, MSc.
Founder and CEO
Center for Medical Technology Policy

July 21, 2011

Dr. Tunis is the Founder and CEO of the Center for Medical Technology Policy (CMTP), an independent non-profit that works to improve the quality, relevance and efficiency of clinical research. Dr. Tunis was previously the chief medical officer at the Centers for Medicare & Medicaid services, and holds adjunct positions at Johns Hopkins, Stanford and the University of California at San Francisco schools of medicine. CMTP receives funding from private foundations, federal agencies, health plans and life sciences companies. This includes unrestricted funding from a number of pharmaceutical companies, including Genentech.

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Introduction

Mr. Chairman, Senator Corker, and other Senators of the committee, thank you for the invitation to appear before the committee today. The issues being discussed today are of central importance to the Medicare program and more generally to public health. The Medicare program can almost certainly spend less on drugs without any negative impact on health outcomes for Medicare beneficiaries. In my view, there are at least three important activities that can be pursued by the Medicare program to spend less and achieve the same or better health outcomes:

1) Medicare should have the authority to link drug prices more directly to health outcomes;
2) Medicare should implement additional policies to promote high priority clinical research; and
3) Medicare should develop a systematic policy approach to promoting health technology innovation.

Health care technologies, including drugs, devices, procedures, diagnostics and other services, receive a great amount of attention in discussions of health care costs because they are generally viewed as being responsible for up to 50% of the increase in health spending over time[i]. Along with the frequently cited estimate that up to 30% of health care services are unnecessary or of unknown effectiveness[ii], it is reasonable to conclude that substantial savings could be achieved simply by reducing payments for services that are not known to improve health outcomes.

In practice, it is often very difficult to know with a high level of certainty which drugs, devices or procedures are most effective, because the studies needed to answer those questions have never been done or are of poor quality. A recent report looking at the strength of scientific evidence supporting clinical recommendations on treatment of six common heart diseases by the American College of Cardiology and the American Heart Association showed that only about 10% of these recommendations were supported by high quality clinical studies[iii]. The number of situations in which compelling evidence is available to compare the benefits, risks and costs of alternative diagnostics and treatment approaches is limited, in part because high quality comparative studies are rarely done.

As Chief Medical Officer for the Medicare program, I was consistently struck by how much uncertainty existed about the relative benefits, harms and costs of widely used health technologies. Decisions about what to pay for and how much to pay were made substantially more difficult by these gaps in knowledge. Despite this uncertainty, there were plenty of examples where it seemed possible for Medicare to spend less for certain drugs and devices without any possibility of harm to Medicare beneficiaries. In some cases, one could be confident that it was possible to reduce drug costs to Medicare, without any sacrifice of health

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outcomes for Medicare patients. For reasons discussed below, the pathway to achieving those savings was far from easy, and would be considerably advanced with several specific policy changes.

I recognize that the approaches to reducing Medicare drug spending being discussed today will not by themselves produce dramatic spending reductions in Medicare. More fundamental payment reforms and delivery system innovations will be required to ensure the long term solvency of the Medicare program. However, it would still seem worthwhile to consider changes to coverage and payment policy that could potentially reduce federal spending by 100 million dollars or substantially more per year in treating specific medical conditions, particularly when those savings could be achieved with the same or better health outcomes.

Medicare should have authority to link drug prices to health outcomes

One relative straightforward approach to reducing Medicare spending on drugs without negatively affecting patient health would be to restore the Agency's authority to pay the same price for drugs that produce similar benefits and harms.

Medicare does not generally attempt to factor a service's relative effectiveness or its cost relative to alternatives in setting prices for covered items and services[iv]. However, Medicare's regional contractors have been adjusting prices highly selectively based on clinical effectiveness evidence for more than 15 years. This has been through their use of a least costly alternative (LCA) policy for certain types of items, including durable medical equipment and Part B drugs. The policy's rationale is that Medicare, beneficiaries, and taxpayers should not pay more for a service when a similar service can be used to treat the same condition and produce the same outcome at lower cost[v].

Using this reference pricing approach, Medicare contractors have not paid the added cost of a more expensive service if a clinically comparable one exists in particular categories of items and services. Examples include manual wheelchairs, power mobility devices, seat lift mechanisms, supplies for tracheostomy care, and anti-androgen drugs for patients with advanced prostate cancer. Beneficiaries are allowed to obtain the more costly item if they choose to pay the difference between the approved payment amount for the reference item and the amount for the one they choose.

There is no statutory provision giving specific authority or prohibiting the application of LCA. CMS has considered its "reasonable and necessary" statutory language[vi] to provide the needed authority to adopt this approach for equivalent drugs and equipment. However, a recent court decision constrains Medicare's current use of LCA determinations. In a case involving LCA for inhalation drugs for asthma, the Court found that the pricing formula for part B drugs specified in the Medicare Modernization Act did not allow Medicare the flexibility to apply the LCA approach to drug pricing, and the Agency and its contractors have not pursued the approach since that court decision[vii].

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In 2003, the Medicare program used a different legal authority to achieve comparable pricing for two similar drugs, Aranesp and Procrit, that were both approved to treat anemia caused by cancer chemotherapy. Because these drugs were very similar in their molecular structure, worked through the same biological mechanism, and had similar benefits and risks, Medicare decided to determine the price to be paid for Aranesp based on the price that was paid for Procrit, which resulted in a much lower level of payment for Aranesp than would have been determined through the standard methods of using average wholesale price. Medicare described these two drugs as being "functionally equivalent" to one another, and an existing payment authority, called "equitable adjustment" was used to establish the price.

This simple sounding idea turned out to be technically difficult, particularly the analysis required to determine the amount of each drug that produced the same improvement in the severity of anemia. There were few head to head studies of these two drugs, which significantly impaired the ability to calculate clinically equivalent dosing. The effect of this policy was estimated to have reduced Medicare spending by as much as $150 million dollars per year, without any negative impact on the health of cancer patients. The underlying logic of linking the price paid to the health benefits produced has intuitive appeal if one's intention is to buy as much health as possible with whatever resources are available, and ideally not to pay more than necessary to obtain a given amount of health benefit.

The basic premise is that we are generally inclined to pay similar prices for products and services that provide similar benefits. This is a fairly standard approach that consumers take to making purchasing decisions, and is the central premise behind the concept of value-based purchasing. We pay a higher price for things that provide more benefit, and less for items that offer less value. In the case of health care, we generally want to recognize better outcomes by paying more for them. That is why Medicare and private health plans are moving in the direction of tying the payments directed to clinicians, hospitals and other providers to the quality of the care that they provide. The same notion that motivates linking provider payments to outcomes can be applied to the prices paid for drugs, devices, procedures and other services. When health outcomes are better, prices are higher; when they are the same, the payment level is the same.

Clearly there are some reasonable concerns about the approach, including the possibility of reduced incentives for investment in new drug development and the challenges inherent in arriving at a definitive conclusion about equivalence of benefits and harms. There are also down sides to retaining a payment system that provides financial rewards for "newness" alone – in that the signal communicated to the product development community is that meeting the current standard of care will be financially rewarded. The signal that would ideally be sent is to guarantee significant rewards for substantial improvements over current therapy. Further discussion of the role of Medicare with respect to biomedical innovation appears below.

Medicare should implement additional policies to promote clinical research

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The CATT results underscore the importance to Medicare of having the capacity to rapidly identify, design, and implement clinical trials on questions of substantial importance to the Medicare program. Current research and payment policies create substantial challenges to consistently and efficiently conducting these studies. The successful implementation of the CATT required extraordinary and persistent efforts by a number of individuals and organizations, including the American Academy of Ophthalmology, the National Eye Institute, CMS and others to overcome substantial barriers and delays in executing the study. Senator Kohl was instrument in addressing challenges with handling co-pays for patients enrolled in the trial, and helped to craft language addressing this problem in the Medicare Improvements for Patients and Providers Act of 2008. This law provided new authorities to the Secretary of Health and Human Services to develop alternative payment mechanisms that would reduce reimbursement barriers to future comparative trials, and it would be helpful for CMS to outline the process through which this new authority will be implemented[viii]. A more reliable, less burdensome approach will be essential to ensure that we efficiently learn how best to put new discoveries into clinical use to achieve good clinical outcomes.

There is a critical need to produce critical information for patients, consumers, clinicians, payers, particularly in the context of payment reforms and new insurance benefit designs that will increase the reliance on clinicians and patients to weight the benefits, risks and costs of alternative health care choices. Faced with these difficult choices, these decision makers are going to need credible, unbiased information on which treatments work best, and at the lowest available cost. Judging from past experience, credible and relevant evidence will frequently be unavailable[ix].

Proton beam therapy for prostate cancer[x] and vertebroplasty and kyphoplasty for vertebral compression fracture[xi] are examples of widely used technologies for common clinical problems for which Medicare spends hundreds of millions of dollars, with little or no evidence that these treatments are better than inexpensive conservative therapies. Over $900 million was spent in the US in 2008 on surgical procedures for vertebral compression fractures despite the recent clinical guidelines from the Academy of Orthopedic Surgery which noted strong evidence against the use of vertebroplasty and weak evidence to inform recommendation about the use of kyphoplasty.

To ensure that Medicare dollars are directed to clinical services that are likely to improve the health of Medicare beneficiaries, the Medicare program will need to become considerably more active in ensuring that adequate studies are conducted on interventions that are widely used in their covered populations. Collaborative relationships with the Agency for Health Care Research and Quality, National Institutes of Health, and Patient-Centered Outcomes Research Institute will need to be strengthened, ideally with direction from a well defined list of clinical research priorities reflecting the perspectives of the Medicare program.

Medicare can also promote critical research by making more systematic use of coverage with evidence development (CED). Coverage with Evidence Development (CED) is a policy tool that

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links coverage of a technology with a requirement that patients receiving the service are enrolled in prospective clinical studies designed to inform future revisions to the coverage decision. The term was coined specifically for Medicare[xii], but is now part of a growing array of options for insurers to share in the costs of data collection in order to support their collective interest in reducing uncertainty when making coverage decisions[xiii]. Under CED, Medicare reimbursement is contingent on a beneficiary's participation in a clinical study as part of a systematic data gathering exercise.

CED remains a promising idea, for which the implementation has been done with great diligence, but there are still few unequivocally successful examples of CED leading to the generation of the type of relevant and reliable evidence originally envisioned. Although Medicare has applied CED in more than a dozen national coverage decisions in the last 15 years, data from the resultant studies have been used for policy in only two cases: for lung volume reduction surgery to treat late-stage emphysema in 2003 and the use of positron emission tomography (PET) for cancer in 2009. In both cases, Medicare made positive coverage policies that have been viewed as more permissive than was justified by the evidence generated from the studies[xiv,xv,xvi].In many other cases, appropriate studies were never designed, funded, or implemented, for a variety of reasons. In short, the promise of CED as a mechanism to support clinical research on urgent topics has not yet been realized.

Improving Medicare's use of CED will require explicit statutory authority for the Agency to apply this policy mechanism, as past efforts have had limited success largely due to the ambiguity of its legislative authority. Although CMS has issued guidance attempting to clarify the authorities for CED[xvii], each application has involved much internal legal debate. Without a clear legal mandate to pursue CED, CMS' efforts have, again, by necessity been *ad hoc* – with no formal process for selecting appropriate topics, little learning from one initiative to the next, limited resources and lack of dedicated staff skilled in navigating the political and operational issues raised by CED, including CMS' ability to require provider and supplier compliance with CED reporting requirements. This experience has dampened CMS' enthusiasm for pursuing this policy tool, as it requires considerable staff time and resources just to get approval, resulting in the failure to apply the policy for technologies which would most benefit from additional study. A series of steps through which Medicare can improve the implementation of CED is outlined in the most recent MedPAC annual report[xviii] and in the background paper on this topic prepared by the Center for Medical Technology Policy at the request of MedPAC[xix].

Medicare should develop a systematic policy framework to promote biomedical innovation

Sustaining innovation in the life sciences industry is clearly an important public health priority, and many analysts have made persuasive arguments regarding the health of the biomedical innovation ecosystem in the US. As has been frequently noted, the cost and time required to bring a new drug from initial discovery into clinical use are substantial, and appear to be increasing with time. Policy interventions by Medicare that aim to reduce the prices paid for some new drugs would have the potential to reduce the attractiveness of investments in early

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stage products, as the probability and magnitude of large profits can only be reduced in this context, particularly for new drugs and other technologies that do not impart significant new clinical benefits.

To my knowledge, the goal of promoting biomedical innovation has never been an explicit objective in the strategic plan for the Medicare program. The currently stated mission on the Medicare web site states that the program is: "to ensure effective, up-to-date health care coverage and to promote quality care for beneficiaries." And to the extent that promoting innovation is a high priority for the Department of Health and Human Services, little or no attention has been focused on the role that the Medicare program might play in achieving that objective.

Given the current size of the Medicare program and the dominant impact that is has on the market for drugs and devices in the US and globally, it is impossible for Medicare to avoid having an impact on innovation, whether or not it is an explicit programmatic goal. It would therefore be important for the Medicare program to seriously and systematically consider the impact of various Medicare policies on innovation, much as was done by the FDA in their 2004 report: Innovation or Stagnation: Challenge and Opportunity on the Critical Path to New Medical Products[xx]. This white paper describes the concerns about the impact of regulatory policy on innovation, and outlined a number of policy tools and strategies that could be pursued by the FDA, along with private sector partners, to promote innovation while continuing to fulfill their primary institutional mission of ensuring that medical products are safe and effective. The FDA report concludes that the primary barrier to innovation is that the applied sciences for medical product development have not kept pace with biomedical discovery, and that new tools were needed to accelerate product development:

> "A new product development toolkit -- containing powerful new scientific and technical methods such as animal or computer-based predictive models, biomarkers for safety and effectiveness, and new clinical evaluation techniques -- is urgently needed to improve predictability and efficiency along the critical path from laboratory concept to commercial product. We need superior product development science to address these challenges -- to ensure that basic discoveries turn into new and better medical treatments. We need to make the effort required to create better tools for developing medical technologies. And we need a knowledge base built not just on ideas from biomedical research, but on reliable insights into the pathway to patients."

The FDA has devoted substantial staff time and resources over the past 7 years to advancing the applied sciences for product development in the life sciences, working closely with private sector partners. The Critical Path Institute, a private, non-profit founded and led Dr. Ray Woosley, and supported in part by the FDA, has assembled a highly skilled staff of 55 scientists and clinicians to support pre-competitive collaboration in the life sciences industry to help develop the new product development science called for by the FDA. Many other professionals and organizations have also focused on carrying out the FDA's strategic vision.

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It would be extremely valuable for the Medicare program to take a similarly comprehensive and structured look at the relationship between Medicare policy and biomedical innovation, and then carefully consider the full range of Medicare policy mechanisms through which Medicare innovation could be sustained at optimal levels. This is particularly critical as the Medicare program continues to face strong spending pressures, and is likely to undergo significant reforms in payment policy and benefit design that may have important impact on innovation.

In the absence of a thorough assessment of the range of Medicare policy levers that may impact innovation, it is unclear whether the optimal response to the recognized concerns about biomedical innovation are best addressed by retaining current pricing mechanisms that are linked to average sales prices rather than to clinical evidence of comparative benefits and harms. The argument that Medicare should continue to pay more for new drugs as a mechanism to promote innovation should be considered with the benefit of a more comprehensive framework that considers a full range of alternative policy mechanisms through which Medicare might promote innovation.

Recognizing the important impact of health care reimbursement on innovation, the National Institutes of Health and Clinical Excellence (NICE) commissioned a report to examine how their analytic approach to new drugs might better reflect the value of innovation. The final report, Appraising the Value of Innovation and Other Benefits[xxi], provided 25 specific recommendations to NICE and the National Health Service about how their decision making processes could be made more sensitive to biomedical innovation. A similar assessment of Medicare reimbursement policies and biomedical innovation would be extremely valuable.

An initial effort to provide an assessment of specific Medicare policies that could promote innovation was taken on by the National Venture Capital Association (NVCA) in their 2007 report: Proposal for a Reimbursement Critical Path for CMS[xxii]. This report paper provided 8 specific recommendations for changes in CMS policies and procedures that were viewed as having potential to promote innovation. Some examples of these recommendations included:

- Develop clear process descriptions through which new technologies achieve coding, coverage and payment
- Establish specific timelines for all phases of the reimbursement process
- Clarify evidence requirements necessary to obtain new technology add-on payments and to quality for a separate Medicare billing code
- Undertake a thorough review of the process through which new CPT-coded are assigned to new technologies
- Explore approaches to parallel review and approval by the FDA and CMS

CMS should more explicitly recognize the critical impact of the Medicare program on innovation, and systematically evaluate policy strategies to sustain and promote innovation, analogous to the FDA's efforts to identify and address barriers to innovation from their perspective as a regulator. As with the FDA, a serious effort by Medicare to develop a policy strategy and the applied reimbursement science needed to promote innovation will require

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sustained attention and resources, and will likely require collaboration with a range of public and private experts and stakeholders.

