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EXAMINING IMPLEMENTATION OF THE BIOLOGICS PRICE COMPETITION AND INNOVATION ACT

THURSDAY, FEBRUARY 4, 2016

House of Representatives

Subcommittee on Health

Committee on Energy and Commerce

Washington, D.C.

The subcommittee met, pursuant to call, at 10:30 a.m., in Room 2123 Rayburn House Office Building, Hon. Joe Pitts [chairman of the subcommittee] presiding.

Members present: Representatives Pitts, Barton, Guthrie, Whitfield, Shimkus, Blackburn, Lance, Bilirakis, Long, Ellmers, Bucshon, Brooks, Collins, Green, Schakowsky, Butterfield, Castor, Sarbanes, Matsui, Schrader, Kennedy, Cardenas, and Pallone (ex officio).

Staff present: Leighton Brown, Press Assistant; Rebecca

Card, Assistant Press Secretary; Paul Edattel, Chief Counsel, Health; Carly McWilliams, Professional Staff, Health; Katie Novaria, Professional Staff, Health; James Paluskiewicz, Professional Staff, Health; Graham Pittman, Legislative Clerk, Health; Chris Sarley, Policy Coordinator, Environment and Economy; Jennifer Sherman, Press Secretary; Adrianna Simonelli, Legislative Associate, Health; Heidi Stirrup, Policy Coordinator, Health; John Stone, Counsel, Health; Sophie Trainor, Policy Advisor, Health; Christine Brennan, Minority Press Secretary; Jeff Carroll, Minority Staff Director; Tiffany Guarascio, Minority Deputy Staff Director and Chief Health Advisor; Samantha Satchell, Minority Policy Analyst; Matt Schumacher, Minority Press Assistant; Kimberlee Trzeciak, Minority Health Policy Advisor; Arielle Woronoff, Minority Health Counsel.

Mr. Pitts. The subcommittee will come to order. The chairman recognizes himself for an opening statement. Biologics are used to treat a number of serious diseases and conditions and have improved the lives of millions of Americans. They are produced from living cells using biotechnology and are often significantly more time consuming and resource intensive to consistently manufacture than small molecule chemical drugs. Due in large part to these complexities, biologics tend to be more expensive and why the traditional generic approval pathway is not suited for bringing lower-cost alternatives to market.

In 2009, this committee passed the Biologics Price Competition and Innovation Act, BPCIA, by a vote of 47 to 11. Enacted in 2010, BPCIA established a new abbreviated pathway at FDA for biological products determined to be biosimilar to or potentially interchangeable with a previously approved reference product.

FDA approved the first biosimilar in March 2015. It is convening an advisory committee next week to consider a second application. And while there are close to 60 additional proposed biosimilar products enrolled in FDA's Biosimilar Development Program, the Agency has yet to issue guidance documents on several key policy issues that could have a significant impact on patient safety, prescriber decision making, and market competition.

I look forward to hearing from Dr. Woodcock about where these

documents are in the review process. And I would like to walk away from today's discussion with a better understanding of the Agency's current thinking on issues such as naming, labeling, and interchangeability.

Meanwhile, in preparation for biosimilars coming to market, the Centers for Medicare and Medicaid Services recently issued payment guidance related to Medicare Part B for biosimilars. Members will want to understand the implications of this broad-payment policy and if it will account for variations and differences between biosimilar products and moreover, what might that payment policy mean for the eventual growth in this market and innovation.

With both witnesses here, we will be able to explore how could or should pending issues before FDA, for example, naming and interchangeability, impact the reimbursement policy under the Medicare program as well as access and portability for beneficiaries.

The committee will have an opportunity to hear directly from FDA and CMS on their progress with implementation of BPCIA and future outlook.

I yield the balance of time to Chairman Emeritus, Mr. Barton.

Mr. Barton. Thank you, Mr. Chairman, for holding the hearing. And thank you for holding it at 10:30 where I can actually be on time. I appreciate that.

Six years ago, I co-authored, along with Congresswoman Anna Eshoo, the Biologics Price Competition and Innovation Act, commonly known as BPCIA, which just as an aside you would have thought we would have come up with a better name than something like that.

Today, we sit as a subcommittee with numerous concerns about the implementation or more appropriately the lack thereof of this important piece of legislation. Only one biosimilar has been approved. Numerous products are waiting to proceed through the approval process and many physicians, patients, and concerned individuals like myself are concerned with the lack of progress.

We all agree that it is important for FDA to get it right, but most of us think it is also time for FDA to get on down the road and decide exactly how to proceed with the approval process. There have been seemingly unending delays that are frustrating to legislators, innovators, doctors, and patients. I have sent letters to the FDA, the OMB, CMS, expressing these frustrations. I am concerned that the CMS decision regarding reimbursement for biosimilars to be the average sales price for all biosimilars plus six percent of the Reference Product ASP. This approach undermines the real intent of the legislation.

We want to foster a robust biosimilar market. CMS' approach eliminates any financial incentives in reimbursement for biosimilars by potentially forcing doctors and patients to use

one non-interchangeable biosimilar in place of another based on price alone. This is detrimental because all biosimilars, as we all know, are not equal. By definition, they are not equal. I cannot overstate the importance of treating each biosimilar individually rather than as if they were a generic drug.

I am also concerned about the lack of FDA guidance regarding interchangeability in naming. Due to the absence of any such guidance, the FDA approved a biosimilar, Zarxio, with a placeholder name 6 years after the bipartisan, bicameral BPCIA was signed into law. We are still waiting and this is simply unacceptable.

Thank you, Mr. Chairman.

Mr. Pitts. The chair thanks the gentleman and now recognizes the ranking member on the subcommittee, Mr. Green, for 5 minutes for his opening statement.

Mr. Green. Thank you, Mr. Chairman. Before I start, I would like to have unanimous consent to place in the record a letter from Biosimilar Council and ask unanimous consent.

Mr. Pitts. With no objection, so ordered.

Mr. Green. Thank you and good morning. Dr. Woodcock, welcome again, and Mr. Cavanaugh, thank you for being here.

Today's hearing is the first we have had in the House of Representatives on biosimilars since the passage of the Biologics Price Competition and Innovation Act, or BPCIA, as part of the

Affordable Care Act in 2010. It is particularly timely because the FDA is both developing the standards for approval of biosimilars and reviewing and acting on a growing number of applications for approval. At the same time, CMS recently released its final Physician Pay Schedule, PFS rule, which detailed the Medicare Part B payment methodology for biosimilars. Determinations on biosimilars that are approved, regulated, and reimbursed is critical to the success of this new and emerging market and must be in alignment to facilitate our robust, safe, and competitive marketplace.