Conclusion

The issues raised in today's hearing are of critical importance to the Medicare program and the health of the American public. The challenge of reducing drug costs to Medicare raises broader issues of how best to ensure that Medicare spends money on technologies and services that provide the most possible benefit to beneficiaries. Responding intelligently to this challenge becomes more urgent as health care cost trends inevitably promote greater downward pressure on spending. In the specific context of drugs and other medical technologies, we believe that there would be value in further exploring three policy strategies: 1) Medicare should be given authority to link drug prices more directly to health outcomes; 2) Medicare should implement additional policies to promote high priority clinical research; and 3) Medicare should develop a systematic policy approach to promoting health technology innovation.

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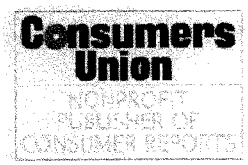

Testimony of

LISA SWIRSKY
Senior Policy Analyst

On behalf of

CONSUMERS UNION

Before the
Senate Special Aging Committee
on
Reducing Medicare Drug Costs

July 21, 2011

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Thank you Chairman Kohl and Ranking Member Corker for the opportunity to testify in front of your Committee today.

Consumers Union, the non-profit publisher of Consumer Reports magazine, has a long history of advocating for improving health care and lowering the cost of drugs for consumers. Our print and online publications also have a long history of helping consumers navigate and understand the complex world of health care. CR's information is regularly made available to up to 10 million subscribers via our numerous media products, including *Consumer Reports* magazine, ConsumerReports.org website, and *ShopSmart* magazine, and to nearly 20 million viewers of our syndicated television news segments from CRTV.

Our popular *Best Buy Drugs* reports reach 100,000 readers per month and provide rigorous, evidence-based, comparative information on a range of commonly used drugs through our websites. We do not accept advertising, relying only on paid subscribers, donors and some grants. Consumer Reports also provides access to *Best Buy Drugs* free of charge.

Best Buy Drugs reports rely on the credible systematic reviews of available clinical evidence conducted by expert researchers at the Drug Effectiveness Review Project, based at Oregon Health & Science University (OHSU). The DERP analyzes hundreds of studies on a given class of drugs to treat a condition. We use price information from a leading health care data and analytics company. Consumer Reports writers and editors then translate the clinical evidence for our readers and present it in the consumer friendly language and format that is a hallmark of our organization. As part of that, we select specific medications as "Best Buys." To earn that designation a drug must generally be at least as effective and safe as other medications in its class and less expensive. However, if the data show that a brand name drug is notably safer or works better than a lower-cost medicine, the drug is deemed a CR *Best Buy Drug*.

The goal of our *Best Buy Drugs* program is to help doctors and patients base treatment decisions on independent and unbiased scientific evidence. Our free reports cover 25 classes of drugs for more than 35 conditions, including allergies, diabetes, high blood pressure, high cholesterol, and muscle pain. Over the years, CR has frequently recommended lower cost generics or lower cost brand alternatives where the research indicates it is as effective. CR has done so for cholesterol-lowering drugs, certain pain medications, diabetes and heartburn drugs.

Consumers Union believes generics are safe and effective and their use can save consumers money. Below are some specific examples from *Best Buy Drugs* which illustrate savings that can be achieved by using generic alternatives.

Cholesterol lowering drugs, one of the most common medications, is a prime area where Consumer Reports has found lower cost drugs to be as effective and as safe as more expensive brand name drugs. While CR recommends the brand drug Lipitor for those who have had a heart attack and require very substantial lowering of their LDL

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cholesterol levels, many consumers take this type of medication for preventive reasons: they haven't yet had a heart attack. For these millions of Americans, generic Lovastatin is as effective, just as safe, and considerably less expensive. For example, a daily dose of Lipitor would cost an individual without insurance $112 per month compared to $4 per month for Lovastatin. And these savings can be achieved without compromising the safety and efficacy of treatment.

Diabetes medication is another area where our organization has found low cost alternatives to be effective and safe. An older diabetes drug, generic Metformin, is our Best Buy recommendation: it works better than all other diabetes medications, and at $4 a month, is a bargain compared to the pricey brand drug Actos, which can set consumers back by up to $280 a month. Metformin is also the safest—newer medications Actos and Avandia both carry a higher risk of increased heart failure. Avandia was severely restricted by the FDA last year when it was linked to a higher risk of heart attack and stroke, proving that you don't always get what you pay for when it comes to drugs.

Proton Pump inhibitors for heartburn are yet another example of a class of drugs where CR has consistently recommended an older, less expensive generic drug that is as safe and effective as brand name drugs. For instance, the highly advertised and expensive drug Nexium also known as "the purple pill"-- can cost up to 10 times as much for a month's supply over the Consumer Reports Best Buy Drug, omeprazole—which you can get over-the-counter for just a few dollars.

Finally, when it comes to pain medications, CR has reported that simple, low cost, over-the-counter medications such as naproxen (the generic version of Aleve) and ibuprofen (the generic version of Advil)—already in most families' medicine cabinets—work as effectively as more expensive and heavily advertised Celebrex for short term pain relief due to arthritis. A daily dose of these medications run a few dollars a month compared to $139 a month for Celebrex.

These real life examples show how effective and safe generic drugs are and how they can save consumers precious dollars. Our organization strongly believes that Congress should pursue policies that improve access to generic drugs.

One way to do this is to enact Senator Kohl's and Senator Grassleys bipartisan legislation that would end collusion between brand and generic companies to delay generic competition. As you know, one example of this tactic is for brand companies to pay generics to delay entry into the market in exchange for the brand manufacturer delaying the entry of their own "authorized generic." According to the FTC, such "agreements with compensation from the brand to the generic on average prohibit generic entry for nearly 17 months longer than agreements without payments…" The CBO and the FTC estimate these agreements wind up needlessly costing Americans billions of dollars.

In addition to promoting generics, we would urge Congress to reform Medicare and Medicaid payment processes to incorporate evidence about the safety and efficacy of drugs. For example, Congress could consider legislation to allow Medicare to use

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reference pricing where strong evidence of clinical comparability exists using evidence from rigorous, systematic comparative reviews of drug classes. Congress might also create incentives to make sure that Part D and state Medicaid formularies carry the generic as a preferred drug when there is strong evidence of comparability. Of course, doctors must always have the ability to specify the brand alternative when it is best for the patient.

Congress should also act to improve the way pharmaceutical companies convey safety and efficacy information to consumers so that they can better understand and use available clinical evidence to make choices about their treatment. A recent Consumer Reports investigative report that compared prescriptions filled for the well-known blood thinner drug, Warfarin (Coumadin is the brand-name) uncovered dangerous findings. Critical drug warning information was missing from several bottles; important patient information materials included with the prescription, which had instructions on proper use, were nearly unreadable—filled with medical jargon and printed in impossibly small type. And incredibly, four of five pharmacies failed to send Consumer Reports secret shoppers home with mandated FDA Medication Guides for this potentially dangerous medication. To remedy problems such as the ones uncovered by CR, Congress should consider creating consumer friendly standards for Patient Medication Information such as package inserts and package labeling. Further, CU has long advocated for the development of a Drug Fact Box so that patients can more easily asses the benefits and risks of drugs.

In conclusion, consumers should have reliable, trustworthy information about drugs readily available to them. This will help them make safe, cost effective decisions about their own health and that of their families. Consumers Union appreciates the efforts of this Committee and the leadership of Senator Kohl on drug costs, and we look forward to continuing to work with you on this important matter.

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AMERICAN ENTERPRISE INSTITUTE

Statement before the Special Committee on Aging
United States Senate

A Prescription for Savings: Reducing Drug Costs to Medicare

July 21, 2011

Scott Gottlieb, M.D.
Resident Fellow
American Enterprise Institute

Dr. Gottlieb is a Resident Fellow at the American Enterprise Institute. He is a practicing physician and a Clinical Assistant Professor at the New York University School of Medicine. Dr. Gottlieb consults with and invests in drug companies. The views expressed in this testimony are those of the author alone and do not necessarily represent those of the American Enterprise Institute.

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Introduction

Mr. Chairman Kohl, Mr. Ranking Member Corker -- thank you for the opportunity to testify before this Senate Committee.

Over the past decade, the drug space stands apart from other segments of the healthcare industry in terms of how much the underlying business model has changed. The life science sector has undergone a fundamental transformation to focus on delivering more value and more basic innovation to consumers.

These economic tenets – value and innovation – are now firmly embedded in considerations of which new drug products and new companies get taken forward. In part, this transformed business model is a consequence of well-designed government policies that have made the marketplace for drugs more competitive.

Industry pipelines also have more new compounds in late stage development than at any time before. More of these experimental drugs are derived in part, or wholly from profoundly new areas of drug science such as regenerative medicine, genomics and proteomics, and humanized immunotherapies. More of these new drugs are aimed at fundamentally new targets and more address unmet needs in medicine. Many are targeted at niche conditions or smaller subsets of common diseases.

The price of creating this innovation continues to rise, in part because development costs have exploded. But the drug prices paid by consumers and health plans have – on the whole – moderated to a level closer to overall inflation. Generic drug utilization has also reached all time highs. Nearly 75 percent of all prescriptions filled in the U.S. are for generic drugs, a number that will continue to rise.

These low cost generics are available only because investments were made in the original development of an innovative drug in the first place – a drug that subsequently lost its patent protection. This is a unique feature of the drug market that makes it one of the most competitive parts of the healthcare industry. The ability of innovative products to be brought forward, and subsequently be subject to generic competition once patents have expired, is also a feature on which public policy has become dependent. Low priced generic medicines are dependent upon the initial investment that made the original innovation possible.

Biologics, which represent some of the most breakthrough innovation taking place today, are also going to benefit from this cycle of innovation and generic competition. New legislation to create a pathway for follow-on versions of biologics will provide for more price competition in this technology segment. Taken together, there are many positive developments when it comes to competition in the drug industry, the delivery of value, and the ability of patients to access new technologies.

The economic framework for delivering value when it comes to new drugs is more firmly established than at any point in recent years as a result of competitive forces

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that are sweeping the drug industry. The firm footing for this framework is also a consequence of the regulatory process. Medical products are one of the few consumer goods that must undergo pre-market approval before consumers can use them. They are one of just several products that must submit data demonstrating safe use and effectiveness to the government, and must undergo an extensive review process, prior to being cleared for sale. As such, compared to other consumer goods and even healthcare services, there is an extensive amount of information available about how medical products can be used most effectively.

But despite recent progress, challenges remain. There are still consumers who are priced out of healthcare. There are still cases where reasonable people could argue that a drug's price does not approximate the clinical value that is being delivered to an individual patient. The cost of developing drugs is rising at an unsustainable pace. New company formation in the biotech sector has dwindled. The overall cost of healthcare continues to rise. Too many diseases remain poorly treated.

So we must craft policies that provide proper incentives for new technology while making sure that we are getting more value for programs like Medicare.

Today I would like to outline some policies that I believe are working toward achieving these goals, in particular, the recent success of Part D. I would like to comment on some proposals to lower drug prices that are currently before this committee – proposals that I believe will undermine our recent progress, and otherwise create more ill effects relative to any benefits that they deliver.

Finally, I want to outline some proposals that I hope this committee considers. One of these is the idea of folding the medical drug benefit into the prescription drug benefit under the Medicare program -- merging the Part B and Part D drug schemes.

There are many challenges to this kind of scheme that would need to be carefully considered. It would not be a straightforward policy change. But properly designed, this reform would mirror changes taking place in the private insurance market. It could also foster greater price and brand competition, and reduce or eliminate peculiar constructs that have the potential to get in the way of making sure patients are getting the most clinical value out of the drugs they are prescribed.

Part D, Price Competition, and Consumer Benefits

Any discussion of policies that have worked to bring more price competition to the prescription drug market, and lower overall spending, has to begin with Medicare's "Part D" prescription drug program. Competition between more than 1,000 drug plans has resulted in costs that are substantially less than what was first envisioned, wider use of generic medicines than at any time, and discounts on branded drugs that have facilitated access and helped save money for seniors and taxpayers.

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The Congressional Budget Office originally estimated that Part D would cost taxpayers $551 billion between 2004 and 2013.[i] The actual cost was much less. Federal outlays for the drug program, netting out premiums paid by enrollees and Medicaid payments made by states, totaled $214 billion through 2010. By 2013, we will have spent a total of $375 billion on the drug program. This sum is 32 percent less than what the Congressional Budget Office originally estimated.

A new report from the IMS Institute for Healthcare Informatics -- titled "Medicare Part D at Age Five: What Has Happened to Seniors' Prescription Drug Prices?" – tells part of the story. It shows the experience of drug prices in the major therapeutic drug classes that are used by seniors. The report finds that inside the Part D program, the average daily costs of therapy for eight of the top ten therapeutic drug classes fell on a sustained basis between 2006 and 2010.

Specifically, between January 2006 and December 2010, the average daily cost of therapy in the ten classes identified in the report declined by more than a third, from $1.50 to $1.00. With some widely used drugs coming off patent over the next several years, IMS projects that the average daily cost of therapy in these classes will continue to decline, reaching $0.65 by the end of 2015. This represents a cumulative 57 percent decline in the cost of these drugs since January 2006.[ii]

Critics argue that these savings are an artifact of one-time events, and not a result of increased market competition for how drugs are priced – competition that has been sparked by the Part D program. More specifically, they argue that the savings are really the result of lower than expected enrollment in the Part D program and a larger than expected number of costly, blockbuster medicines losing patent protection and becoming cheap generics. According to my AEI colleague Joe Antos, an economist who held leadership positions at both the Congressional Budget Office and the Centers for Medicare and Medicaid Services, neither factor accounts for the disparity between the CBO estimate and actual experience in the Part D program.

Antos observes that to gauge the size of the enrollment effect, one could multiply the additional number of people assumed by CBO (7.1 million, a number that has been relatively constant over time) by CBO's estimate of the federal cost per enrollee ($1,600) for each year. That totals about $92 billion through 2013, or only about 17 percent of the cost difference.

If anything, this is probably a conservative estimate. That's because many of the people not enrolled in Part D probably choose to forgo the drug insurance because they are not on any expensive, chronic use medications.[iii] In other words, their annual cost would be much less than the $1,600 figure. Thus they don't view the drug coverage as representing a good deal for them.

In short, overstated enrollment could be said to contribute to some of CBO's original prediction of higher costs. But clearly other factors are more important.

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Moreover, comparing Medicare's spending trends with that of the entire health sector shows that the slowdown in drug spending inside the Medicare program was greater than the slowdown in drug spending outside the Medicare program. Antos notes that this suggests that an expiry of patents, which benefited all consumers equally, cannot explain the smaller-than-expected cost of the Part D program.

Competition between the Part D plans has to be a significant factor in the lower-than-expected cost of the program. CBO and the Medicare actuary don't take full measure of these competitive impacts because they don't have actuarial experience measuring these effects. Competitive behaviors such as: discounting by drug companies eager to secure preferred placement on drug plan formularies; drug plans that squeezed margins to gain market share or ran their plans as a loss leader for entry into other business segments; or drug plans that aggressively pushed generic substitutes as a way to lower premiums, cut costs, and boost profits.[iv]

As Mr. Antos notes in a recent article, had Part D been structured like traditional fee-for-service Medicare, then none of this would have happened. If we paid for each individual prescription the way we pay for each individual health service, there would be no incentive for drug plans to encourage the use of generics over brands. Indeed, economic and political incentives would drive out low-cost drugs in favor of products with higher profit margins in a cost-plus payment environment.[v]

Part D: Delivering Benefits System Wide

The economic benefits of Part D's competitive structure aren't confined to the Medicare program. They spill over into the rest of the commercial market, partly as a result of the additional discounts that prescription drug plans are able to secure for their members – discounts that apply across a drug plan's entire book of business. This delivers additional savings to consumers of private health insurance.

According to one recent estimate, on average, Part D has lowered retail prices for non-Part D commercially insured patients by 5.8 percent. The estimated external cost-savings to the entire commercial market amounts to $2.6 billion per year.[vi]

The authors found that the larger the number of Part D beneficiaries an insurer enrolled, the steeper the discounts a plan was able to secure. Enrolling an additional 100,000 Part D beneficiaries enables an insurer to negotiate 2.1 percent lower prices for non-Part D seniors (5.4 percent lower for generics and 0.3 percent for brands) and 1.8 percent lower prices (3.7 percent for generics and 0.35 percent for brands) for non-seniors enrolled in commercial plans that also participate in Part D.

Overall, Part D lowered retail prices for non-Part D elderly by 8.5 percent (19 percent for generics and 0.9 percent for brands). Part D likely led to bigger price discounts on drugs commonly used by the elderly. This is why elderly enrollees not in Part D realized larger price declines than the commercially insured non-elderly.[vii] The experience in Part D might have also had implications for the commercial drug

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market's use of generics. The shift toward generics among the elderly in Part D may have caused physicians to change prescribing behaviors for all their patients.[viii]

In addition to the direct economic savings realized by the price competition that has ensued as a result f Part D, the increased drug utilization among seniors that resulted from Part D has also helped to improve health outcomes among the elderly, saving money for other parts of the Medicare program.

One recent study[ix] found that for conditions sensitive to medication adherence, hospitalization rates for Medicare beneficiaries declined more in states that saw the biggest coverage gains due to the implementation of Part D, relative to states with smaller Part D-induced changes in drug coverage. Comparing changes in hospitalization rates from 2005 to 2006 and 2007 (relative to changes among the near-elderly, who did not experience a change in benefits), Part D reduced the overall rate of hospitalization by 20.5 per 10,000 (4.1 percent), or by 42,000 annual admissions across eight medication sensitive conditions that were studied.

The authors speculated that if the results were extended to the entire over-65 Medicare population this would represent an aggregate reduction of 77,000 annual hospitalizations across those eight medication-sensitive conditions. Part D did not affect incentives for hospitalization, so any changes related to Part-D induced changes in drug coverage are likely due to changes in underlying health status.

Another study found that while enrollment in Part D was associated with increased direct spending on prescription drugs; other healthcare savings compensated for much of these outlays. Specifically, groups of enrollees that had no or minimal drug coverage before the implementation of Part D had reductions in other medical spending that approximately offset their increased spending on drugs.

That study found that after two years of enrollment in Medicare Part D, enrollees that had no drug coverage had increased their monthly drug spending by $41, but that outlay was roughly offset by a decrease of $33 in their monthly medical spending. The authors speculate that this was due to the fact that increased use of medication led to improved control of chronic illnesses. The same fact was observed for beneficiaries who had some drug coverage, but not enough to provide a full range of prescription benefits. For example, those seniors whom previously had a $150 quarterly cap on drug spending increased their spending by $27 once they enrolled in Part D. This was offset by a decrease of $46 in their medical spending.[x]

Proposals Being Considered by this Committee

These are some of the positive developments from policies that were put in place with this Committee's help less than eight years ago. As budget pressures mount, however, we are forced to consider additional ways we can make sure Medicare is continuing to get value for what it spends on drugs. I want to take a moment to provide some thoughts on a few of the proposals that I expect we will discuss today.

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Some of the same data that I cite above, demonstrating the health benefits that accrue to patients from their appropriate use of prescription medicines, also underscores why issues of access are so important. While the price of drugs, and access to them, are distinct policy issues, in a world of mounting deficits and finite resources they cannot be divorced from one another. So as we discuss ways to ensure continued access to affordable drug coverage, we also need to take measure of the cost of developing medicines in the first place, how drugs are priced as a result, and how we make sure we continue to bring new drug innovations to market.

First, I want to comment on some of the recent proposals to import pharmaceutical price controls into the Part D benefit. These include recent suggestions to extend the Medicaid Best Price to the dually eligible Medicare and Medicaid beneficiaries who are enrolled in Part D, as well as those receiving a Low Income Subsidy (LIS). These mandatory rebates are a form of drug price controls that serve to distort market prices. The more that price controls creep into the Part D program, the more we erode the commercial forces that have made that market highly competitive.

One example: mandatory rebates create a strong incentive for companies to launch drugs at higher prices in anticipation of the payments that they will have to provide to the states and the federal government. These rebates also discourage additional discounting. Moreover, as more beneficiaries come under these kinds of tacit price control regimes, it will erode the ability of health plans to use competitive negotiations to move their market share and improve profit margins. This will, in turn, reduce their incentive to try and drive hard bargains with drug companies.

Once the Part D plans become fully commoditized insurance products – a reality hastened by the introduction of price controls into this market – plans will have little incentive to compete by negotiating discounts with drug makers. The Part D plans will, in essence, become price takers rather than price negotiators.

As Antos notes in a recent working paper that he co-authored with Guy King, if proposals for mandatory rebates in the Part D program are adopted, patients will ultimately bear the cost. Higher-income seniors who are not eligible for Medicaid or other Part D subsidies would feel the immediate impact. As drug plans respond to the new financial and regulatory incentives, enrollees who qualify for Medicaid or other low-income subsidies also could find fewer attractive options. The plans that continue to offer coverage to LIS enrollees without an additional premium are likely to have tighter formularies and less access to newer or more expensive drugs.[xi]

I know members of this Committee have also considered proposals to expand the authorities of Medicare to enable the Medicare program's staff to take clinical criteria into consideration in how they make payment and coverage decisions. I am talking here about constructs such as Least Costly Alternative authority.

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In particular, some have postulated that these authorities could be combined with the capacity for CMS to demand clinical information, or develop its own clinical studies and comparative effectiveness research, to turn the coverage process into one resembling the FDA approval process – where CMS uses its interpretation of clinical data to make more granular decisions about what it choose to pay for.

There is nothing inherently wrong with a payer carefully judging the clinical data supporting the use of a particular medical product or service to determine what it will reimburse. But there are some things very particular to Medicare that makes the program no ordinary payer, and its decisions no ordinary matter.

One problem is that CMS has no tradition of making these kinds of decisions. As a consequence, it has little capacity to engage in these kinds of judgments. But the issue is larger than just hiring a bunch of clinical experts to make these decisions.

If Medicare were to engage in making clinical judgments about new technology at the time of first introduction, to deny reimbursement in cases where the clinical data is still not firmed up, it would undermine the way innovation unfolds in life sciences. In many instances, much of the innovation takes place post-market as new technologies are introduced and demonstrate additional benefits from real world use. Demanding early lifecycle demonstrations of value, however measured, skews heavily against this sort of post market innovation. Yet it's this post-market experience that is a big part of how clinical medicine has historically advanced.

We should consider how past treatments that we now recognize as profound advances would have fared under an LCA policy. We should also consider how such a construct would affect future decisions. An LCA process would invariably skew investment decisions, creating a binary hurdle that new technology would need to clear, much like the FDA approval process. This would invariably discourage investment decisions; not only because of the hurdle itself but the fact that the novelty of this requirement would mean that it would hard for investors to handicap this new hurdle. Investment shuns uncertainty, and this creates plenty of ambiguity.

The interplay of LCA authority with IPAB would present particular problems. IPAB could effectively confer CMS with LCA authority, in a wholesale fashion or around specific products or services. Yet IPAB was purposely constructed to be opaque and unaccountable. These elements were designed into the agency's constitution. The ability of this new agency to target individual services, products, and procedures with little accountability would create a high degree of uncertainty in the marketplace and represent a fundamental unfairness to Medicare stakeholders.

In addition, Medicare is not an ordinary payer. Its decisions are widely followed in the private market. It often sets a ceiling on what private payers are willing to cover.

As such, Medicare has a disproportionate impact on what patients in the U.S. will have access to. In turn, the program's practices are widely tracked in the Venture

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Capital community and on Wall Street, where investors carefully weigh how they believe Medicare will treat a new medical product when making decisions to invest in a new technology in the first place. In short, the decisions Medicare makes have wide impact. They reverberate through the entire healthcare sector.

As a consequence, it is important that Medicare is more transparent, more judicious, more rigorous, and yes, in many cases more generous, in how it covers new technology. Medicare's coverage process serves as a de-facto gatekeeper to what patients will have access to in many cases. If we want new technology to advance and its application be optimized through ordinary clinical practice, Medicare has to allow new technologies to reach the market in the first place.

Yet the proposals that have been put forward would turn the coverage process into a more opaque, less rigorous construct. For example, some proposals would allow these decisions to be meted out by an independent and largely unaccountable advisory board. Others would see CMS relying on comparative effectiveness data that is not rigorous enough to form the basis of firm clinical judgments and would be summarily rejected by FDA if it were used as the basis of a regulatory filing.

Innovation in the Balance

We need to continue to pursue policies that aim to deliver more value for Medicare beneficiaries. But we must also consider the impact of these policies on investment in new drug development and continued life science innovation. The economic model that has fueled the investment necessary to endow the U.S. with the lion's share of the world's life science activity remains fragile, and is more in doubt than perhaps ever before. As we consider steps to implement policies that affect how drugs are priced, we should understand that biotech investment model better.

The cost of drug development has gone up significantly. Even while total R&D spending by a consolidating industry still continues to rise, and venture flows have remained level, the number of experimental drug programs that are being funded by this spending has shrunk. The result is that while the existing pharmaceutical industry consolidates, and acquires successful biotech firms for their products, the amount of new biotech company formation has fallen off sharply. There is a long lag time between investment in an early-stage venture and the payoff in terms of a late stage drug candidate. But make no mistake, these business trends will eventually show up in the form of fewer drugs being put into late stage development.

This phenomenon is worth considering, because the policies we are discussing here today could have implications on the investment model. New costs could further erode the ability to fund new drug development ventures, and start new companies.

Many of the costs are regulatory. According to some recent analyses, the actual direct costs of the clinical development of a new drug can approach $500 million.[xii] [xiii] The cost of some recent development programs have topped $1 billion in direct

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costs for new cardiovascular drugs, a therapeutic class that often requires especially large trials as a result of clinical and regulatory factors. One example is the new super aspirins approved for the treatment of stroke and acute coronary syndrome.

The new antiplatelet drug Brilinta was compared to Plavix in a trial that enrolled 18,624 patients hospitalized with acute coronary syndrome during a median treatment of nine months.[xiv] A similar drug, Effient, was studied in several significant trials. The largest of these studies enrolled 13,608 patients and compared its effects to the blood-thinning effects of Plavix in patients with a threatened heart attack or an actual heart attack and about to undergo coronary angioplasty.[xv]

Trials of this size are mammoth economic investments, not only to the drug makers but also to society.[xvi] Each patient enrolled in a pivotal study adds more than $30,000 to the cost of the trial. A single pivotal trial can easily top several hundred million in direct costs. These costs get baked into the retail price of the drug.

Combine the increased cost of development with some other factors that are affecting the biotech investment model.

First, more of these regulatory costs are front-loaded. In other words, proportionally speaking, the spending on early stage development has risen more sharply than the cost of late stage development.

Second, the "value creation" stage in life sciences has been pushed out much further as a result of regulatory uncertainty that has increased the risk of late-stage failure. Before a novel phase II drug asset had substantial value, and biotech companies could raise capital around reaching such a milestone. Now a company must often take a promising new drug candidate into phase III clinical trials to be able to monetize the development program by selling the asset or raising capital from the public markets to fund its continued development. This means that raising the capital to fund development programs has become more difficult, and expensive.

Finally, layer on top all of this the declining patent life of new compounds. The average duration of intellectual property protection around new molecular entities is continuing to decline (it's now less than 13 years) as a result of recent policies aimed at encouraging generic competition and generic drug entry.

This is, in short, a perfect recipe for undermining a field of scientific innovation: Front load the regulatory costs, push out the value creation stage, and diminish the window in which entrepreneurs have to earn back a return on their investment.

Arbitrary policies to impose controls on certain prices, or penalize certain drug classes and individual compounds, impose more uncertainty and discourage investment capital from flowing into this industry. It drives up the cost of development by increasing the cost of capital needed to fund new innovations.

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Now don't get me wrong. The news isn't all-bad when it comes to our vibrant life science industry. It's just that the investment model that has endowed the U.S. with a vibrant life science sector is fragile. The creation of this industry is one of our singular achievements. It has been the result of private and public capital and it is something that we should be mindful to preserve as we debate policies about how we try and influence the pricing of the products that result from these endeavors.

It's true that venture capital continues to flow into the life sciences. People are still making big bets on new science. Data on venture capital flows into various industries are tracked by an annual survey jointly published by Price Waterhouse Coopers and the National Venture Capital Association. The good news is that venture capital money continues to flow into life science ventures. These investments accounted for nearly 30 percent of the $21 billion invested in venture-backed companies in 2010. Moreover, the returns on these investments have been strong over time, a reason why investors continue to make bets on new drugs.[xvii]

But there is a cautionary tale. It is something that can be observed anecdotally from those who follow the biotech industry. Nearly 60 percent of biotech initial public offerings from the past four years are trading below their issue price versus about 30 percent of tech IPOs. Moreover, post-IPO, the performance has been dramatically better for the IT companies than it has been in Life Sciences. IT companies that get public tend to be more mature business with revenues and often-significant earnings; in biotech, most companies remain cash burning for a very long time, precisely because the development programs have become so long, and so costly.

This difference in IPO results is important. Healthcare companies usually require substantially more money to get to an answer than other kinds of venture capital investments. To fund a new drug program these days can cost about $200 million of capital to get to a "value creation stage" where investors can monetize a successful endeavor. As a result, some smaller venture capitalists are exiting this space. Even bigger firms that can write bigger checks are nonetheless often looking at new models of investing that don't require new company formation. Rather than start a new biotech company to develop a new concept or a family of promising drug companies, some investors are looking to fund drug development in a one-off fashion, creating virtual companies around individual compounds.

The result is much less new company formation, less big bets in the life science sector. As the great lions of the biotech industry – Genentech, Genzyme, Millennium – are themselves absorbed by larger pharmaceutical companies, there are fewer new biotech companies being formed to create the next crop of great American life science companies. The great hubs of biotech start-ups such as San Diego are starting to look more like biotech ghost towns. This will have an impact on employment in this sector, and it will eventually have an impact on pipelines.

Proposal: Merging Part B and Part D

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Policies that encourage more price competition, and more clinical competition between similar drugs attacking similar diseases or targets, can help drive more value for beneficiaries while encouraging the sort of scientific competition that could lead to more, not fewer, opportunities for new innovations. Which gets me to the idea of merging Medicare's drug and medical benefits – folding its Part B program for drugs administered in the doctors' office into the Part D program.

There is good clinical and economic rationale for providing drugs under a single, unified program. Many private plans have already merged the drug and medical benefits. Folding Part B into Part D could provide substantial savings to Medicare. The savings would be a result of greater therapeutic substitution between oral and injectable drugs as well as more price competition between similar drugs.

Doctors would no longer have to choose an injectable therapy over an oral alternative because of concerns about whether a patient can afford a co-payment. With more oral agents competing with injectable agents to target the same biological mechanisms and same diseases, there's no reason why artificial economic constructs should be weighing on decisions about which drug to employ. These decisions should be based solely on the clinical question of what's best for patients.

It's true that there are many drug targets that are best attacked in injectable drugs. But for those that can be approached by both oral and injectable agents, there are plenty of reasons why injectable drugs often have advantages owing to their ease of use for the patient, and the lower cost to Medicare of administering a drug orally. Paying for drugs under one policy scheme would level the playing field and make scientific considerations the only factor in how to attack a particular disease.

Now moving Part B into Part D is enormously complex, and full of potential for damaging, unintended consequences. It would need to be considered carefully. It is also not worth doing if it only invites more temptation to import price controls into the resultant drug program. This is a competitive reform, but it will only work to lower prices and drive more high value use of drugs if drugs are allowed to compete with one another in reasonable and competitive market-based framework.

Moreover, not all of this savings would actually accrue to Medicare. Some of it would need to be used to help offset the rise in premiums and out-of-pocket costs incurred by beneficiaries in the form of higher Part D co-pays and program costs. Medicare would also have to create new physician payment codes to compensate doctors directly, at a fair and sustainable rate, for the cost of infusing drugs in their offices.

Conclusion

Right now, the custom of paying doctors by allowing them to keep a spread on the drugs they infuse creates incentives for all of the wrong kinds of behavior – higher launch prices, more use of the costliest biological drugs even when there are

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cheaper alternatives, and a penalty for companies that discount their medicines by ultimately increasing the cost to the physician who chooses to infuse a cheaper drug.

The drugs that are in late stage development right now, and that have been launched in recent years, are perhaps more promising than at any point in recent memory. The Wall Street Journal recently took note of this trend in a recent front-page news article.[xviii] "Because developing drugs takes many years, changes in how companies approach the process take a long time to show effects," the paper reported. "Today's new-drug output appears to mark the beginnings of a payoff from a research reorientation the industry began undertaking several years ago."

This innovation is by no means a sure bet. The model that has made our life science successes possible remains in doubt. The decisions that we make about how we regulate the development of these products, and pay for their costs, have direct and prompt effects on whether or not these endeavors get undertaken in the first place.

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[i] These costs, projected at the outset of the program, were expected to be offset by an estimated $142 billion in lower federal payments to state Medicaid programs

[ii] Murray L. Aitken and Ernst R. Berndt. Medicare Part D at Age Five: What Has Happened to Seniors' Prescription Drug Prices? IMS Institute for Healthcare Informatics July 2011. Available at www.phrma.org/sites/default/files/202/ihiimedicarepartdreport-final.pd

[iii] Moeller J, Mathiowetz N. Prescribed medicines: a summary of use and expenditures by Medicare beneficiaries. National Medical Expenditure Survey Research Findings, National Center for Health Services Research and Health Care Technology Assessment. Rockville, MD: U.S. Public Health Service, September 1989.