As we know, biologics place an important and growing role in our healthcare system. They arguably represent the future of therapeutics and hold immense promise to further transform the way we treat and prevent diseases.

According to the RAND Corporation, world-wide sales of biologics were $46 billion in 2002, representing 11 percent of the global pharmaceutical market. Experts are predicting that by 2017, biologics are expected to grow to between $205 to $235 billion, representing approximately 20 percent of the global pharmaceutical marketplace.

Recognizing a need for non-innovative biologics is analogous to the generic drug market facilitated by Hatch-Waxman. And I worked with then Representative Tammy Baldwin and former representative and now governor, Jay Inslee, years ago to

introduce a bill proposing a pathway for approval of biosimilars, not long after the BPCIA became law, paving a way for injection of competition in the biologics space.

I know we all agree that competition is good for patient safety, consumer choice, and drive savings for consumers and the healthcare system at large. There are a number of outstanding issues on how these will be evaluated and treated by the FDA including naming, interchangeability, labeling, and exploration.

The complexity of these issues are difficult to overstate and I thank FDA for their on-going efforts to develop policies on these questions. However, decisions on these major questions should not be on a case-by-case basis and it is time for the FDA to articulate clear guide rails and principles to industry and the public so that rules of the road are established and understood.

Public and provider trust in the safety of biosimilars is vital to the success of this market. Acceptance of some generics did not happen overnight. Only through a public, transparent process of developing guidelines and rulemaking will the public trust be earned. I look forward to hearing from FDA on the status of these policies and how the Agency is moving these efforts forward.

Recently, CMS detailed how biosimilars would be treated under Medicare Part B and I have serious concerns about the final

rule. While I appreciate the Agency's desire to control costs, I fear that in this instance it would undermine this infant market and create a race to the bottom. If all biosimilars are on the same blended code, we actually disincentivize companies for investing in further trials for additional indications and would drive folks away from this market that we are trying to foster.

Robust competition will ultimately realize the most sustainable, significant savings for the program and the best for patients. This rule seems in conflict with the efforts of the FDA to foster the biosimilars marketplace. I look forward to hearing from CMS on how this determination was made, responses to the concerns about potential undermining of the biosimilars market and I thank you all for being here today. And I yield back the balance of my -- well, does anybody want a minute on our side? No? Okay. I yield back my time, Mr. Chairman.

Mr. Pitts. Thank you. And I would like to ask unanimous consent to submit the following documents for the record: Statements from the Global Healthy Living Foundation and the National Association of Chain Drug Stores. Without objection, so ordered.

The chair now recognizes the vice chair of the full committee, Ms. Blackburn, for 5 minutes for her opening statement.

Ms. Blackburn. Thank you, Mr. Chairman. And I want to say welcome. We are delighted to have you all here. And we do have

some questions about what is transpiring.

And Dr. Woodcock, I want to come to you first. As you have heard from statements on both sides, we realize that this is essential, that the biosimilars are going to fill a place. It is an emerging component, but I want to go to one thing you said in your written testimony. And that is this, "Stakeholder confidence is essential to the success of the biosimilar program." This is something I am going to come back to you on.

When I talk to innovators that are in Tennessee, what they are confused about is the lack of certainty. The chairman mentioned to you about the documents and the lack of guidance, where you all are in the process. So the hearing today is important because of that. These innovators are looking to get some certainty. These are complex decisions. We appreciate that. We know that this is a new class of medicines.

And that brings me to my second point and Mr. Cavanaugh, I will discuss this with you as we move forward with the hearing. Looking at the realization, biosimilars and generics are not the same thing. And we want to be certain that you all are addressing this in the appropriate manner. We appreciate your written testimonies. We look forward to digging down with you on some questions and maybe some things that we are going to request for a written answer.

And Mr. Shimkus, I am going to yield the balance of the time

to you.

Mr. Shimkus. You are so kind. Thank you. Because I want to make sure that we submit for the record, Mr. Chairman, I have two surveys both by -- for the Alliance for Safe Biologic Medicines and one is a physician survey. One is a pharmacist survey. If our goal is to ensure access to these products and to the marketplace, shouldn't we enact a transparent labeling policy that creates confidence in the healthcare market? So if you would share these with the minority and accept these, I would for the record appreciate it. And that is all I have. Thank you.

Mr. Pitts. All right, they will take a look at them and we will come back to that.

The chair now recognizes the ranking member, Mr. Pallone, for 5 minutes for opening statement.

Mr. Pallone. Thank you, Mr. Chairman. I want to thank you for holding this hearing and also thank Dr. Woodcock and Director Cavanaugh for being here to discuss the implementation of the Biologics Price Competition and Innovation Act.

Biosimilars hold enormous potential to offer patients with serious and life-threatening diseases access to more treatment options and potentially lower cost options. And I look forward to hearing your testimony today about how FDA and CMS are working to establish a clear pathway for approval, as well as an appropriate reimbursement structure. These are both critical

elements to ensuring the success of this market.

The use and sale of biologics continues to rise here in the United States and elsewhere. By 2017, sales of biologics are estimated to be between $205 and $235 billion, approximately 20 percent of the global pharmaceutical marketplace. And this is why encouraging and facilitating competition is so critical.

While biosimilars have been available in Europe for some time, Congress did not establish an abbreviated pathway here in the U.S. until the passage of the act as part of the Affordable Care Act in 2010. And I supported the creation of the pathway for biosimilars and for empowering FDA with the authority and resources to ensure that biosimilars are safely available here in the United States for the patients that need them most.

I was pleased when FDA approved the first biosimilar, Zarxio, in March 2015. And this action demonstrated that the approval process is working. But I have also heard that greater clarity is needed from the FDA.

Since 2012, FDA has issued important guidance meant to inform industry sponsors that they consider developing biosimilar products including scientific and quality considerations. Additional guidance is still needed though, particularly in the areas of developing and marketing biosimilars, guidance on interchangeability, labeling, and naming are still outstanding. And FDA's thinking in this area will be vital to companies looking

to enter the biosimilars market.