[iv] The average profit a pharmacy earned on generic drugs is $2.25 higher than for brands

[v] Joseph Antos. What Does Medicare Part D Say About the Ryan Plan? RealClearMarkets.com. June 15, 2011. Available at http://www.aei.org/article/103728

[vi] Darius Lakdawalla and Wesley Yin. National Bureau of Economic Research Working Paper. Insurers' Negotiating Leverage and the External Effects of Medicare Part D. April 2011. Availabe at http://www.nber.org/papers/w16251

[vii] Darius Lakdawalla and Wesley Yin. National Bureau of Economic Research Working Paper. Insurers' Negotiating Leverage and the External Effects of Medicare Part D. April 2011. Available at http://www.nber.org/papers/w16251

[viii] James C. Capretta, "Klein's F on Part D," National Review Online, June 21, 2011; http://www.nationalreview.com/articles/270099/klein-s-f-part-d-james-c-capretta.

[ix] Christopher C. Afendulis, Yulei He, Alan M. Zaslavsky, Michael E. Chernew. The Impact of Medicare Part D on Hospitalization Rates. Health Services Research 2011, Volume 46, Issue 4, pages 1022–1038. Available at http://onlinelibrary.wiley.com/doi/10.1111/j.1475-6773.2011.01244.x/abstract

[x] Yuting Zhang, Julie M. Donohue, Judith R. Lave, Gerald O'Donnell, Joseph P. Newhouse. The Effect of Medicare Part D on Drug and Medical Spending. New England Journal of Medicine 2009; 361:52-61 http://www.nejm.org/doi/full/10.1056/NEJMsa0807998

[xi] Joseph Antos and Guy King. Tampering with Part D Will Not Solve Our Debt Crisis. The American Enterprise Institute, AEI Health Studies Working Paper 2011-03, June 29, 2011. Available at http://www.aei.org/paper/100234

[xii] J DiMasi, RW Hansen, and HG Grabowski. The price of innovation: new estimates of drug development costs. Journal of Health Economics 2003;22:151–185.

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[xiii] Boston Consulting Group. A Revolution in R&D: How Genomics and Genetics are Transforming the Biopharmaceutical Industry. 2001, The Boston Consulting Group, Boston, Massachusetts.
[xiv] L. Wallentin, R. C. Becker, A. Budaj, C. P. Cannon, H. Emanuelsson, C. Held, J. Horrow, et al., "Ticagrelor versus Clopidogrel in Patients with Acute Coronary Syndromes," New England Journal of Medicine 361 (2009): 1045-57.
[xv] S. D. Wiviott, E. Braunwald, C. H. McCabe, G. Montalescot, W. Ruzyllo, S. Gottlieb, F. J. Neumann, et al., "A Clinical Trial of Prasugrel vs. Clopidogrel in Patients with Acute Coronary Syndromes," New England Journal of Medicine 357 (2007): 2001-2015; S. D. Wiviott, E. M. Antman, C. M. Gibson, G. Montalescot, J. Riesmeyer, G. Weerakkody, K. J. Winters, et al., "Evaluation of Prasugrel Compared with Clopidogrel in Patients with Acute Coronary Syndromes: Design and Rationale for the Trial to Assess Improvement in Therapeutic Outcomes by Optimizing Platelet Inhibition with Prasugrel Thrombolysis in Myocardial Infarction 38 (TRITON-TIMI 38)," American Heart Journal 152 (2006): 627-35; and S. D. Wiviott, E. M. Antman, K. J. Winters, G. Weerakkody, S. A. Murphy, B. D. Behounek, R. J. Carney, et al., "Randomized Comparison of Prasugrel (CS-747, LY640315), a Novel Thienopyridine P2Y12 Antagonist, with Clopidogrel in Percutaneous Coronary Intervention: Results of the Joint Utilization of Medications to Block Platelets Optimally (JUMBO)-TIMI 26 Trial," Circulation 111 (2005): 3366-73.
[xvi] Scott Gottlieb. The FDA Should Not Mandate Comparative-Effectiveness Trials. The American Enterprise Institute Health Policy Outlook, June 2011. Available at http://www.aei.org/outlook/101056
[xvii] Bruce L Booth and Bijan Salehizadeh. In defense of life sciences venture investing Nature Biotechnology 2011;29: 579–583
[xviii] Jonathan D. Rockoff and Ron Winslow. Drug Makers Refill Parched Pipelines. The Wall Street Journal, July 11, 2011. P A1.

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PRESCRIPTION DRUG PRICES:
FINDINGS FROM INTERNATIONAL COMPARISONS AND A DOMESTIC STORY

A REPORT

OF THE

MAJORITY STAFF OF THE SPECIAL COMMITTEE ON AGING
UNITED STATES SENATE

July 21, 2011

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As Chairman of the Special Committee on Aging, Senator Kohl authorized information requests on prescription drug pricing as part of two separate inquiries. The first inquiry sought information from pharmaceutical companies regarding the disparity in American pricing when compared to other developed countries. The second inquiry, with Representatives Waxman, Pallone and DeGette, senior members of the House Energy and Commerce Committee, sought information on how two pharmaceutical companies set prices for two newly Food and Drug Administration (FDA) approved drugs. This report summarizes the Committee's findings from the information collected from these inquiries.

PART I: Inquiry on Drug Prices: Comparing U.S. to Foreign Market Drug Prices

On September 30, 2009 the Special Committee on Aging held a hearing on reducing health care costs. As a follow up, Senator Kohl asked Senator Bill Nelson of Florida to chair a Special Committee on Aging hearing on March 17, 2010, "Seniors Feeling the Squeeze: Rising Drug Prices and the Part D Program,"[1] on the rising cost of drugs. At that hearing, Senator Kohl noted that Americans pay, on average, twice as much for prescription drugs as people in other developed countries. Further, the large discrepancies in the cost of identical drugs could not be explained by differences in production or manufacturing. Following the hearing, the Committee sent letters to AstraZeneca, GlaxoSmithKline, Eli Lilly, Novartis, Pfizer, and Sanofi-Aventis to ask about these discrepancies.

Letter to AstraZeneca: *www.aging.senate.gov/letters/drugcostsaz.pdf*
Letter to GlaxoSmithKline: *www.aging.senate.gov/letters/drugcostsgsk.pdf*
Letter to Lilly: *www.aging.senate.gov/letters/drugcostslilly.pdf*
Letter to Novartis: *www.aging.senate.gov/letters/drugcostsnovartis.pdf*
Letter to Pfizer: *www.aging.senate.gov/letters/drugcostspfizer.pdf*
Letter to Sanofi-Aventis: *www.aging.senate.gov/letters/drugcoststsanofi.pdf*

These letters asked the following questions:

1. Why are there discrepancies between foreign and domestic prices for certain medications?
2. Have these prices changed in the past 10 years, and if so, why?
3. Ireland recently announced price reductions of 30 percent or more. Why are prices in Ireland, and other countries such as the Philippines being reduced, while prices continue to increase for U.S. consumers?
4. Manufacturers and industry representatives contended that U.S. pharmaceutical research is unparalleled, and accounts for a disproportionate share of needed innovation in pharmaceuticals and biologics. Despite the enormous investment made by the U.S. government and American consumers in pharmaceuticals, why aren't U.S. drug prices competitive when compared to the prices in other industrialized countries?
5. What percentage of your research budget is comprised of U.S. Federal funds?
6. What are your profit margins and distribution costs in each of the countries listed? What percentage of overall profit comes from the U.S.?

[1] Videocast and materials available at http://aging.senate.gov/hearing_detail.cfm?id=323166&.

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7. Please list the number of employees in every country in which you employ them. What percentage of your operation is in the U.S.?
8. How much did your company spend on marketing each of these drug(s) in each country listed? How much was spent directly on marketing to physicians?
9. Did your company manufacture free samples of any of the aforementioned drug(s)? If so, please provide data on the cost associated with manufacturing and distributing these samples.

Findings: Detailed Questions and Responses

1. Why are there discrepancies between foreign and domestic prices for certain medications?

The companies were fairly consistent in their responses.

First, companies suggested that drug prices are higher in the U.S. than in other developed countries because other countries use price controls while the U.S. has a market-based system.

The respondents noted that foreign governments usually set prices in their countries. As Novartis said, "in other countries, where the government is the sole or majority provider of health care benefits, governments mandate the price of pharmaceutical products." Most companies also claimed that they prefer the U.S.'s market-based approach because it is more attractive to investors, leads to a stronger pharmaceutical industry, and allows prices to be set by market forces.

According to the manufacturers, foreign governments rely on price controls and formulary restrictions to ensure medications are affordable. As AstraZeneca stated in their reply, "pharmaceutical pricing reflects the policy choices and domestic market forces of this country."

Second, companies claimed that it was difficult to compare prices between countries because of the variety of requirements pharmaceutical companies face. GlaxoSmithKline said, "There are different regulations and market conditions in different countries which impact the pharmaceutical industry. These differences result in wide variations in labeling, manufacturing and packaging standards. Multinational pricing comparisons are extremely difficult as a result of these differences."

Several companies noted that the wholesale acquisition cost (WAC), which is the price cited in the Committee's letters to the companies, does not represent the net price paid by the purchaser. Novartis went so far as to say that "it is inaccurate and misleading to attempt to compare these foreign prices to WAC prices in the U.S., since WAC prices may not reflect the discounts offered or rebates paid." Aside from disputing the numbers used in the Committee's letter, Sanofi-Aventis noted that the Federal Supply Schedule, which sets prices for the Department of Veterans Affairs, Department of Defense, Indian Health Service and the Coast Guard, are derived from market prices.

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2. Have these prices changed in the past 10 years, and if so, why?

Drug companies declined to clearly explain how and why drug prices changed over time.

The Committee found that U.S. prices are higher than foreign prices, and that they tend to rise more rapidly. The companies' responses provided a varying level of detail about the price disparities. Some offered fairly detailed tables, with the qualification that the pricing data they supplied was proprietary.

No company offered a business case for the setting or changing prices, other than mentioning the prices charged by rival products in the same drug class. The price rationales provided were vague, and referred to the differences in regulation between nations (see below). Some companies also noted the impact expiring patents would have on future prices as justification for current prices.

Several companies noted that the rate of increase in U.S. drug prices has declined in recent years.

3. Ireland recently announced price reductions of 30 percent or more. Why are prices in Ireland, and other countries such as the Philippines being reduced, while prices continue to increase for U.S. consumers?

Drug companies again pointed to the free-market approach in the U.S. versus the price control system in Europe as a problem in directly comparing prices.

The Committee's letters to the companies noted Ireland had recently announced reductions of 30 percent or more for many drugs, and that prices in the Philippines were much lower than U.S. prices.

The responses resembled the replies to the first question about why prices are lower in foreign markets. For example, Sanofi-Aventis wrote "[i]n contrast to the free market dynamics that dictate pricing in the United States, the governments in various countries in Europe play a significant role in setting prices." Eli Lilly wrote "[w]ith regard to the Philippines, it offers a particularly poor comparator to the United States. Its health care system is subject to widespread expressions of concern regarding the adequacy of access to the most basic of services for a clear majority of the population."

In addition, the policy changes that resulted in lower prices for some drugs did not apply to all the drugs about which the Committee asked. One company provided another cost methodology, "Net Wholesale Price," which indicated that prices for their drugs had increased in Ireland and the Philippines, though they still remained less than half of U.S. prices.

The companies also suggested other reasons why U.S. prices were appropriate. One company noted that with insurance subsidies, patients' out-of-pocket costs for their drugs were only 10 to 30 percent lower in foreign markets. Several others argued that the pricing policies of other nations lead to lower availability of innovative medications. AstraZeneca wrote, "...in Canada a

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single government entity, the Patented Medicine Prices Review Board (PMPRB), regulates the prices charged by manufacturers for patented medications. Although recent changes to the PMPRB guidelines have created a more competitive pricing environment, a 2008-2009 study showed that only 55% of innovative medicines received approval from Canada's Health Technology Assessment appraisal system, compared to an international average of 73%."

4. ***Manufacturers and industry representatives contended that U.S. pharmaceutical research is unparalleled, and accounts for a disproportionate share of needed innovation in pharmaceuticals and biologics. Despite the enormous investment made by the U.S. government and American consumers in pharmaceuticals, why aren't U.S. drug prices competitive when compared to the prices in other industrialized countries?***

See Question Above.

5. ***What percentage of your research budget is comprised of U.S. Federal funds?***

Drug companies assert that they receive 0 to 1 percent of funding from the U.S. government for research. However, the companies did not take into account governmental programs that support innovation, including research programs, the patent system and tax preferences.

The companies all answered this question directly and reported little to no direct federal support—between 0 to 1 percent of their total research and development budget. Four of the six companies provided their overall research budgets, which varied from $4.4 billion to $10 billion.

Despite this purported lack of direct federal investment, pharmaceutical companies may benefit from government investment in training and research. Seventy-nine percent of drug and medicine patents cite the results of public science.[2] The Congressional Research Service[3] compiled several studies that found research funded by the National Institutes of Health (NIH) directly contributed to the development of four to nine percent of the top selling drugs studied. Specifically, CRS found two relevant studies:

> In response to congressional direction, the National Institutes of Health looked at 47 FDA-approved drugs that had sales of $500 million or more a year to determine the role of NIH-sponsored technologies in their development. As described in the resulting July 2001 report, *A Plan to Ensure Taxpayers' Interests are Protected*, "NIH sought to determine whether the agency, directly, or through a grantee or contractor, held any patent rights to the drugs."[4] NIH funded technologies were found to have been used in the development of four of these pharmaceuticals:

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[2] Francis Narin, Kimberly S. Hamilton, and Dominic Olivastro, "The Increasing Linkage Between U.S. Technology and Public Science," *Research Policy*, 1997, 328. See also: G. Steven McMillan, Francis Narin, and David Deeds, "An Analysis of the Critical Role of Public Science in Innovation: The Case of Biotechnology," *Research Policy*, 2000, 1.

[3] Congressional Research Service. Federal R&D, Drug Discovery, and Pricing: Insights from the NIH-University-Industry Relationship. Wendy H. Schacht, Specialist in Science and Technology Policy January 6, 2010 (RL32324).

[4] *A Plan to Ensure Taxpayers' Interests are Protected*, National Institutes of Health, 2001. http://www.nih.gov/news/070101wyden.htm.

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- Epogen® and Procrit® are based on different uses of a patented process technology developed at Columbia University with support from NIH grants. Columbia licensed their technology to Amgen for Epogen® and to Johnson & Johnson for Procrit®.
- Neupogen® is manufactured by Amgen using patented technologies for a process and a composition licensed from Memorial Sloan-Kettering Cancer Center (MSKCC). These technologies were developed with NIH grant support.
- Taxol® is manufactured by Bristol Myers Squibb (BMS) using a patented process technology developed by Florida State University (FSU) with NIH grant funds. In addition, the NIH has rights to an underlying technology arising from a NIH CRADA collaboration with BMS. The NIH has received from BMS tens of millions of dollars in royalties from FY1997 to FY2000 under the license to the NIH technology.

A 2003 study by GAO found that government financial support of extramural research and development had resulted in inventions that "were used to make only 6 brand name drugs associated with the top 100 pharmaceuticals that VA [the Veteran's Administration] procured for use by veterans and 4 brand name drugs associated with the top 100 pharmaceuticals that DOD dispensed in 2001."[5] What these, and other reports document is that "while NIH's federally funded research has contributed in a substantial, dramatic, yet general, way to advances in medicine and biology, the direct contributions to a final therapeutic product as a consequence of the Bayh-Dole process[6] is limited and difficult to determine."[7] In addition to multiple sources of innovation, tracking the federal contribution is made more difficult by the fact that the government does not retain ownership of inventions made by contractors.

These studies only capture research that leads to top-selling drugs. They do not capture other federally-supported efforts that do not result in a marketable drug. These research projects are also important to manufacturers, as they indicate which research directions will not be productive, thereby increasing the effectiveness and return of research investments made directly by manufacturers.

6. What are your profit margins and distribution costs in each of the countries listed? What percentage of overall profit comes from the U.S.?

Responses regarding profit and distribution costs in foreign markets were evasive and uninformative.

Expense information was included for one family of drugs, in which most of the expenses associated were related to marketing.

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[5] General Accounting Office, *Technology Transfer, Agencies' Rights to Federally Sponsored Biomedical Inventions*, July 2003, GAO-03-536, 2.

[6] The Bayh-Dole Act, as implemented in 37 C.F.R. 401, gives intellectual property rights for inventions arising from federal research funds to the awardee institution, such as a university or small business.