We have seen how our healthcare system has benefitted from the competition that comes with a robust market. Competition has helped to lower healthcare costs for small- molecule drugs, saving the U.S. health system $254 billion in 2014. And it is my hope that we continue to do all we can to lay the foundation for these types of savings.

Our federal health programs will also play a large role. CMS has the ability, through both Medicare and Medicaid, to encourage this new marketplace and that is why I was concerned that CMS finalize the Part B payment policy for biosimilars last year combining all biosimilars into one average sale price calculation and payment code. I worry that this inappropriately treats biosimilars like generic drugs and will disincentivize manufacturers from entering the biosimilars marketplace because biosimilars are not generics. Each is its own unique product. And biosimilars go through a much more stringent approval process. In fact, Medicare Part D and Medicaid both acknowledge this in their respective programs.

This marketplace is only just emerging with only one approved biosimilars, so it is important that we hear from both FDA and CMS, not only about what they are doing in the space, but how they are coordinating to ensure that the biosimilar marketplace is both safe and robust.

I just wanted to add one thing. I think all of you know that the issue of drug pricing continues to rise in terms of the concerns of the American public. Our Democratic Steering and Policy Committee actually had a hearing on drug pricing. And at that hearing, I was concerned to hear -- I think I asked a question about generics and I was told by the witnesses there that generics increasingly are not a way of reducing prices because of the changes that are occurring in the marketplace. And so I do worry that it is important in the case of biosimilars or generics that that continue to be a way of reducing drug pricing. If it isn't, then we are going to have even more of an outcry that prices are too high and that there should be some kind of intervention by Congress or by the Federal Government in the marketplace.

So I think that this is an issue. Even though we are talking about biosimilars today, Mr. Chairman, this is part of a larger issue of Americans being very concerned about drug pricing. And of course, it has entered into the presidential sweepstakes or whatever, as well. So you know, this is an important hearing, not only in terms of what is happening to biosimilars, but just the larger issue of drug pricing. Thank you, Mr. Chairman.

Mr. Pitts. The chair thanks the gentleman. That concludes the prepared opening statements. As usual, all members' written opening statements will be made a part of the record. Without objection, the two documents that Mr. Shimkus asked to enter in

the record are ordered to be entered into the record.

We have one panel today. On our panel, we have Dr. Janet Woodcock, Director, Center for Drug Evaluation and Research, Food and Drug Administration; and Sean Cavanaugh, Deputy Administrator and Director, Centers for Medicare and Medicaid Services. Thank you very much for coming today. Your written testimony will be made a part of the record. You will each have 5 minutes to summarize your written testimony.

And Dr. Woodcock, you are recognized first for 5 minutes.

STATEMENT OF JANET WOODCOCK, M.D., DIRECTOR, CENTER FOR DRUG EVALUATION AND RESEARCH, FOOD AND DRUG ADMINISTRATION; AND SEAN CAVANAUGH, DEPUTY ADMINISTRATOR AND DIRECTOR OF THE CENTER FOR MEDICARE AT THE CENTERS FOR MEDICARE AND MEDICAID SERVICES

STATEMENT OF JANET WOODCOCK, M.D.

Dr. Woodcock. Thank you, Mr. Chairman and members for allowing me to be here today and testify.

Biological products are used to treat patients who have serious and life threatening medical conditions such as rheumatoid arthritis, cancer, serious gastrointestinal diseases, and so forth. It is important for the health of the public to have access to safe, effective, and affordable biological products. Biosimilars can provide more treatment options to patients and possibly lower treatment costs resulting in better access.

FDA, in general, and I personally have long supported getting the availability of a biosimilar pathway and we were very pleased when Congress enacted this pathway. I have been involved in the developing of biological therapeutics myself for about 30 years and I have seen the transformation they have caused in healthcare in some areas.

I am a rheumatologist and the biologics have totally changed the face of rheumatology. We used to have patients in wheelchairs

lined up in our clinics. And we are talking about joint replacements. We are talking about nursing home care. And now instead of talking about that, we talk about treating to remission, how to make the disease go away, how to make those folks function as if they weren't sick. It has been a miraculous transformation. But this needs to be accessible to all Americans, not inaccessible to some because of cost.

Since the biosimilars pathway was created in 2010, we have actually seen a lot of progress. Now much of this progress is under the hood, so to speak, because the law required that the biosimilar drugs be found biosimilar to a reference U.S. product which meant that they had to study it to the products that were on the U.S. market and it takes an amount of time. But we did prove the first biosimilar in 2015.

We have an advisory committee coming up next week for another biosimilar for the advisory committee to consider and there are almost 60 programs under development in various stages. They have talked to us and we know of perhaps several dozens of others where they have reached out to us, but haven't engaged in our process.

I know people are anxious to see more progress and more certainty. I do understand that about the pathway.

Now if you look at small-molecule generics, as we just said, they have been successful. Over 88 percent of dispensed

prescriptions in the U.S. are generic drugs and actually they are continuing to save a lot of money. There are a small number of products that do not have generic competition still and they may have price increases. But this has been a very successful program, a generic program. But if we compare it, we did not have success overnight in that program. It took a while to establish the parameters, to get the industry to the state they need to be and to get acceptance of the clinical community. So I think maturity of the industry and gaining confidence of the healthcare community was really critical to this 88 percent of dispensed prescriptions being generics.

To earn and to sustain both physician and patients' confidence in biosimilars and interchangeable products, we must apply scientifically rigorous review process and approval standard that people believe in and trust because these products have been life changing for many people and they don't want to sacrifice any performance. And we don't intend that they would sacrifice any performance if they take a biosimilar.

Although the first biosimilar is now marketed, there are a lot of legal, technical, and policy challenges ahead. Some of them you have raised about various policy issues that must be resolved. We fully recognize that and intend to do it, but I will assure you that there is a bright future ahead for our biosimilars program and I believe it is going to provide the same access to

important medications that our current generics program is doing and really a benefit of the health of the public.

I am happy to answer questions.

[The statement of Dr. Woodcock follows:]

**********INSERT 1**********

Mr. Pitts. The chair thanks the gentlelady.

I now recognize Mr. Cavanaugh for 5 minutes for your summary.

STATEMENT OF SEAN CAVANAUGH

Mr. Cavanaugh. Thank you, Mr. Chairman, and members of the subcommittee. I appreciate you inviting me here today to talk about Medicare Part B payment policy for biosimilars.