[7] *A Plan to Ensure Taxpayers' Interests are Protected*, National Institutes of Health, 2001. http://www.nih.gov/news/070101wyden.htm.

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Companies responded to the question as narrowly as possible, sometimes stating the drug was not marketed in all countries and was therefore impossible to compare. Answers ranged from no response to claims that over 80 percent of worldwide sales for specific drugs were made in the U.S.

One company provided a detailed response on one family of drugs, including information about one generic version, several new versions on patent, and a rival patented drug in same class owned by a different firm. Domestic sales in this class are in the multi-billion dollar range. For the patented versions owned by the responding firm, sales revenue exceeded brand expenses by 32.8 percent. Brand expenses included Manufacturing and Distribution (22.5 percent of revenue) and "Amortization of Intangibles," or the write-off for the declining value of the patent (9.7 percent of revenue). There were no research costs reported for the family of drugs in that calendar year. All other costs were associated with marketing (e.g. sales force, promotion, etc.).

U.S. Expenses and Sales in CY 2009 for a drug family, as supplied by one respondent[8]

Direct Brand Expenses	
Standard Costs (Manufacturing and Distribution)	(22 5%)
Cost of Goods Sold Variance	0.1%
Sales Force Expenses	(12.8%)
Promotion Expenses	(17.6%)
Marketing Management	(0.3%)
Medical Management	(0.2%)
Local Clinical Studies	0
Amortization of Intangibles	(9.5%)
G&A Allocation	(2.9%)
Marketing Administration	(0.7%)
Medical Administration	(0.9%)
Sales Less Direct Brand Expenses	**32.8%**

7. ***Please list the number of employees in every country in which you employ them. What percentage of your operation is in the U.S.?***

Even though the U.S. represents the world's most profitable pharmaceutical market, almost all of these companies employed the majority of their workforce in foreign countries.

[8] Respondent emphasized that this table is not a profit and loss statement for its product and does not reflect the "profit" of product sales in 2009. Among other things, it does not include several indirect brand costs such as the costs of defending patents, defending product liability actions, transporting product doses from manufacturing sites, and researching and developing new products. The direct brand expenses also do not include taxes. For these reasons, the data above should **not** be considered a true reflection of product profitability.

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Of the companies we questioned, we found the portion of U.S. based employees averaged 30 percent of all employees (ranging from 14 percent to 50 percent of employees). However, our review of these companies' annual reports and other financial disclosures reveal U.S. sales ranged from 32 percent to 45 percent of all sales.

Percentage of Employees in the United States

Company	% US Employees
Company A	25%[9]
Company B	23%
Company C	20%
Company D	50%
Company E	14%
Company F	45%

8. How much did your company spend on marketing each of these drug(s) in each country listed? How much was spent directly on marketing to physicians?

The companies provided different levels of granularity in their responses, either citing marketing costs for specific drugs or total overall marketing budgets.

Some companies reported marketing costs for individual drugs that varied from $80 million to $285 million per year worldwide. For those reporting overall marketing costs, they reported marketing and sales expenses between $8 and $11 billion per year worldwide.

9. Did your company manufacture free samples of any of the aforementioned drug(s)? If so, please provide data on the cost associated with manufacturing and distributing these samples.

Companies offered free samples and discounts, which were associated with marketing costs.

The companies noted that they offered free samples and direct-to-consumer discounts, some of which were based on the financial needs of the patient. Both of these efforts are counted as marketing costs, although they may not increase sales. Not all the companies listed the amount of free samples or discounts per drug for all of their products, but some volunteered that they provided hundreds of millions of dollars in free samples of all products, and hundreds of millions of dollars in discounts of their products to low-income or uninsured patients. [10]

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[9] The percentage is for all North American employees.

[10] Limited price reductions can generate sales that might not have otherwise occurred at a higher price point, and encourage patient and physician use that extends beyond the discount period. For example, the Institute of Medicine found "[i]n academic medical centers, drug samples may be associated with the prescription of new brand name drugs in situations in which the sample drugs are different from the physician's preferred drug or are not recommended by evidence-based practice guidelines or in situations in which less expensive drugs or generic equivalents are available for the same indication."[10] Page 135. IOM (Institute of Medicine). 2009. *Conflict of Interest in Medical Research, Education, and Practice.* Washington, DC: The National Academies Press. Page 135. IOM (Institute of Medicine). 2009. *Conflict of Interest in Medical Research, Education, and Practice.* Washington, DC: The National Academies Press.

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Conclusions

Drug prices are obscure

The Committee found pharmaceutical companies to be purposefully vague about pricing strategies within the U.S. market. Drug companies contend that the prices listed in our letters of inquiry may not be representative of actual prices paid by a purchaser, because they did not necessarily account for rebates, bulk discounts or other incentives. Companies said that the "true" prices vary by payer, are difficult to calculate, and are almost always privileged information. Further, publicly available drug price information is often inaccurate, and does not reflect the actual price paid by the purchaser or the consumer.

None of the data received demonstrated a direct relationship between prices charged and the costs of manufacturing, distribution, or research and development. Across the board, the largest expense category was always marketing.

U.S. Drug Prices Compared Internationally

Drug prices are higher in the U.S. because prices are market based, not governmentally negotiated. Drug companies set U.S. prices based on what the market will ultimately bear. Publicly held pharmaceutical companies also emphasized their fiduciary obligation to their shareholders to maximize their profit.

PART II. Investigation of Specific Drug Prices

In May 2011, Senator Kohl, along with Representatives Waxman, Pallone and DeGette, sent letters of inquiry to two pharmaceutical companies, URL Pharma and Avanir, regarding their two drugs, Colcrys and Nuedexta, respectively. Colcrys (colchicine) is prescribed as a treatment for gout and Familial Mediterranean Fever (FMF), and Avanir is prescribed as a treatment for pseudobulbar affect (PBA), which causes uncontrollable emotional outbursts and can accompany Multiple Sclerosis (MS) and Amyotrophic Lateral Sclerosis (ALS).

Each drug had previously been reported to be available as an "unapproved drug," which is a drug that has not been approved by FDA but is available commercially, though the Committee later learned that only one drug was widely available previously. In June 2006, FDA announced a new drug safety initiative to bring the roughly 2,000 unapproved drugs into the approval process.

Once the drugs were approved, their prices were many times higher than the reported cost of the unapproved drug. Both companies suffered negative attention from the media and Congress when these cost increases became public. This section summarizes the findings of the investigations into URL Pharma's and Avanir's pricing decisions. In the letters to the companies, they were asked about the costs of the clinical trials that led to approval; the determination of the list price, manufacturing and marketing costs; and, the patient access programs established to increase availability of the drug.

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URL Pharma

The letter to URL Pharma asked about the pricing decision for Colcrys after media outlets reported that colchicine, the active ingredient, had been available for about $0.09 per tablet before FDA approval.[11] URL Pharma charges $4.85 per tablet of Colcrys, which is taken one to three times per day. The company spent $48 million to get Colcrys approved. After approval, URL Pharma petitioned FDA to take a strong stance against other single-ingredient colchicine applications, and clear the market of unapproved colchicine.

There were several items of note in URL Pharma's response. First, URL Pharma recognized that the federal government would be a major purchaser in the sale of Colcrys. According to the company, Colcrys revenues derived from Medicare (25%), Medicaid (4%) or other federal or state health care programs (20%) account for 49% of total revenues from product launch to April 2011.

Second, in response to the question about the determination of the sales price, URL Pharma submitted several presentations from outside consulting companies and internal notes. According to the documents, URL Pharma settled on the price of $4.85 after considering both substantially lower and higher prices recommended by outside contracting firms, and acknowledged that the higher price could result in pushback from the purchasing community.

Colcrys' labeling states that the company did not include enough seniors in their clinical trials to determine if they respond to Colcrys differently than younger patients. Despite this, the FDA found Colcrys to be safe and effective in the elderly at the time of approval, and allowed it to be dosed as such. In an internal memo on the pricing decision, URL Pharma tells its staff that one of Colcyrs' "core product messages" which "may be delivered through a variety of vehicles and may not be stated overtly" is that "Colcrys has been demonstrated to be safe and effective in elderly patients" (page 4300 – 4301).

While not necessary, considering that FDA found Colcrys to be safe and effective for seniors and allowed it to be dosed as such, URL Pharma elected to conduct an additional post-market study[12] to evaluate and compare the pharmacokinetics in young and old patients. This study found no difference in the pharmacokinetics of Colcrys, though the study included only three individuals over the age of 65 out of 18 elderly patients.

While investigating URL Pharma for pricing practices with regard to Colcrys, the Committee learned about URL Pharma's improvements to the unapproved drug. There were hundreds of adverse events and 169 deaths associated with the unapproved colchicine available on the market before FDA approved Colcrys.

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[11] See, for example, Arthur Allen, "A Giant Pain in the Wallet: How drug companies are making crucial, common drugs up to 100 times more expensive," *Slate*, March 29, 2011 (http://www.slate.com/id/2289616/).

[12] Mutual Pharmaceuticals Company, Inc. Relative Bioavailability of Colcrys™ 0.6 mg Tablets in Healthy Young and Elderly Volunteers Under Fasted Conditions. In: ClinicalTrials.gov [Internet]. Bethesda (MD): National Library of Medicine (US). 2000- [cited July 20, 2011]. Available from: http://clinicaltrials.gov/ct2/show/NCT01001052 NLM Identifier: NCT01001052.

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In 2006, FDA started the Unapproved Drug Initiative to clear the market of drugs that had never been approved, such as colchicine. URL Pharma answered FDA's call to conduct clinical trials on colchicine for its submission. In doing these clinical trials, URL Pharma discovered that a lower dosing regimen preserved the benefits of colchicine while minimizing harmful side effects. Still, some patients write that the lower dose schedule of the drug is less effective than previous unapproved versions; in a briefing for staff the company stated that these cases are inexplicable outliers. In addition, URL Pharma found several dangerous drug interactions, now included in Colcrys' label and the labels of several contraindicated drugs. FDA has commended these efforts.

The company has an extensive patient assistance program, and has helped over 50,000 patients thus far. The program provides benefits to patients earning up to 6 times the federal poverty level, and many patients receive the drug for free or for $5 per month's supply.

Avanir

The letter of inquiry to Avanir Pharmaceuticals concerned the pricing decision on their drug Nuedexta after media outlets reported that a combination of the drug's two active ingredients was available from compounding pharmacists for roughly $0.66 per day before FDA approved the drug, compared to $16 per day for Nuedexta. We found that the company invested heavily in the development of Nuedexta. It provided documents detailing expenditures of $239 million in overhead and research, and nearly all of its resources from 2009 to 2010 on developing the best proportion of ingredients and for testing.

Before Nuedexta, there was no approved treatment for PBA, which causes uncontrollable emotional outbursts in patients with MS and ALS; doctors previously treated PBA with powerful psychotropics used off-label. While some compounding pharmacist groups claim that the drug was indeed compounded[13], it appears that these instances were infrequent and could not have been the same safe and effective dosage that Avanir discovered. Avanir has suggested to staff that compounders started making the medication, which is comprised of two common and inexpensive ingredients, for former participants of Avanir's clinical trials after the trials ended. The company stated to staff in briefings that FDA treated the drug as a new entity.

The price of Nuedexta is slightly lower than the average (Wholesale Acquisition Cost of $16 per day, or $8 per tablet) of what consulting companies advised in 2010 (range between $6 and $43 per day, or between $3 and $21.50 per tablet). Notably, the company has told staff in briefings that the price was determined using a market-based approach, not based on expenditures, and that the company had lost money every year for 20 years prior to the approval.

Avanir Pharmaceuticals also has a co-pay assistance program. The drug entered the market in February 2011, and as of the end of June 2011, 343 patients have used this program.

Conclusion

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[13] Angela Townsend, "Protests help bring down price for preterm birth drug Makena," *The Plain Dealer*, April 11, 2011 (http://www.cleveland.com/healthfit/index.ssf/2011/04/the_high_price_of_preventing_p.html).

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From the recent investigation into URL Pharma and Avanir, we have learned that drug companies charge prices based mostly on what the market will bear without strong pushback from purchasers, and less on what the drug costs to develop, market or manufacture.

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Senator Robert P. Casey, Jr. Statement for 7/21/2011 Aging Committee Hearing on Reducing Drug Costs to Medicare

Mr. Chairman, I want to thank you for scheduling this important hearing. It is critical that we fully investigate all issues surrounding prescription drug prices and the effect that these prices have on Medicare beneficiaries and the Medicare program.

This is a critical moment for the Medicare program. The demand for Medicare services will increase exponentially in the coming years with the first of the baby boom generation reaching 65 this year. The United States Census Bureau estimates that the number of adults aged 65 and older will almost double from 37 million to over 70 million between 2005 and 2030. This is an 8 percent increase from 12 percent to 20 percent of the United States population.

In Pennsylvania, the projected increase is slightly larger. People over 65 will comprise 22.6 percent of the population by 2030 going from 1.9 million to over 4 million older citizens.

As the baby boom generation ages, we will need to ensure that we continue to provide high quality care to America's older citizens while containing costs. The Affordable Care Act took steps to reduce costs and ensure quality care. The law is working to close the Medicare prescription drug "donut hole" by 2020. In 2010, more than 1.2 million Medicare beneficiaries who hit the "donut hole" received a $250 rebate check. This year any beneficiary that falls into the donut hole will receive a 50% discount on their brand name prescription drugs. As of the end of May, almost 36,000 Pennsylvanians have already received this discount.

While these were important steps to protecting America's older citizens, we still must work to ensure that we keep prescription drugs affordable for all of America's older citizens. We must attempt to keep co-pays low and to reduce the cost to the Medicare program.

In 2010, the United States spent $300 billion on prescription drugs. Medicare and Medicaid paid for approximately one-third of these expenditures. According to the testimony given by the Organization for Economic Cooperation and Development (OECD) to the Special Committee on Aging in 2009, "the average price of 181 pharmaceutical drugs in the United States in 2005 was 30 percent higher than the average in other OECD countries. Other studies (e.g. McKinsey Global Institute, 2008) suggest that this is an underestimate, and the true difference in price is as much at 50 percent".

Congress must work to improve data sharing among Part D plans and ensure that we work together with all the Part D plans to prevent waste, fraud and abuse of the Medicare program. The Affordable Care Act made important changes in the Medicare and Medicaid programs by strengthening Medicare and Medicaid's existing compliance and enforcement tools, reducing fraud and abuse and saving billions of taxpayer dollars.

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These new and important initiatives will lead to better care and lower costs for Medicare and Medicaid beneficiaries and taxpayers.

These are important steps forward that we must take. Our older citizens need and deserve quality and coordinated health care as they age. We must ensure this generation that fought in our wars, worked in our factories, taught our children and gave us life are loved and cared for. We owe them respect and dignity as they age. While we may differ on solutions on how to lower prescription drug prices, it is imperative that we continue the discussion on the best solution for America's older citizens. This is a daunting task, but a task we simply must undertake.

I look forward to hearing the testimony of all the witnesses today as they share their knowledge and experiences with the committee. And I look forward to working with them, the members of this committee and others to ensure that our older citizens will have the care they need – and deserve – in their later years.

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Senator Jay Rockefeller
Statement for the Record
Senate Committee on Aging
Hearing on Drug Costs to Medicare and Medicaid
July 21, 2011

Mr. Chairman:

Thank you for holding this important hearing today on the cost of prescription drugs to Medicare and Medicaid. As we continue the difficult discussions about the status of our national deficit, these critical programs once again find themselves under scrutiny. Yet all too often, it is the beneficiaries themselves who are scrutinized, and even punished, during these types of debates – instead of addressing the true underlying costs of care.

Already this year, we have seen multiple proposals that would raise out-of-pocket health care costs for seniors and people with disabilities who rely on Medicare and Medicaid. The House Republican budget would end Medicare as we know it, forcing tomorrow's seniors to spend on average $6,400 more for their health care compared to current law. As if that wasn't bad enough, our Republican colleagues have now proposed a Balanced Budget Amendment that would result in additional Medicare benefit cuts of up to $2,500 per senior.

These drastic proposals – as well as other proposals that would raise seniors' health care cost-sharing to reduce the deficit – are simply not grounded in the reality of seniors' lives. Half of all Medicare beneficiaries live on less than $22,000 per year. More than half of dually eligible beneficiaries, who rely on both Medicare and Medicaid, scrape by on less than $10,000 per year. Medicare beneficiaries pay, on average, 25 percent of their total health care expenses out-of-pocket. Given these pressures, it is imperative that we closely examine the special interests who all too often drive up health care costs without any measurable improvement in seniors' health.

We should be talking about ways to lower health care costs for seniors– not just piling more cost-sharing onto beneficiaries. That is why I am grateful today for the opportunity to talk about ways to reduce one very important component of health care costs – the cost of prescription drugs, which millions of seniors depend on. The health reform law already made some important strides to make prescription drugs more affordable for seniors, with a $250 rebate check for those who fall into the Medicare prescription drug "doughnut hole" last year, a 50% discount on brand-name drugs this year, and ultimately closing the coverage gap by 2020.