As you know, the Affordable Care Act created an abbreviated pathway for approval of biosimilars by the FDA and it created a provision for the establishment of Medicare payment policies for these products. Biosimilars hold great promise for all Americas including Medicare beneficiaries and we are committed to policies that will provide fair payment in a healthy marketplace.

In 2014, Medicare Part B spent $21.5 billion on prescription drugs with the top 15 products accounting for $11.5 billion in its total. Eleven of those 15 products were biologics and the top 6 products were all biologics and each one of those contributed over $1 billion in spending.

CMS has an obligation to make sure taxpayers' dollars are used responsibly. This includes creating good payment policy and making appropriate coverage decisions that provide access to innovative services and treatments while incentivizing these treatments and delivery models that are efficient.

When the first biosimilar entered the market last year, we quickly assigned a billing code to facilitate Medicare beneficiaries' access to this new therapy. And we began an

outreach process to the provider community to make sure we could share with them guidance on how to submit claims for the new biosimilar product.

Also as several of you have noted, last year in our annual Physician Fee Schedule rulemaking, we proposed and finalized a policy that promotes fair payment in a healthy marketplace. It was important that we implement a Medicare payment policy for biosimilars now before the second biosimilar for any reference product becomes available to provide certainty for providers and suppliers who will be billing Medicare for these products in the near term.

The statute provides for payment for biosimilar products in the same manner as the statutory methodology for multi-source drugs where more than one drug product is included in the same billing code. We are confident that our interpretation of the law is sound and it represents good policy that will facilitate innovation and competition in the market.

We implemented this new policy through our normal rulemaking process. We solicited, thoroughly reviewed, responded to, and in some cases modified our proposed policy based on comments from the public. For example, in collaboration with our colleagues at the FDA and in response to public comments, we implemented a requirement for claims for biosimilars to include a modifier that identifies the manufacturer of the specific product. We recently

published guidance on the use of this modifier on our website. This will allow us and others to track which specific biosimilars a beneficiary receives.

Overall, the availability of generic drugs in competition with each other and with branded products, has improved price and availability of drugs. Competition among biosimilars can do the same for Medicare beneficiaries. Like multiple-source drugs, CMS sees biosimilars competing for market share with each other as well as competing with the referenced product. Encouraging this competition reflects a top priority at CMS.

The field of biosimilars holds great promise for future improvements in health, value, and outcomes. We believe patients, manufacturers, providers, insurers, and government all share a common goal to foster a healthcare system that leads in innovation, delivers affordable high quality medicines, and results in healthier people. CMS policies will continue to ensure Medicare beneficiaries have access to biosimilars and other innovative treatments.

As more biosimilars are approved, we will monitor developments in the market and consider refinements to our policy as needed based on experience with this new segment of the market. We look forward to continuing to work with this committee, to gathering information from providers, suppliers, and other stakeholders to better inform our guidance and regulations in the

future.

Thank you and I look forward to your questions.

[The statement of Mr. Cavanaugh follows:]

**********INSERT 2**********

Mr. Pitts. The chair thanks the gentleman. Thank you for your opening statements. I will begin the questioning. I recognize myself for 5 minutes for that purpose.

Dr. Woodcock and Mr. Cavanaugh, can you please explain how your two agencies have been coordinating on implementation efforts and discussing policies that could impact each of your agencies' decisions?

Dr. Woodcock.

Dr. Woodcock. Yes. We work very closely together on those matters where our jurisdictions may interact with one another or where they impact. We certainly have had long conversations about the need for safety tracking of these products and I think CMS was very helpful to us in enabling this identifier so that claims data will have some sort of identification so that we can track these products that are paid by Medicare.

We have worked together on numerous activities where needed, but generally, we are on a scientific track and they are taking care of beneficiaries.

Mr. Cavanaugh. Yes, I would add to that that as the FDA has worked through its processes for the naming convention for biosimilars, they have repeatedly kept us in the loop to make sure we understood what was going on, solicited comments. We actually don't take a position on the naming convention, but they have been generous in their consultation.

This really -- and biosimilars, our collaboration really builds on a legacy of work. We collaborate in the regulation of lab safety. We recently implemented a process of parallel review when the FDA is approving products and we can do a coverage decision for Medicare at the same time. So I am sure there are improvements, but there is a history here of working together and a commitment to continuing that.

Mr. Pitts. Has FDA ever sat down with CMS to walk through the many fundamental differences between biosimilars and generic drugs?

Dr. Woodcock. Yes. Yes, members. Medical staff at CMS are very well aware of all these and have been privy to many of our discussions about them.

Mr. Pitts. Dr. Woodcock, FDA has spent several years now grappling with policy decisions on how a biosimilar should be named and labeled in relation to its reference product, particularly if it is approved for different indications and if there are other biosimilars for the same disease or condition. Are you concerned that these nuanced decisions could be undermined by CMS' decision to lump them all together for coverage and reimbursement purposes like they were generic drugs?

Dr. Woodcock. Being able to track for purposes of safety and attributability is different than the payment. So the issue of tracking has been resolved by what CMS has done with the

modifiers. And I am not competent to talk about how reimbursement is arranged.

Mr. Pitts. Mr. Cavanaugh, in its draft naming guidance, FDA seems to make the case that distinguishing biosimilars from their reference product and other biosimilars is critical to patient safety. If this is the case, why did CMS not share this view and take the opportunity to have different billing codes?

Mr. Cavanaugh. As Dr. Woodcock said, they have been very generous in their time helping us make sure we understand completely the clinical and therapeutic distinctions between generics and biosimilars. What CMS has put policy out though is on payment and coding and physicians don't typically look through billing codes in order to understand which product they are ordering. It is a very different process.

So as Dr. Woodcock suggested, and I would agree with, there is really no disagreement here and no conflict in that payment policy which has to be informed by the clinical, but it doesn't have to be entirely reflective of the clinical distinctions.

Mr. Pitts. Well, combining all products under one code inherently removes some incentive for biosimilar companies to develop data on specific indications or seek interchangeability. Did CMS consider these impacts on innovation?

Mr. Cavanaugh. We did. The purpose of our policy was to spur innovation and we believe it will do that. You know, the

fact that there are multiple products under the same billing code in the generic market has not, I think, depleted innovation or competition. In fact, the history of the generic market is robust competition and a lot of products being developed, not immediately as Dr. Woodcock said, but we think it is a sound policy and we think it will spur innovation, not hinder it.

Mr. Pitts. Thank you. The chair now recognizes the gentleman, Mr. Pallone, for 5 minutes for questions.

Mr. Pallone. Thank you, Mr. Chairman. I wanted to ask Dr. Woodcock some questions. FDA has been criticized for not being transparent in the development and implementation of the BPCIA. FDA has also been criticized for not releasing guidances more quickly and not doing enough to educate patients and healthcare professionals. Obviously, these critiques are significant, given that the BPCIA was enacted in 2010. I guess we had an estimate from the Congressional Budget Office of the $7 billion savings in the first 10 years, okay? So a lot is at stake. And of course, I talked earlier about the whole issue of drug pricing, being sort of a national priority right now.

So how much money did Congress appropriate for the program that has the potential to provide so much savings? And will the funding FDA has received from Congress and industry be sufficient to keep up with the growing interest in the development of biosimilars?

Dr. Woodcock. Congress did not appropriate any additional funding for us to do the biosimilar program.

Mr. Pallone. I didn't hear you, Dr. Woodcock.

Dr. Woodcock. Congress did not appropriate any additional funding for the biosimilar program. When the user fee program -- they put in place the ability 2 years later for us to enact a user fee program. When that user fee program was put in place, it was stipulated that we take $20 million out of our existing BA budget and put it into the base. But that was not additional funds. That was the funds we had to take from other activities such as OTC monographs, compliance activities, and so forth.

And of course, when we put the user fee program into place, there really wasn't an existing market unlike the prescription drug user fee.

Mr. Pallone. Right.

Dr. Woodcock. Or the PDUFA or GDUFA, and so we couldn't bill an industry that didn't exist. So we put in place a staggered fee structure for development meetings that we have been enacting and we have collected monies from that to help build the program. But of course, that has been only in the latter parts of the program.

Mr. Pallone. Well, then I guess the larger question which I am trying to get at is to what extent is this funding or lack of funding not working and making it more difficult in terms of

having a backlog of applications, increasing review time lines, and contributing in some way to some of the criticism? And what do you need? What do you suggest we do so that you have enough money?

Dr. Woodcock. We have begun to collect more monies under the user fee program. In FY13, we finally had collected $6 million, so that was the extent of the program. In fiscal year '14, we collected $13 million; and last year, FY15, $23, million, $23.8 million. So we are beginning to build. And as drugs get on the market, biosimilar drugs, we will be able to have a different, perhaps more robust funding for this program. But this program was not funded by appropriations.

Mr. Pallone. Okay, but I guess what I wanted to ask and I don't have a lot of time, specifically, to what extent, because you don't have this money, is that contributing to the backlog, you know, the not releasing guidance, not having enough education, development implementation of the program? And what do you suggest we do in order -- we are getting all these criticisms, and it sounds to me, although you haven't said so, that part of it is a lack of funding?

Dr. Woodcock. Well, you know, what has been as water under the bridge, going forward, we do expect to release drafts or many finals of this guidance in this current year, this coming year. So hopefully, some of these criticisms will be addressed, although

these are controversial issues.

Clearly, had we had more staffing and funding at the get go and we could have set up a program in 2010, that we would have been better off now. However, what I am concerned about is that this program is going to explode, that we are going to have -- we are seeing multiple entries potentially for many of the existing biosimilars. Those top 6 or 11 or whatever. Naturally, there are people who would like to have a part of that market or compete into that market. And I am concerned that we will not have the staff because we are always waiting to catch up.

Mr. Pallone. All right, well, this sounds -- I have got 3 seconds left. Sounds to me like you need some kind of appropriation and having the industry pay a fee is not good enough. But I guess you are not going to tell me you need the appropriation.

Dr. Woodcock. I can't comment on that.

Mr. Pallone. I know you can't. But that is what it sounds like. Thanks.

Mr. Pitts. All right. The gentleman's time has expired. The chair now recognizes the chair emeritus of the full committee, Mr. Barton, 5 minutes for questioning.

Mr. Barton. Thank you, Mr. Chairman, And I wish that Congresswoman Eshoo was a member of the subcommittee because while I know a little bit about this, Anna is really the expert on this issue. She and I are the authors of the bill that got put into

the Affordable Care Act on biosimilars.

It looks to me like we have two issues here. We have an FDA issue, you are right, seeing Ms. Woodcock. We have an FDA issue about how to approve them and then we have a CMS issue on how to charge for them. If you don't get the approval process right, it doesn't matter what you charge for them because there is nothing to be used. But if we can get the approval process right, it doesn't matter unless we get the charging reimbursement process right because if you set a reimbursement process that there is no incentive to create the drug in the first place, the biosimilar, nobody is going to do it.

And on the approval, I would give the FDA a C+, maybe a B-. I think your heart is in the right place. I know you, ma'am, based on my interchanges with you in the past, plus what you said in your opening statement, you want to get it right and you want to get it done.

With regard to CMS, I would give you a D-. The only reason I won't give you an F is because at least you are trying. You have got something out there. I guess to go back to the FDA, we need some labeling guidance.

The first biosimilar, Ms. Woodcock, that the FDA has approved, the labeling is just not as complete as it should be, as transparent as it should be. Could you comment on that? Does your agency plan to address the labeling issue and try to get it

better?

Dr. Woodcock. Yes. We do plan to issue draft guidance. We have many opinions on how the label should be. We have received much input from stakeholders and that is one of the guidances that we would like to get out this year as a draft.

Mr. Barton. So to clarify, you do plan on changing that specific label?

Dr. Woodcock. We will issue a draft and then we need to issue a final and get a policy together. And then the labels will conform, all labels would conform to that. If you recall, the statute that was enacted was considered to be self-implementing, as I understand, without guidance.

Mr. Barton. But you still have to do it.

Dr. Woodcock. Well, we are approving, as they become available, we will approve biosimilars regardless whether we have final guidance out or not. That does create more ambiguity and so perhaps the language in the statute that considered, it was a self-implementing program, was a little optimistic in the sense that --

Mr. Barton. That is true. I will accept that.

Dr. Woodcock. There were a lot of policy issues that we needed much more detailed discussion and settling on to move forward with the robust program and I think that always happens with these type of complex programs. But we do plan to get

labeling. I have been personally involved in many discussions about this. We understand the issues and the positions of the various parties and we will put something out that people can comment on.

Mr. Barton. Okay, now I need to ask Mr. Cavanaugh a question, but before I get off of FDA, can you comment on the interchangeability that basically nothing has happened with regard to interchangeability and what the FDA's plans are? I am talking to the FDA representative.