However, there is much more we can do to slow the growth of prescription drug costs and achieve major savings for seniors and taxpayers. The U.S. spent $250 billion on prescription drugs in 2009, andU.S. spending is projected to increase to $457.8 billion in 2019. According to

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the Medicare Trustees, Medicare spent $61.7 billion on prescription drugs last year, and the cost of the Medicare prescription drug plan is expected to grow by 9.7 percent per year over the next nine years. As for Medicaid, prescription drugs have been one of the fastest-growing Medicaid services, with spending totaling $19.4 billion for prescription drugs in 2008.

We need to responsibly reduce our deficit, but taking away health care for seniors and other vulnerable people is a step backward. Rather than dismantling Medicare and Medicaid, or increasing cost-sharing for already struggling seniors, we can save seniors and taxpayers money through a series of measures, including some being unveiled here today. Therefore, I would like to turn to just two proposals that could be considered as a way to save money on prescription drugs.

The first proposal would help generic drug companies enter the market more quickly. It is widely acknowledged that generic drugs are a significant cost-saver. For example, according to the Congressional Budget Office, generic substitution (dispensing generic drugs rather than their brand-name counterparts) reduced total Medicare prescription drug costs in 2007 by about $33 billion. If no generics had been available, the Medicare program and beneficiaries would have paid 55 percent more for prescription drugs. More broadly, for the decade 2000 through 2009, the use of generic prescription drugs in place of their brand-name counterparts saved the nation's health care system more than $824 billion dollars. According to an analysis by IMS health, every 2% increase in generic utilization in Medicaid programs saves taxpayers an additional $1 billion annually.

It is because of the importance of access to generic medications that my colleagues and I introduced the *Fair Prescription Drug Competition Act of 2011* (S. 373). This bill will eliminate one of the most prominent loopholes that brand name drug companies use to limit consumer access to lower-cost generic drugs, ending the marketing of so-called "authorized generic" drugs during the 180-day exclusivity period that Congress designed to provide specific incentives to true generics to enter the market. An authorized generic drug is a brand name prescription drug produced by the same brand manufacturer on the same manufacturing lines, yet repackaged as a generic. After up to 20 years of holding a patent for a brand name drug –the brand-name manufacturer – which has already been handsomely rewarded for its investment -- doesn't want to let go of its profits. So, it repackages the drug and refers to it as a generic in order to extend its market share, while cutting in half the financial incentive for an independent generic to enter the marketplace. This is a huge problem and one that is becoming even more prevalent as patents on some of the best-selling brand name pharmaceuticals expire. Authorized generics only serve to reduce generic competition, extend brand monopolies, and lead to higher health care costs for consumers over the long-term.

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To further hold down costs, the federal government should use its purchasing power to obtain lower drug prices in the Medicare prescription drug program. That is why my colleagues and I introduced the*Medicare Drug Savings Act of 2011 (S. 1206).*This bill, which to date has been endorsed by AARP, the American Federation of State, County and Municipal Employees, the Center for Medicare Advocacy, Families USA, Leading Age, the Leadership Council on Aging Organizations, the Medicare Rights Center, and the National Committee to Preserve Social Security and Medicare, would require that prescription drug manufacturers pay higher rebates for prescription drugs provided to low-income seniors through Medicare. Prior to the establishment of the Medicare Part D Prescription Drug Program, brand-name drug manufactures paid a drug rebate for dually eligible beneficiaries in Medicare and Medicaid. However, when the new Medicare drug program was established, drug companies no longer had to provide rebates for the now 9.2 million dual eligibles who receive coverage through Medicare Part D. Since that time, prescription drug prices have continued to rise faster than inflation, and drug prices for dual eligibles were significantly higher under the new Medicare prescription drug program than they would have been with the rebate under Medicaid.According to the Congressional Budget Office, this legislation would reduce the deficit, saving taxpayers $112 billion over the next ten years. And contrary to the claims of some critics, this bill has nothing to do with price-fixing or taking benefits away from beneficiaries. Part D plans will continue to set benefit levels for all beneficiaries in the same way they do now. All this legislation does is say that drug manufacturers have to give the same discounts to Part D that they give to Medicaid for low-income populations.

These proposalsare responsible steps to get our growing deficit under control. Rather than dismantling Medicare and Medicaid, or placing an ever-increasing burden on seniors, we should be supporting fair and sensible proposals to reduce health care costs, not allowing special interests to take advantage of loopholes.

I thank the Chair.

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Statement of Senator Mark Udall

Senate Special Committee on Aging

Hearing: "A Prescription for Savings: Reducing Drug Costs to Medicare"

Thursday, July 21, 2011

Thank you Chairman Kohl and Ranking Member Corker for convening us here today. The timing of this hearing could not be more appropriate. As we continue working whatever hours and as many weekends as it takes to find a compromise on addressing our deficit and debt, our enormous spending on drugs must be part of the discussion. Both for the fiscal health of our country, but also for the physical and mental health of American seniors. And their pocketbooks.

Mr. Blum, thank you for being here today – I don't envy the task that both you and the rest of the team at CMS have in front of you. I imagine it's both exciting and sometimes a thankless job - ensuring the success of the Affordable Care Act and pushing for smart changes and innovations to make Medicare and Medicaid work better is a tall order. But it's imperative.

I want to commend you, Administrator Berwick, and all of your hardworking colleagues for your commitment – and, quite frankly, on your patriotism for taking on this endeavor.

Thank you, also, to the rest of the panelists here today. I know your expertise and experience will help make this a fruitful discussion.

I want to say from the outset that I know this is a difficult balance. Ensuring both innovation and financial success for the pharmaceutical and biotech sectors while keeping a downward pressure on costs to patients and the government is not easy.

But, while we can sit here and spar over the numbers, going to the granular level in disputing profit levels, costs for branded drugs versus generic drugs, the success of Medicare Part D, and so many other issues associated with the cost of prescription drugs, the big numbers are too hard to ignore.

We're trying to avoid a default on our debt that could send the global economy into a tailspin. And many of my colleagues here today and I are trying to find common ground on painful decisions to get our fiscal house in order so that we don't leave our economy in shambles for our children. Now, I've seen the numbers, and we simply *cannot* afford to spend more than $250 billion per year on prescription drugs – particularly when that number is estimated to more than double in ten years.

The solutions need to go both ways – if we're going to consider policies or legislation that would squeeze the profits of the drug industry, we also need to consider polices that will decrease burdensome regulations and continue to drive innovation.

So I look forward to this discussion. It is an important one. And it's timely.

And as we continue on, I want to just remind myself and those participating of the human element here. Yes, these are big numbers with economic consequences. But, at a base level, this is about getting affordable drugs into the hands of those who need them most in order to stay healthy – or, in many cases, in order to save their lives.

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August 1, 2011

The Honorable Herbert H. Kohl
Chairman
Special Committee on Aging
U.S. Senate
G-31 Dirksen Senate Office Building
Washington, D.C. 20515

Dear Chairman Kohl:

Thank you for your July 21st hearing entitled "A Prescription for Savings: Reducing Drug Costs to Medicare." The Academy of Managed Care Pharmacy (AMCP) is pleased to have the opportunity to submit the following comments and positions on this important topic for the record of the Special Committee on Aging.

AMCP is a national professional association of pharmacists and other health care practitioners who serve society by the application of sound medication management principles and strategies to improve health care for all. The Academy's 6,000 members develop and provide a diversified range of clinical, educational and business management services and strategies on behalf of the more than 200 million American covered by a managed care pharmacy benefit.

Managed care pharmacists in health plans and pharmacy benefit management (PBM) companies are responsible for a broad and diversified range of clinical and quality-oriented drug management services. There are more than 18,000 pharmacists working for health plans and PBMs. It is their responsibility to make sure that the plan they design provides individual patients with appropriate drugs and drug therapies, conveniently, safely and cost-effectively. They are committed to ensuring that medications are used appropriately to improve a patient's health.

Of the many functions managed care pharmacists perform, of particular relevance to the Committee's hearing is their work in business and cost management of prescription drugs. Managed care pharmacists contract with employer and health plan clients, pharmacies and manufacturers to structure business arrangements and perform the following tasks:

- Negotiating with manufacturers for discounts on drug prices for clients in exchange for moving market share when clinically appropriate;
- Allowing their clients to customize clinical and reporting requirements to meet their individual population needs;

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RegenceRx
Boise, ID

Chief Executive Officer
Judith A. Cahill, CEBS
AMCP
Alexandria, VA

100 North Pitt Street | Suite 400
Alexandria, VA 22314
800 827 2627 | 703 683 8416
Fax 703 683 8417
www.amcp.org

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- Assisting clients in assessing the appropriateness of new drugs; and
- Establishing networks of pharmacies to provide accessibility for patient populations and assure participating pharmacy compliance with patient safety and quality programs.

In addition, managed care pharmacists help their clients (employers, HMOs, trust funds, Medicare, Medicaid, etc.) manage costs, as well as evaluate and improve their pharmacy benefit by engaging in the following activities:

- Encouraging prescribers to make cost effective drug choices that are clinically appropriate;
- Integrating improvements so that costs are actually saved, not merely shifted;
- Introducing system interventions that enhance the quality of patient care and save costs; and
- Using data to identify adherence and nonadherence with prescribing guidelines, and by creating measures for assessing physician performance, identifying prescribing patterns and determining opportunities for improvement;

The following summary statements set forth AMCP's positions on issues in which the Academy maintains established policy and which are issues of high priority to its members:

Generic Exclusion Agreements

We have appreciated working with you and continue to support your leadership in pursuing legislation to prohibit generic exclusion agreements. AMCP believes that such agreements deny patients access to affordable generic drugs, unnecessarily raising prescription drug costs for consumers, employers, health plans and taxpayers. AMCP will continue to work with you to support passage of S. 27, "Preserve Access to Affordable Generics Act."

Federal Government Negotiation of Prescription Drug Prices

With regard to the Medicare Part D program, AMCP reaffirms its support for continuation of the competitive model enacted in the Medicare Modernization Act of 2003. AMCP seeks to maintain the success of the Medicare Part D program and believes that federal government negotiation of drug prices would undermine the financial stability of the successful Medicare Part D program by removing a critically important tool that prescription drug plans have used to effectively manage the quality and costs of prescription drugs. It is important to note that the Congressional Budget Office (CBO) has stated that federal negotiation of prescription drug prices would provide no significant additional savings to the government than currently is achieved by Medicare prescription drug plans. Attached are four papers on the future of the Medicare Part D program that reflect the Academy's views on this subject in greater detail.

Medicaid Rebates for Dual Eligibles

With regard to Medicaid rebates for dual eligibles, AMCP believes that to provide the greatest value to Americans who need prescription drugs, market forces must effectively ensure that manufacturers of similar drugs compete with one another to establish reasonable pricing levels

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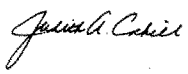
and maintain consumer access to needed therapies. While government has a responsibility to protect consumers against anticompetitive activity, the government must not establish rules that have the unintended effect of undermining competition. AMCP believes that the best price provisions of the Medicaid prescription drug rebate program represent interference by the government into the competitive marketplace that has raised costs unnecessarily by preventing the commercial market from allowing true market dynamics to emerge. Attached is the AMCP position statement entitled, "Where We Stand on the Best Price Requirements of the Medicaid Rebate Program" that addresses the Academy's views in greater detail.

Regulation of Pharmacy Benefit Management Companies

With regard to transparency in pharmacy benefit management (PBM) companies, the Academy believes that government should encourage an environment in which pharmacists working within managed care organizations, including PBMs, can continue to develop innovative and integrated strategies to manage prescription drug benefits for a given patient population. A properly developed and managed pharmacy benefit not only maximizes positive patient outcomes, but also helps to maintain the affordability of the prescription drug benefit itself. It is essential that managed care pharmacists have broad latitude to exercise their professional judgment in structuring drug benefit programs. Onerous regulations can prevent managed care organizations from properly reacting to clinical and economic realities (e.g., safety issues or encouraging the use of a less expensive, yet therapeutically equivalent alternative), patient noncompliance with drug regiments and other practical considerations. AMCP recognizes that certain proprietary information, such as negotiated drug prices and rebates, needs to remain confidential in order to maintain a competitive marketplace. Attached are two position statements entitled "Where We Stand Position Statement on Transparency in Health Care" and "Where We Stand on Regulation of Pharmacy Benefit Managers (PBMs)."

Please do not hesitate to contact us whenever we may be of assistance. The Academy stands ready to discuss these issues with you further. Thank you again for your efforts to ensure access to safe and affordable prescription medications.

Sincerely,



Judith A. Cahill
Chief Executive Officer

cc: The Honorable Bob Corker
Ranking Member

Attachments:
The Future of Medicare Part D: Competitive Model

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"A Prescription for Savings: Reducing Drug Costs to Medicare"

Senate Special Committee on Aging

July 21, 2011

2:00 pm

Statement of

Daniel P. Perry

President and CEO

Alliance for Aging Research

750 17th Street, NW Suite 1100

Washington, DC 20006

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Chairman Kohl and Ranking Member Corker, thank you for the opportunity to provide testimony on behalf of the Alliance for Aging Research related to the Senate Special Committee on Aging's hearing exploring how to reduce drug costs in Medicare.

The Alliance for Aging Research is a private, not-for-profit organization dedicated to improving the lives of Americans as they grow older. We do this by advancing biomedical and behavioral research in aging and health.

As you well know on January 1, 2011 the U.S. went from having approximately 6,000 Americans turning age 65 every day, to 10,000 marking a 65th birthday. We will stay at that high level for the next 18 years. This aging of the population, accompanied by an increase in life expectancy, will result in a dramatic increase in the prevalence of chronic diseases and disability.

When I founded the Alliance in 1986, I did so believing that accelerating medical and scientific research into aging and promoting the translation of this research into treatments and cures for age-related disease was our best hope for staving off the prolonged human and economic burdens that the aging of the population will place on this country. I still believe that supporting the development of interventions aimed at keeping older American's healthier and more productive in their later years, is one of our best investments. Unfortunately, many of these innovations that delay the onset of disease and disability come at a cost.

Three years ago, the government announced a large and daring clinical trial designed to answer an intriguing question about how to treat age-related macular degeneration (AMD), the most common cause of blindness in the United States. The Comparison of Age-Related Macular Degeneration Treatments Trial known by its acronym, CATT, compared Lucentis, a drug designed specifically to treat the disease, with Avastin, a cancer drug with a mechanism similar to Lucentis that is, increasingly, also used off-label to treat AMD. Avastin had not been widely studied in or approved in AMD, and researchers were curious to know if the drugs had the same effect.

This could have been a study of interest mostly to eye specialists, but instead it gripped a much wider community because of one stark difference between the drugs: Avastin is much less expensive in treating AMD. A finding that the two drugs were equal would provide evidence to the government or insurance companies that it might be safe to encourage more widespread use of the less expensive option.

In May, the results came in. The two drugs might be similar, but not the same: Avastin's effectiveness was worse than Lucentis', but by an amount too small for researchers to declare it inferior. And Avastin had slightly more side effects. For individual doctors and patients, the data will no doubt make decision-making more informed.

But the results are also concerning, because the research – and other comparative effectiveness research like it – will no doubt be considered by insurers, who will wish to use it to influence treatment decisions for a huge population of people, potentially removing – rather than adding to – patient and physician choice. And unlike the investigators in the clinical trial, these officials will consider the costs of the two drugs.

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On its face CER is reasonable and laudable. CER calls for scientific analysis of what works best in health care by comparing the relative effectiveness of medications and other health care interventions. And that is a good thing for doctors and patients to have this information when making care decisions.

But using CER becomes precarious when insurers, both private payers and Medicare, are placed in the position of asking the question: is less efficacy and a questionable safety profile in some patients worth the cost savings?

The study of biology and medicine over the past 10 years has been an object lesson in individual variation: each patient is unique, and each patient requires a treatment that is appropriate for them. The use of CATT findings by insurers to limit treatments for a large number of patients based solely on cost would send a signal to drug makers that runs directly counter to what we have learned about medicine, and has the potential to allow cost to trump investment in future research and development.

On November 3, 2010, prior to CATT's s' completion, the Alliance for Aging Research convened a panel of international policy experts, academics, economists, regulators, payers, physicians, and patient advocates for a global summit on CER and patient access. The primary goal of the summit was to frame the challenges presented by CER that includes cost considerations as a driver for initiating the research and form consensus on necessary steps that should be taken to continue promoting innovation in an environment where evidence is becoming more readily available to influence provider, patient, and payer decision-making. The summit discussion used CATT in the US and similar trials abroad as a lens through which to view the potential impact of head-to-head clinical trials on innovation and patient access.