Dr. Woodcock. Yes. We also plan to put out guidance on interchangeability draft guidance. We have discussed interchangeability in our scientific considerations and our Q&A guidances already that are out there. So there is quite a bit of discussion because companies may need to do the scientific work during their development program. But we plan to put out a specific guidance on interchangeability and we hope to get that out this year as well as a draft.

Mr. Barton. And finally, Mr. Cavanaugh, I apologize for lack of time here, but I understand where CMS is coming from. You want to have a fair pricing scheme, reimbursement scheme, but biosimilars are different than generics. You understand that and your agency understands that?

Mr. Cavanaugh. We do.

Mr. Barton. If you don't allow for some differentiation

since it is more expensive to create, you are not going to create an incentive to do the drug and to do the biosimilar in the first place. Does your agency have any plans to go back and revisit their initial decision on how these are priced?

Mr. Cavanaugh. Thank you for the question. In the regulation we published last year, we did indicate that we would monitor the market closely and that we would do rulemaking in the future. We thought that our payment policy was not as well -- was not accomplishing what we were expecting it to. So there is that possibility.

I want to return to your point though. From a clinical perspective, you are right and CMS knows biosimilars are not the same as generics. However, from a regulatory and market perspective, there are some similarities. These similarities were pointed out in the Senate Committee Report that they are approved in similar processes and that they refer to an existing product's evidence. So there is multiple -- they are going to compete a reference product and against each other. So from a market and a regulatory perspective, there are similarities. From a clinical therapeutic perspective, there are similarities and differences. And we recognize all of that and we think that translates into the payment policy.

We created similarities to how generics are priced, but there are differences as well. We don't have the original reference

product in the same code with the same ASP. So there are differences, but we did think the analogy to generics from a payment perspective was reasonable.

Mr. Barton. My time has expired. I thank the chair for the courtesy.

Mr. Pitts. The chair thanks the gentleman and now recognizes the gentleman from Oregon, Mr. Schrader, for 5 minutes for questions.

Mr. Schrader. Thank you, Mr. Chairman, I appreciate that. I appreciate the witnesses being here.

I am concerned about the market developing. You have testified, both of you, to that effect and it seems to be a nascent market that has yet to be mature and don't want to stifle the competition, don't want -- and you are struggling with payment policy, trying to figure out what is the best way to encourage good competition, hopefully to build the market and ultimately at the end of the day, drive down prices safely for people. It is nice that the ACA allows this opportunity and we are able to get this type of legislation and apparently it has been on a dais, if you will, for a long, long time. So that is good.

Dr. Woodcock, you are not in the payment policy business particularly, but could you talk a little bit about interaction between you and CMS in terms of how to interpret the previous generic policies and since this is not a generic situation, how

you came about working with Mr. Cavanaugh, your contemporary on the other side, to develop the best payment policy for the biosimilars?

Dr. Woodcock. CMS consulted us more on what the biosimilars were medically and clinically rather than how they should be paid for because that is not our expertise. And we interact with their medical staff who have a very clear understanding, I think, of the parameters of how we are analyzing the biosimilarity, what the standards are for biosimilarity and what the standard is going to be for interchangeability and the clinical performance that is expected from both of those. And the fact that in some cases we may not include all the indications in a biosimilar's label that are in the innovator label for various reasons. So they are aware of all of the scientific and clinical parameters in the course of making their decisions.

Mr. Schrader. I guess for Mr. Cavanaugh, you talked a little bit about these modifiers. Could you elaborate? I am concerned by lumping all the biosimilars together that if there is adverse reactions and stuff that can happen to any product, for goodness sakes, how are you going to tease that out? Could you elaborate a little bit?

Mr. Cavanaugh. Certainly, and thank you for the question. This is an issue that we were sensitized to through our conversations with the FDA which is when they are trying to monitor

adverse reactions, they often look in large databases of claims to try to figure out which patients receive which product and then look in future claims or in other databases to see whether they had adverse reactions. And they made us sensitive to if the only indicator of multiple biosimilars was a common number, they would not be able to distinguish between the product which is important here.

So what we did was working with them say well, we will keep the same billing code, but we will have a modifier that is specific to each manufacturer so that when you do query our databases and you see a patient that had an adverse consequence, you will know which manufacturer's product they got. And so that is how we expect it to work and we have confidence that it will work.

Mr. Schrader. Biosimilars are paid for by the Government in several different programs, such as Medicaid Part D and Part B, apparently. Why do we have different methodologies in each of those? I understand some are intrinsic to the programs themselves, but there is enough variation. Biosimilars are treated, frankly, very differently in the other two programs than they are here. And they are kept individual. One biosimilar is not treated the same as another biosimilar. Why have you chosen to do it differently in Part B?

Mr. Cavanaugh. Thank you for that question. The answer is, as you were suggesting, there are different programs, but more

importantly than being different programs, there is different statutes behind each of the programs. I have responsibility for both Medicare Part B, but also Part D.

The statute that created the payment methodology for biosimilars in Part B is Section 1847A, large A. That is very specific to Part B and has nothing to do with Part D and rebates and payment prices there. So I think it all derives from very different statutes. In an ideal world, you would have harmonization across these, but occasionally we find that there are not consistencies across programs and that is not always a bad thing. I think here it created an opportunity for good policy.

Mr. Schrader. I think the policy is being put ahead of the marketplace right now, maybe down the line as you alluded to, but that might be appropriate. But I am very worried that with all the differences that we currently have, it is confusing and makes it difficult for drug manufacturers to step up and try and create these wonderful drugs for our citizens. With that, I will yield back, Mr. Chairman.

Mr. Pitts. The chair thanks the gentleman. I now recognize the gentleman from Kentucky, Mr. Guthrie, for 5 minutes for questions.

Mr. Guthrie. Thank you, Mr. Chairman. Thank you both for coming. I guess my questions are similar to what Mr. Pitts has

said and what Mr. Barton asked Mr. Cavanaugh. And it is the payment policy. We are using a similar policy for generic small molecule drugs are you said for biosimilars. And you said, I think to Mr. Barton, there are similar market characteristics you are looking at and you recognize there are different clinical characteristics that the FDA is concerned with. And that gets to all biosimilars are not tied -- not all biosimilars are tied to the same product or the same reference product.

And so my question is this. It is the concern about the payment policy and the clinical differences that you recognize exist? And we have heard from physicians. We have heard from patient groups, biosimilar manufacturers, a lot of different -- at least one insurer, that this policy could lead to a couple of unintended consequences. One would be inappropriate switching between biosimilars, switching to a lower cost that is not the same, and as well as a less vibrant biosimilar market altogether.

I believe some of these were stakeholder concerns that were raised during the process and wonder why you moved forward and did you consider these warnings?

Mr. Cavanaugh. Thank you for these questions. We did consider these because we received these in the public comment process and we thought very deeply about each of those issues.

First of all, the concern about a less vibrant market. I mentioned some statistics earlier, but in Part B the top six drugs

that we spend money are all biologics. We spend over $1 billion on each of them. I think that alone creates the opportunity for a very vibrant market in biosimilars and I think that is why you are seeing the level of interest that the FDA is seeing in approving products.

As far as inappropriate switching, first and foremost, physicians do not order biologics or other drug products by billing code. And Dr. Woodcock is a physician and can extrapolate on that. And similarly, pharmacists do not derive what switching they are allowed to do based on billing codes. There are other conventions in place. So we fought long and hard about that concern. We talked to our pharmacists. We talked to our physicians. We talked to the FDA and thought that we had heard that publicly that that was not a concern.

Mr. Guthrie. So FDA is not concerned that that could come to pass, those -- the two things I just mentioned could happen?

Dr. Woodcock. Well, again, what we are seeing would be that a biosimilar would be either written by a physician, by the name, right, or it would be switched. If it were interchangeable, it would be switched at the pharmacy level based on our Purple Book where we said it was interchangeable. So those are the processes we see and with the e-prescribing and so forth, there are menus that come up and those have to do with the name of the product.

Now I don't know, I have been out of practice too long to

know how this reimbursement loop which has really gotten very complex recently, how that would impinge. But that is how the ordering would be done.

Mr. Guthrie. Well, thanks. I have another question. So Mr. Cavanaugh, I noticed that you treat biosimilar as a multi-sourced product for the purpose of payment. However, they are treated as a single source for the purpose of the Medicaid rebate. Can you explain the contradiction or apparent contradiction, I guess?

Mr. Cavanaugh. Certainly. It all derives from these being different programs and different statutes that authorize payment. I am on the Medicare side of CMS so I am not as conversant in the Medicaid statute, but as I mentioned earlier, the statute that created the authority for payment for biosimilars is very specific to Part B drugs. And so it by definition would not apply to Medicaid or Part D and so I think any difference is derived from the statutory differences.

Mr. Guthrie. It is kind of a contradiction to have multi-source one way and single the other. Something needs to be corrected or fixed. Maybe it needs to be fixed statutorily?

Mr. Cavanaugh. They are not consistent, but again, the statutory authorities are different.

Mr. Guthrie. Okay. Well, Mr. Chairman, I yield back the balance of my time.

Mr. Pitts. I thank the gentleman. I now recognize the gentlelady from Illinois, Ms. Schakowsky, for 5 minutes for questions.

Ms. Schakowsky. It is really exciting that in recent years that we have seen such breakthroughs in drugs entering the market that creates such hope for treatment or cure for illnesses of millions of Americans. But the cost of these drugs, I want to focus on that because I think it is simply unaffordable for far too many people.

In 2013, the average cost of specialty drugs was over $53,000, an increase of 193 percent from 2005. And this average drug cost is greater than the median U.S. household income, more than double the median income for Medicaid beneficiaries and nearly time and a half -- as much as the average Social Security retirement benefit. And a recent Kaiser poll found that 73 percent of Americans believe the cost of prescription drugs is just simply unreasonable.

And so it is clear that we need additional federal authorities to combat this growing problem and that is why I introduced the Medicare Fair Drug Pricing Act which would require HHS to negotiate the price of biologics and sole-sourced drugs covered by Medicare Part D, one possible solution.

The high drug costs are a problem for both Medicare Part D and Part B. Mr. Cavanaugh, you well know, in 2010 biologics

accounted for $8.3 billion or nearly 43 percent of all Part B drug spending and that number is likely to rise. High-cost biologics are continued to enter the market. I am happy that the biologics are entering the market. Medicare beneficiaries are also struggling to afford the copays that are associated with these drug prices.

In addition to enhancing HHS's ability to control drug costs, we need to ensure that we have a robust marketplace for biosimilars. Several studies estimate the projected savings from the approval of biosimilars for current high-cost biologics to be anywhere from $44 billion to $250 billion over 10 years. We have an opportunity here to expand access to life-saving drugs and lower costs for patients.

Dr. Woodcock, as a rheumatologist, you were talking about the exciting new drugs, but you were also talking about the cost. Are you aware of people who have been actually turned down, in other words, walking away from the pharmacy? I have talked to some pharmacists about people who do walk away.

Dr. Woodcock. Yes, I am aware of it. And I am aware of what my colleagues currently go through now to try to get their patients drugs that are indicated for the condition by FDA.

Ms. Schakowsky. It is a huge concern. Mr. Cavanaugh, how have biologics, I know you talked a bit about that, contributed to the increase in Part B spending on drugs, Medicare Part B?

Mr. Cavanaugh. So Part B, like the rest of the drug world, has been going up faster than the rest of the healthcare economy. It has put a strain on the Medicare program. The biologics in Part B are the vast majority of the spending. I mentioned that the top six drugs in total spending each individually is over $1 billion and they are all biologics.

And so the Agency shares your concern and I think you have expressed the right balance which is as Dr. Woodcock said, therapeutically some of these are terrific products and they change lives and improve lives and we don't want to lose that at all. But we want to balance it with making sure everybody has access and that comes through affordability.

Ms. Schakowsky. I just want to say in some ways I feel like the feelings of an individual that is almost worse and more painful to know that there actually is a treatment or a cure out there that they can't afford and thinking there isn't one. It is right there, I can see it, I can feel it, I know it would help me, but I simply can't afford to get that. And I think that we have to address that problem.

What are the solutions so that Part B, Part D is not going to go bankrupt, that insurance companies will be able to afford to provide the help that people are going to get. What are we going to do? Either one, both.

Mr. Cavanaugh. Again, I just want to reiterate that we share

your concerns. As you may know, last year, Secretary Burwell and Administrator Slavitt convened a listening session. So we have been hearing from patients, from pharmaceutical manufacturers, from pharmacies and others about ideas they have. And we are hopeful that some of those ideas can come to fruition. I don't know that the silver bullet has been discovered yet, but I think there is a conversation going on that could produce something that we could all support and achieve that balance that we are looking for between affordability, but still have access to these live-changing drugs.