During the summit a question was posed to the group, "What systemic changes are needed to balance spending and innovation?" Participants suggested a number of ways to drive innovation while remaining cost effective: including: tax incentives, risk sharing, cost sharing, value-based purchasing, public/private partnerships, trial periods for products to prove effectiveness, and more accountable care. At the heart of all of these is the ability to judge, reward, and incentivize high value innovation. These values are at odds with system in an approach in which small but meaningful advances in medicine can be rejected primarily because of cost concerns. Such an approach will surely drive a majority of biopharmaceutical development away from research into promising approaches to our most deadly and disabling diseases.

While we recognize the important role CER plays in health care– and hope that CATT moves us closer to shrinking the disabling effect of vision loss on older Americans – we remain wary. Head-to head trials and the evidence they produce can hold great potential to steer and impact future innovation and could lead to exciting advances, when as a tool to make decision- making smarter. But used as a tool to remove choice from health care providers and their patients both now and in the future, it carries considerable peril.

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Written Testimony by Narinder Sharma,
Chief Executive Officer, AMD Alliance International
to the Special Committee on Aging
Thursday, July 21, 2011

Senator Kohl, Ranking Member Corker, Honorable Members of the Special Committee on Aging, and special guests. Thank you for the opportunity to provide written testimony on behalf of patients in the US and around the world living with age related macular degeneration.

My name is Narinder Sharma, and I am Chief Executive Officer of AMD Alliance International, a global consortium of 70 patient organizations in the US and around the world.

Our mission is simple, we exist to bring hope and help to individuals living with macular disease.

I write to you today to ask the committee to consider and recommend: maintaining a high standard of patient safety regardless of cost savings; investigating and responding early to warning signals of any evidence of harm; not changing the current legal framework or public health policies guaranteeing patient safety; not incentivizing physicians to use off-label treatments; and not denying patients access to approved treatments as a result of CATT.

We very much hope that you will seek to ensure that the regulatory framework for medicines is not undermined and patient safety is not compromised regardless of cost savings.

Today, in the U.S. alone, nearly two million Americans are visually impaired by AMD, while more than seven million are at increased risk of vision loss from the disease.

Untreated or inappropriately treated, wet AMD leads to declining sight and potentially blindness, which in turn dramatically increases the burden to society in direct health care costs, lost productivity, and the need for supportive services and care. Low vision and blindness rob a person of independence by impeding the ability to perform common daily tasks or to work at a paying job.

Today patients are fortunate to have access to Lucentis (Ranibizumab), an FDA-approved treatment that can halt vision loss and in some cases, restore vision.

Recently, one year results of the CATT[i] study concluded that the efficacy of off-label Avastin (Bevacizumab), a drug developed to treat colon cancer, appears to be non-inferior to Lucentis.

However, there are a number of things CATT does not tell us.

Although the CATT trial was not powered for safety, there were never-the-less safety signals in the study which warrant further investigation.

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The signals are concerning as there are additional studies which also highlight certain safety issues and include increased risks for mortality and hemorrhagic stroke from off-label use of Avastin (an 11% higher risk in overall mortality and a 57% higher risk of hemorrhagic cerebrovascular accidents [ii]); and an increased risk of arterial thromboembolic events in patients when treated with Avastin[iii].

In addition, safety signals, of new medicines, especially for relatively uncommon events such as endophthalmitis, stroke or myocardial infarction are notoriously difficult to detect, oftentimes for years following the approval and licensing of a new drug therapy. Examples of this abound, such as the recall of Vioxx®, the black box safety warnings for systemic bevacizumab and rosiglitazone (Avandia®), to name some germane instances.

Studies such as CATT can bring additional scientific insights on questions of efficacy. Other trials from around the world are due to report in the coming years. At a minimum these trials should all be brought to completion because the accumulated data from these trials will be crucial to a better understanding of the relative safety risks of these two drugs. The accumulated clinical trial data of bevacizumab vs. ranibizuamb will hopefully make possible a high quality meta-analysis to compare the safety signals mentioned.

Since the one-year results were released, we have been concerned that policy-makers may use the results of CATT and anecdotal reports to undermine a high standard of patient safety and circumvent in practice the principle of informed consent.

Our fears are being realized as this hearing today on reducing drug costs to Medicare includes a case study of the comparison of an FDA approved treatment, which has gone through the rigorous approval process, with an off-label treatment.

Patient safety, patient choice and patient informed consent must remain the guiding principles of healthcare systems. Patients deserve access to effective, safe medicines regardless of cost. We also need to ensure that there is a system in place of pharmacovigilance for each and every treatment on the market.

To ensure the best outcomes, patients need treatment choice. We know that individual patients respond differently to treatments. Decisions about treatments should be between a patient and his or her doctor. In addition, new treatments coming to market are critical. Any CER discussion and policy considerations or changes should encourage innovation, not stifle it.

The US has a first class system of evidence-based medicine. We understand the use of off-label drugs when no alternative exists. But we have a safe and proven treatment in Lucentis. The first year CATT data does not provide enough information to change policy.

Therefore, we again ask the committee to recommend: maintaining a high standard of patient safety regardless of cost savings; investigating and responding early to warning signals of any evidence of harm; not changing the current legal framework or public health policies guaranteeing patient safety; not incentivizing physicians to use off-label treatments; and not denying patients access to approved treatments as a result of CATT.

We very much hope that you will seek to ensure that the regulatory framework for medicines is not undermined and patient safety is not compromised regardless of cost savings.

Until all safety questions are answered, patients deserve access to effective, safe medicines regardless of cost and full disclosure about all options, benefits, and risks.

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The gold standard for the treatment of "wet" age-related macular degeneration remains intra-vitreal ranibizumab. Cost savings should not trump safety concerns in any disease.

Thank you.

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[i] The CATT Research Group. Ranibizumab and Bevacizumab for Neovascular Age-Related Macular Degeneration, *New England Journal of Medicine*, April 28, 2011 (10.1056/NEJMoa1102673)
[ii] http://www.abstractsonline.com/Plan/ViewAbstract.aspx?sKey=3a667d20-f42d-421e-a859-e1b680de80ed&cKey=4e534aee-b678-4b9d-91dc-20a9d6ae0c56&mKey=%7b6F224A2D-AF6A-4533-8BBB-6A8D7B26EDB3%7d
[iii] Carneiro AM, et al. Arterial thromboembolic events in patients with exudative age-related macular degeneration treated with intravitreal bevacizumab or ranibizumab. *Ophthalmologica* 2011;225:211-221.

Contacts:
Narinder Sharma
narinder.sharma@amdalliance.org

Allie Laban-Baker
Allie.labanbaker@amdalliance.org
443-386-5032

Global Office	USA	Japan:
City Gate East, Tollhouse Hill	10519 Old Court Road	1-31-1-502, Kaminoge
Nottingham, UK	Woodstock, MD,	Setagaya-ku, Tokyo

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Statement of the National Community Pharmacists Association

United States Senate Special Committee on Aging

Hearing on a Prescription for Savings: Reducing Drug Costs to Medicare

July 21, 2011

The National Community Pharmacists Association (NCPA) welcomes and appreciates this opportunity to provide input and suggestions regarding efforts to reduce drug costs within Medicare Part D. NCPA represents the pharmacist owners, managers and employees of more than 23,000 independent community pharmacies across the United States. The nation's independent pharmacies, independent pharmacy franchises and independent chains dispense nearly half of the nation's retail prescription medicines. According to a recent survey conducted by NCPA, in 2010, 30% of the average NCPA member pharmacy's business was Medicare Part D business.[1]

The Federal government pays tens of billions of dollars for the Medicare Part D program. NCPA strongly believes in the mission to generate savings within the program, while maintaining vital patient access to the drugs that Part D beneficiaries need. NCPA and our members stand willing and able to assist Congress in generating savings within the Medicare Part D program, particularly through our focus on dispensing of generic drugs, when generic equivalents are available. We offer our services to streamline the delivery of pharmaceutical products and to promote cost-effective health care. Accordingly, NCPA urges Congress to recognize the role that independent community pharmacies can play in generating savings within the Medicare Part D program.

At the same time as NCPA is willing to assist in generating Part D drug savings through higher generic dispensing rates, we also urge Congress to focus on the billions of dollars in Part D drug savings that remain "on the table" because of the lack of competition and oversight in the management of Medicare drug benefits by pharmacy benefit managers (PBMs). Through recent legislative action, Congress has seemingly demonstrated that it continues to be concerned regarding how PBMs run their businesses. In the Affordable Care Act, Congress imposed new transparency requirements on the PBMs operating within the Medicare Part D program and for PBMs operating in the new state-based health insurance exchanges, which come on-line in 2014.

[1] *Preliminary data from 2011 NCPA Digest, National Community Pharmacists Association.*

THE VOICE OF THE COMMUNITY PHARMACIST

100 Daingerfield Road
Alexandria, VA 22314-2888
(703) 683-8200 PHONE
(703) 683-3619 FAX

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Accordingly, NCPA and independent community pharmacists want Congress to know that we are committed to improving savings within Medicare Part D, but we remain concerned that some PBMs are apparently contributing to waste within the Medicare system and ask Congress to take action to reign in such waste.

Recommendations

PBMs are promoting wasteful mail order programs within the Medicare Part D program and retaining billions of dollars in Medicare Part D rebate savings, which should inure to the benefit of the Medicare Part D program. On the other hand, NCPA and independent community pharmacists are committed to promoting savings within the Medicare Part D program. Accordingly, we offer a number of proposals for achieving that goal:

- Take action to promote generics in Medicare Part D as a method of generating savings instead of promoting mail order, which is actually more wasteful than it appears.
- Take action to force PBMs to share with the government billions in rebate savings that the PBMs retain under the Part D program. Accordingly, NCPA supports Senator Kohl's proposal to require PBMs to disclose to federal government and employer payers the rebates that they receive from manufacturers.
- Pass the Pharmacy Competition and Consumer Choice Act of 2011, S. 1058, which promotes PBM transparency and will highlight savings to be attained from PBM reforms within the Medicare Part D program. The legislation would:
    1. Require that PBMs confidentially disclose certain information to private insurance plans about, for example, whether the PBM is passing along – or pocketing – manufacturer rebates; and
    2. Prohibit forcing patients to use a specific pharmacy (retail, mail, specialty) if the PBM has an ownership interest in the pharmacy (or vice versa).

Increase Use of Lower-Cost Generic Medications

Generic drugs are one-fifth the cost of brand name drugs. Nothing can save Medicare Part D more money than, where medically appropriate, dispensing every possible prescription with a generic drug rather than a brand, even after accounting for the lucrative rebates that PBMs earn on brand name drugs. Yet, Medicare Part D generic dispensing rates remain lower than the national average.

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The Congressional Budget Office (CBO) recently reported that the generic dispensing rate in Medicare Part D is 64%.[2] Mail order generic dispensing rates are even lower. This should be compared to a generic dispensing rate by retail pharmacies of approximately 72%.[3] Accordingly, claims that increasing mail order in Medicare Part D will save the program money are overstated. Not only does the use of mail order fail to capitalize on the use of low cost generic drugs, but the increased use of mail order drives results in fewer opportunities for face-to-face interventions by pharmacists. These interventions improve drug therapy and reduce health care spending on expensive health care encounters in the hospital emergency room. Greater reliance on the delivery of prescription drugs through retail pharmacy will improve generic dispensing rates in Medicare Part D, thereby achieving billions of dollars of savings over the existing system. Here is a comparison of generic dispensing rates of mail order pharmacies to retail pharmacies:

Mail Order Generic Dispensing Rates[4]

CVS/Caremark	61.3%
Medco	61.5%
Express Scripts	60.2%

Community Pharmacies Generic Dispensing Rate[5]

72%

In light of the greater generic utilization-based savings available in retail versus mail order pharmacy, NCPA urges Congress to take action to promote the use of generics within Medicare Part D rather than promoting the use of mail order, which has a lower generic dispensing rate.

Collect Billions of Dollars in Manufacturer Rebates Being Retained by PBMs

Most federal programs use pharmacy benefit managers (PBMs) to administer drug benefits. These include Medicare Part D, Medicaid, FEHBP, and TRICARE. Yet, the federal government is unable to determine accurately whether PBMs are passing through to taxpayers or beneficiaries the billions of dollars in rebates they receive from manufacturers for drugs covered for enrollees in these Federal programs. In addition, federal programs are not currently tracking whether PBMs retain a percentage of total manufacturer rebates as well as funds that are intended for pharmacy professional services and cost of dispensing.

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[2] Congress of the United States, Congressional Budget Office, *Effects of Using Generic Drugs on Medicare's Prescription Drug Spending*, September, 2010.
[3] Based on statistics contained within the 2010 10K SEC filings for the big 3 PBMs.
[4] *Id.*
[5] *Preliminary data from 2011 NCPA Digest, National Community Pharmacists Association.*

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There is cause for concern. A recent OIG report found that for the year 2008, Part D sponsors received $6.5 billion in rebates, yet some sponsors may be inappropriately allocating rebates across their plans in order to maximize reconciliation payments inappropriately.[6] Notably, according to the OIG, most PBMs did not pass the full amount of rebates on to beneficiaries, and only 4 out of 258 sponsors provided rebates to beneficiaries at the point of sale.[7]

The OIG also found that sponsors underestimated rebates in 69% of their bids and 78% of Part D beneficiaries were enrolled in plans that underestimated rebates.[8] These underestimations lead to higher premiums for Part D beneficiaries and overpayments by CMS. This high percentage of underestimates may indicate that some PBMs deliberately underestimate their rebates in order to increase their profits. There is no consistency, uniformity or transparency in determining whether or how these rebates are going to lower drug costs in these programs. Congress should take action to bring transparency to what happens to these rebates and force PBMs to pass through these rebates to the federal government.

NCPA urges Congress to enact the Pharmacy Competition and Consumer Choice Act of 2011, S. 1058, which implements much needed reforms in terms of increasing PBM transparency in terms of accounting for PBM rebates. NCPA also supports Senator Kohl's proposal to boost PBM rebate transparency with regard to government and employer payers. Such transparency will reveal the true cost and lost savings inherent in PBM drug management and demonstrate the availability of potential savings through PBM reform.

Recognize Wastefulness of Mail Order

It is a popular myth that mail order saves money. PBMs want payers to think that mail-order saves because the PBMs earn (and in many cases keep) significant manufacturer rebates from the large quantities of expensive brand name medications that they push through mail order. At the end of the day, however, these rebates may or may not be passed through to payers. Moreover, PBMs repackage medications under their own label, assign them a higher cost basis, and then make it appear that they are still giving a higher discount on mail order prescriptions. Finally, no amount of manufacturer rebates paid on a brand name drug can make a prescription less costly than if a generic is dispensed.

Community pharmacies do a much better job at dispensing generics because we don't have the perverse incentives that PBMs have to push brand name drugs through mail order outlets in order to collect lucrative rebates. The higher generic dispensing rate at retail pharmacies compared to mail order demonstrate that retail pharmacy is much more effective at promoting generic drugs than mail order pharmacies, which results in significant savings. NCPA believes that Congress should take action to stem the tidal wave in existing prescription drug payment policies that push drugs through the mail order channel to the exclusion of retail pharmacies and perversely promotes expensive brand name drugs over generic drugs.

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[6] Department of Health and Human Services, Office of the Inspector General, *Concerns with Rebates in the Medicare Part D Program*, Daniel R. Levinson, March, 2011, OEI-02-08-00050.
[7] *Id.*
[8] *Id.*

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For example, the TRICARE program is attempting to encourage more mail order use, even though the TRICARE mail order contractor only dispenses generic drugs just over 50 percent of the time. [9] This is at least 10 percentage points lower than even other mail order programs, where the generic dispensing rate is already low. Compare this to the fact that retail pharmacies in the TRICARE network dispense generic drugs over 70% of the time.[10]

Conclusion

NCPA and its members remain committed to generating savings within Medicare Part D and stand at the ready to assist with these efforts. To promote savings in Medicare Part D, Congress must enact the proposed legislation above, which will focus on the existing waste within and potential savings from how PBMs handle Medicare Part D rebates. Congress must also recognize the strength of community pharmacy in creating high generic dispensing rates and generating valuable savings for the government through those rates. In the end, NCPA seeks to partner with the federal government in the right way to generate health care savings, while providing high quality health care to our patients.

[9] Based on statistics provided to NCPA by the TRICARE Pharmacy Program.
[10] *Id.*

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A Proposal for a Reimbursement "Critical Path" for CMS

March 9, 2007

Background and Purpose

The National Venture Capital Association (NVCA) is the trade association that represents the U.S. venture capital industry. NVCA's mission is to foster greater understanding of the importance of venture capital to the U.S. economy and to support entrepreneurial activity and innovation. Because the venture capital community works closely with the companies developing innovative new technologies, the NVCA offers a unique perspective on Medicare's procedures and policies for coverage and payment of emerging medical products.

NVCA believes that the interests of Medicare beneficiaries, CMS, product developers and investors could all be served by improving the efficiency, transparency and predictability of the reimbursement process, particularly for novel, high value technologies. CMS has already taken several important broad steps in this direction, including refinements in the national coverage process and establishment of the Council on Technology and Innovation. We believe that building on these initial efforts will help to ensure a favorable economic and policy environment for making valuable new technologies available to patients and clinicians.

Through discussions with venture capital investors, early stage life sciences companies, reimbursement consultants and policy experts, we have identified a number of circumstances under which reimbursement decision making is impeded due to problems with the reimbursement process itself, and not primarily because of inherent questions about the clinical effectiveness or value of the technology under review. We believe that it is in the interest of all stakeholders to minimize such barriers. Such problems are particularly likely when a technology is novel and may therefore not fit readily into established pathways for coverage, coding and payment. Yet it is precisely these novel technologies that are most likely to represent important clinical breakthroughs, and to significantly improve outcomes for Medicare beneficiaries.

The goal of the NVCA Workgroup on Healthcare Innovation Policy (WHIP) is to identify potential improvements in reimbursement procedures and policy that will help ensure access to important, clinically effective, high value technologies. In this memo, we introduce the concept of a "Reimbursement Critical Path", having chosen this name to highlight the common goals shared by this effort and the FDA Critical Path initiative. Both policy initiatives seek to support efficient development and adoption of novel, clinically effective and high value technologies.

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Specific objectives

The reimbursement critical path would be a clearly defined set of procedures and policies that would result in rapid reimbursement decision making for novel, high value technologies. This pathway would include refinements in coverage, coding, and payment. Because most novel technologies will be different than existing technologies, existing reimbursement mechanisms may create uncertainties and delays that impede the development of optimal reimbursement requirements for that technology. The recommendations discussed below are intended to ensure that potential reimbursement barriers to novel technology are identified and refined.

The proposed policy refinements may have a profound impact on investment in and appropriate use of novel, high value technologies. We are particularly attentive to the risks associated with investment in those promising technologies for which there is not yet a well-established reimbursement pathway. The reimbursement critical path could help ensure that such technologies became more attractive for financial support.

It is also important to ensure that reimbursement issues are fully considered in the context of ongoing FDA critical path discussions, in which NVCA is also engaged.

What are novel, high value technologies?

We propose to focus initial attention on improving reimbursement mechanisms for technologies that fit an explicit definition of "novel and high value". We believe that efforts to streamline reimbursement for this subset of technologies may lead to policy enhancements that would impact a wider range of technologies in the future, improving the predictability and transparency of these processes, while ensuring the reimbursement policy works more effectively to enhance both innovation and value in health care.

While it will probably be necessary to go through a process to develop criteria to identify those technologies that are novel and high value from the perspective of CMS, it should be possible to begin pilot efforts at rapid reimbursement decision making by focusing on technologies deemed by the FDA to be eligible for accelerated approval (drugs/biologics) or expedited review (devices). The FDA has existing mechanisms for designating specific technologies to be of sufficient potential public health and clinical importance to qualify for special attention during regulatory review. It may be reasonable to assume that all or most of these technologies would merit preferred reimbursement attention as well.

For a CMS-specific definition, we have done some preliminary thinking about how to define novel, high value technologies: A novel technology is a product that represents the first expression of a technology or use of a technology, that is likely to have clinically significant benefit, and raises potentially significant coverage, coding, and/or payment hurdles.

A number of considerations would be factored into the judgment about whether a technology is "potentially of high value", from the perspective of the payer, the health care system, and society. The criteria listed below will require further refinement, but are intended to capture some of the critical characteristics of new technologies that would reflect on their value.

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- *Safety*: The degree to which the technology may reduce the risk of adverse events for patients or health care providers.

- *Clinical effectiveness*: the expected magnitude of improvement in patient health outcomes, including mortality, morbidity, quality of life, functional status. Improvements in the timely and efficient delivery of care would also be a factor.

- *Clinical efficiency*: the expected impact of the technology on resource utilization, assessed at the level of individual patients. Short term and long term savings would need to be considered, as well as savings that occur across the various care settings.

- *Strength and consistency of evidence*: the level of confidence that the judgments about clinical effectiveness and clinical efficiency are reliable based on scientific studies, pathophysiologic reasoning, economic modeling, clinical judgment and other sources of information. The assessment of the evidence will need to be undertaken with recognition of the practical and economic challenges to being able to definitively prove benefits for novel technologies.

- *Organizational efficiency*: the expected impact of the technology on resource utilization, assessed at the level of health care institutions and the health care system. This would include impacts on worker productivity, increasing the capacity to use existing facilities and technologies, etc.

- *Support future innovation*: Consideration should also be given to those technologies that may represent an early generation of a new category of technologies for which refinements will provide major benefits, and may also lead to important related applications.

Examples of novel, high value technologies

Currently marketed	**Under development**
Angioplasty	Biodegradeable or novel stents
Coronary stents	Neurostimulation
Drug eluting coronary stents	PFO closure
Kyphoplasty	Vulnerable plaque
Laproscopic gallbladder surgery	Cartilage repair
Implantable defibrillators	Endoscopic lung volume reduction
Catheter arrhythmia ablation	Cardiac remodeling device
RF/microwave BPH	Ultrasound enhanced perfusion
Percutaneous femoral closure	Hypothermia
Carotid stents	Percutaneous DVT treatment
Spinal fusion cage	Spinal Nucleus Replacements
	Dynamic Spine Replacements

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Specific Recommendations

The recommendations provided below are those that we believe are sufficiently focused and concrete that they can be implemented within a reasonable amount of time and with existing CMS staff. A few of the recommendations will require more time, further thought and discussion, and additional resources. Whenever possible, we include a specific example to illustrate the type of problem that each recommendation is intended to address, though in many cases we have not provided the name of the company involved at their request. Addressing the concerns that have lead most companies to requesting anonymity in this exercise is a symptom that we hope will be improved through continued dialogue on reimbursement issues.

Recommendation #1: Expand the role of the Council on Technology and Innovation

The Council on Technology and Innovation (CTI) could function as the primary organizational component to support development and implementation of the reimbursement critical path. Because efficient reimbursement policy requires coordination across several CMS components, and in some cases coordination with FDA, NIH, AHRQ and other institutions, a cross-component entity like CTI is ideally positioned to support this process.

The existing statutory language establishing CTI is also entirely consistent with the objectives of the reimbursement critical path, as that language directs the CTI to focus on improving coordination for coverage, coding and payment in order to minimize barriers to access to new technologies.

Rec 1(a): The CTI could be asked to develop a written strategy to be presented to the CMS administrator with a prioritized plan for administrative and regulatory reforms that might support continued progress toward the goal of efficient reimbursement procedures for novel, high value technology.

Rec 1(b): It would be useful to have one or more public meetings at which stakeholders could present ideas for possible inclusion in the CTI strategic plan.

Rec 1(c): The executive director of the CTI, or a designee of that individual, should serve as an ombudsman for product developers who are having difficulty in resolving specific reimbursement problems despite reasonable diligence in working through existing decision making mechanisms within CMS. This is not intended to be a function through which product developers would appeal decisions that they find undesirable, but rather is for those situations in which the usual procedures have not generated a clear policy conclusion after a reasonable period of time and effort.

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Recommendation #2: Develop process descriptions

A common problem for early stage companies is the difficulty of understanding which part of CMS handles which issues, and who they should be talking to about specific reimbursement problems. Many early stage life sciences companies and investors have hired reimbursements consultants because they are unable to identify the appropriate point of access to CMS policy processes and staff. Many basic questions and problems could be resolved with modest efforts by CMS to make their various reimbursement policy making pathways, and their interconnections, more transparent.

Rec. 2(a): CMS should develop a roadmap that describes the primary coverage, coding and payment processes at Medicare for investors and life sciences companies. This document would not need to be highly detailed, but should identify the major decision making functions, what part of the Agency has responsibility for that function, contact information of We are aware that such a document in currently under development, and look forward to seeing the first draft.

Recommendation #3: Establish explicit timeframes

For most early stage companies, particularly those developing medical devices and diagnostics, each month of delay in resolving a reimbursement issue can translate into a substantial "burn rate" for their investment capital. In some cases, a substantial delay in the time required to arrive at a definitive answer on a reimbursement policy question can lead to economic failure even for companies with potentially useful technologies.

Examples include:

1) a company that has developed a unique method for wound debridement, but has been unsuccessful in reconciling differing advice between Medicare contractors and CMS central office staff on appropriate coding for the therapy.

2) a company with a product used to treat patients following certain kinds of strokes, where a conclusion on whether or not the product fits within a Medicare benefit category has remained unresolved after more than a year.

3) a company with a procedure for treatment of a chronic reflux esophagitis that encountered multiple issues around coding and payment rates that were resolve too late to prevent the company from shutting down.

Clearly, Medicare is under no obligation to ensure the economic success of products without clinical value, but the Agency should make itself more accountable for completing their decision making processes in a timely and predictable manner – whether or not those policy processes are governed by fixed statutory timeframes.

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Rec. 3(a) CMS should review current reimbursement policy processes that are not subject to fixed time frame and commit to a reasonable time frame within which these decisions will be made. For most such decision, a turnaround time of 3 to 6 months would seem adequate, though the Agency would be in the best position to determine what time frame would be reasonable. As noted further below, the Council on Technology and Innovation may be well positioned to oversee the development, implementation and monitoring for the proposed procedural improvements.

Rec. 3(b) In order to help identify and prioritize policy processes in need of improvement, CMS could hold a town hall meeting inviting stakeholders to present examples of problems encountered in resolving reimbursement issues. This would serve as an opportunity for CMS to gain a deeper understanding of the nature of these problems, and use that information to target their efforts to those processes that have proven consistently problematic.

Recommendation #4: Reimbursement for devices granted expedited review by the FDA

The potential value of greater coordination between FDA and CMS around review of novel technologies has been discussed over the past 5 years, and was mentioned as a priority in the report of Secretary Thompson's Medical Technology Innovation Task Force. FDA and CMS have some ongoing collaboration (e.g. biomarkers in oncology) and there are several examples of productive communication between CMS and FDA staff around the review of specific technologies (e.g. left ventricular assist devices, coronary stents).

We recognize that limitations on time constrain the level of collaboration than can be sustained between CMS and FDA, and would therefore proposed further exploration of carefully selected opportunities to increase coordination.

Rec 4(a): To begin with, we propose that class III medical devices that are undergoing expedited review by the FDA should be simultaneously reviewed by CMS to ensure that potential coverage, coding and payment policy issues have been identified well before final FDA approval. This would give the product developer and CMS adequate time to address these issues, with the goal that any problems have been resolved by the time the product is approved for marketing by the FDA, leading to simultaneous regulatory and reimbursement policy implementation.

As an initial pilot test, several medical devices currently undergoing review by the FDA under expedited review could be handled through this process. This could almost certainly be done under existing authorities, would require limited staff time and resources, and should quickly reveal whether the approach offers a useful pathway for efficient reimbursement of novel, high value technologies.

The CTI might be well positioned to oversee the implementation and refinement of this expedited reimbursement process, ensuring that all necessary policy making functions within CMS completed their decision making in a timely fashion.

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Rec 4(b): Once the mechanism for expedited reimbursement review had been established for medical devices undergoing expedited FDA review, CMS could expand this approach to other technologies determined to meet the criteria established for novel and high value technologies. A set of criteria have been proposed above, and these could serve as a starting point for further discussions within CMS, and between CMS and other stakeholders.

Recommendation #5: Clarify evidence requirements

The CMS has begun a process of becoming more explicit about the evidence requirement needed for national coverage decisions. The Agency has indicated its intention to generate guidance documents for coverage decision making regarding important categories of technologies, to continue its efforts to more clearly define the type of scientific evidence necessary to obtain national coverage under Medicare. None of these guidance documents have yet been issued, and we recognize that there are staffing limitations that contribute to the slow progress in this area.

More recently, it has become apparent that CMS is using evidence of comparative effectiveness in decisions about coding (whether or not to assign two similar devices to the same HCPCS codes) and payment (use of least costly alternative payment rates for drugs with similar therapeutic effects). As life sciences companies plan their clinical research programs, it is important that the scientific standards to be used in these decision making processes be explained in as much detail as possible.

Rec 5(a): CMS should accelerate its work on developing guidance documents that clarify the scientific evidence requirements for coverage decisions (both national and local), as well as coding and payment decisions. It would be particularly valuable to provide more detailed guidance regarding the circumstances under which CMS assessment of the benefits and risks of new technologies may differ from the determinations made by the FDA.

Given staff limitations, we would encourage CMS to explore the possibility of obtaining outside technical assistance in developing draft guidance documents, possibly from the academic community and/or from industry.

Rec. 5(b): We would also encourage the Agency to expand its efforts to meet with early stage companies to provide feedback on the Agency's expectations for the design of clinical trials that will satisfy coverage requirements for the technologies they are developing.

Recommendation #6: Clarify standards for new technology add-on payments

The application and review process for new technology add-on payments, particularly the administration of the substantial clinical improvement requirement, is inconsistent and opaque. From FY 2003 through 2006, CMS approved six of eleven unique applications where the agency had to apply judgment about whether the applicant had satisfied the criteria. This figure masks the considerable extent to which the unpredictability of the process has likely deterred many more would-be applicants.

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At present, in order to qualify for an add-on payment, an applicant must demonstrate, in addition to the newness and cost requirements, that the product in question "represents an advance in medical technology that substantially improves, relative to technologies previously available, the diagnosis or treatment of Medicare beneficiaries." CMS has suggested that applicants can satisfy this requirement by showing, for example, that their product "reduces mortality, decreases the number of hospitalizations of physician visits or reduces recovery time." However, CMS has not provided clear guidance with respect to the type of data that applicants must submit to make such a showing, and review of the past applications of this general evidentiary standard by CMS are not consistent enough to provide useful guidance to product developers who may be interested in designing studies to demonstrate that there products do provide a "substantial clinical improvement". Are randomized head-to-head trials against current standard of care required in every case? Review of past decisions indicates that this is sometimes, but not always necessary, and in other cases not by itself sufficient. We would also suggest that technologies that improve outcomes by significantly reducing disability, improving quality of life and improving functional status should be considered to meet the substantial clinical improvement requirement.

Rec 6(a): The NVCA proposes that CMS convene a panel of stakeholders, including researchers, clinicians, industry representatives and patient groups to develop specific, generally applicable criteria for the determination of whether a new product represents a substantial clinical improvement, including the creation of objective standards for the use of external data.

Recommendation #7: Explore options to improve coding processes

At a recent meeting of a half-dozen experienced reimbursement consultants, one of the most commonly cited reimbursement challenges related to obtaining appropriate billing codes in a timely manner. Generally speaking, the problems encountered with HCPCS codes involved difficulties in obtaining unique codes that reflected meaningful differences between a new technology and existing products assigned to the same code.

Problems encountered with CPT codes often related to the poorly-defined process of convincing the relevant specialty societies to support a level one code for a specific new technology. Many examples were offered of inconsistencies in the evidentiary and utilization requirements of different specialty societies for obtaining CPT level I codes, and the significant impact that individual clinical champions or opponents can have in the CPT process.

Problems were also cited in the process of assigning category III codes versus unlisted codes, and the difficulty of obtaining the clinical use necessary to qualify for a category I code when payers will generally deny payment when a category III code is assigned.

In each instance assigned above, the success of the technology in the reimbursement process is influenced by factors unrelated to its clinical value, and is subject to uncertainties that produce no benefit to payers, clinicians, patients or product developers.

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Examples included:

- A modified urinary catheter for which there was reasonably good evidence that complications with infection and strictures were reduced
- A device used in surgical therapy for chronic gastro-esophageal reflux for which it was not possible to obtain CPT level 1 code in a reasonable time frame
- A device approved as a non-surgical alternative to tubal ligation, which only succeeded in obtaining a CPT level 1 code through aggressive intervention by a single passionate and persuasive clinician

Rec 7(a): Within the scope of this initial assessment, we were able to determine that coding problems are a major source of uncertainty and frustration for product developers, and that the degree of friction in resolving these problems was often inconsistent with the potential health benefits of a product. We were not, however, able to identify specific, concrete actions that CMS might take to improve the HCPCS and CPT coding process. We would therefore recommend that a town hall meeting be convened to solicit more detailed testimony on the nature of these problems, and to collect potential solutions. NVCA will be doing additional work in this area that we would plan to present either privately to CMS staff or at a public event.

Recommendation 8: Determine additional resource needs

It is clear that implementation of the procedural and policy improvements described above will require additional staff and administrative resources. Much as FDA improvements were dependent on revenue from user fees, CMS may need to explore creative mechanisms to support these proposed changes. The NVCA would be happy to work with CMS to explore acceptable mechanisms through which adequate resources could be obtained.

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