Ms. Schakowsky. Well, I know that members want to be part of that conversation, so I appreciate that. I know you recognize the problem and I yield back.

Mr. Pitts. The chair thanks the gentlelady and now recognizes the gentleman from Kentucky, Mr. Whitfield, for 5 minutes for questions.

Mr. Whitfield. Thank you, Mr. Chairman, and I thank both of you for being with us today. We all recognize the important responsibility that you have, both of you.

I am going to touch on something a little bit different. Certainly, it is my understanding that biosimilars are many times more complicated to consistently produce than generic drugs, but a couple of days ago I was reading an article and there have been many articles about drug shortages. And the American Society of

Health System Pharmacists currently lists inadequate supplies of more than 150 drugs and therapeutics for reasons ranging from manufacturing problems to federal safety crackdowns, to drug makers abandoning low profit products. As a result of that, doctors and hospitals are doing rationing and sometimes they make decisions about who gets a drug based on weight, sometimes on the age of the patient. And in this same article, it talks about that in a survey of cancer doctors, 83 percent of them said over the last 6 months that they had had to -- they were unable to provide the preferred chemotherapy agent at least once during the last 6 months and that a third of them said they had to delay treatment and make the difficult choice of which patient they are going to give it to, so rationing these shortages,

So both of you are well respected in your field. Would you just make a brief comment about this shortage problem and whether or not you all are working with manufacturers because biosimilars is even more complicated than generics?

Dr. Woodcock. Clearly, this situation is unacceptable, that people with cancer or others would not be able to access life-saving treatment. We have had a very robust shortage program for many years where we try to anticipate and respond. I think the fundamental problem is the number of drugs that have a single source or perhaps maybe only one competitor.

If you look at this chart, this shows all the drugs, and I

can put this in the record, Mr. Chairman.

Mr. Pitts. Without objection.

Dr. Woodcock. And you can see that for the 99 drugs that we have, there is only one generic competitor. All right? So there is only two on the market.

And then we have another chart here that shows for 125 innovator drugs, they have no patent or exclusivity protection, there is no generics. And this isn't a result of FDA's backlog or anything. Nobody sent in any applications.

So there is a problem in the market that other entrants don't come in and sometimes we get generic entrants and they don't market the drug. And so then if the single manufacturer has a problem or they decide to raise the price greatly, there is no competition there.

Now our generic user fee program is accelerating the approval of generics overall to a 10-month clock for review. So that will help somewhat with new entrants, but in the meantime it may be that no one is interested in entering that market even though it is a critical shortage of a life-saving drug. And I can't tell you why that is. I don't understand those factors, but that is the reality that we are facing.

Mr. Cavanaugh. I would concur with everything that Dr. Woodcock said and just add that if folks believe that there are CMS policies that are contributing to shortages, I would like to

know and I would like to think about what we could do to be part of the solution. The reason many of us came to work at CMS is to help our beneficiaries. And if they are being denied because of market failure, because of policies, we would like to be part of that solution. So I appreciate you raising the issue. It is of concern to me.

Mr. Whitfield. Thank you. I yield back my time.

Mr. Pitts. The chair thanks the gentleman. I now recognize the gentleman, Mr. Butterfield, for 5 minutes for questions.

Mr. Butterfield. Thank you so much, Mr. Chairman. Mr. Chairman, let me first talk about my district and then build out from there. The health disparities that face African-American communities in my district in eastern North Carolina and across the country are absolutely alarming. The FDA knows that. Anyone who watches this, knows it for sure. We could just talk endlessly about the chilling statistics that show black Americans are more susceptible to serious illnesses than another demographic. Serious diseases such as HIV, diabetes, and cancer more frequently occur in African-American communities. Rare diseases like sickle cell anemia occur more often in African Americans. The sooner that affordable, safe, and reliable treatments are discovered, the better we are all going to be.

Like most, I see the potential that biosimilars can have in combatting health disparities. The Affordable Care Act is

helping to make that possibility a reality. And so the Biologics Price Competition and Innovation Act which is part of the ACA has helped set up a framework to enable the development of new biosimilar drugs. Since 2010, the FDA has worked diligently to implement the program. Biosimilars are complex and we have heard that testimony today. And I agree with Dr. Woodcock that their regulation must be bullet proof. It is critical, therefore, that the public can depend on approved biosimilars and that we encourage the development of new treatments. And so it is my hope that the creation of new biosimilars can make safe treatments more affordable for those in need. I thank both of the witnesses for their testimony today.

Dr. Woodcock, it is my understanding that current FDA guidance allows biosimilar applicants to extrapolate efficacy information based on the reference product. I don't fully understand that, but I am sure that you do. If you would expand on that, please.

Dr. Woodcock. Well, basically, the biosimilar pathway itself is an extrapolation, all right? What is done is it is an abbreviated pathway. So if you can show your product is biosimilar through various means that we have established to the reference product, then you may be able to, depending on what you have shown, have a label that looks exactly like the innovator label. Or you may only have some of the indications, depending

on what you have shown.

So there are two kinds of extrapolations people are talking about. One is the basic abbreviated pathway which means we find the evidence that is submitted by the biosimilar and we say yes, this means that you have the same properties as an innovator drug. And then many of the brand drugs have multiple indications. For example, next week it would be ulcerative colitis, Crohn's Disease and rheumatoid arthritis, all right? Those are different diseases. And so what many people are talking about is extrapolation across from one disease to another.

What amount of data that we need to grant all those indications is a scientific matter and may be of some dispute, obviously, because there is a lot at stake there. But I will tell you, we are not going to approve biosimilar drugs that we don't think have the same performance as the innovator. That is what we are going to do. If a patient is started on a biosimilar, they should expect the same results as if they had started on a brand drug.

Mr. Butterfield. Now there are a lot of stakeholders and a lot of people have an interest in this subject. I am beginning to appreciate that and I see the room full today and I am sure there are a lot of people here who are listening very carefully.

Are there concerns that stakeholder groups have? And are there any concerns that have been shared about extrapolating

clinical safety and efficacy data for biosimilars which treat special populations including children or certain fields including rheumatology?

Dr. Woodcock. Certainly. People have concerns still about generics and there are certain groups such as the neurology community still isn't convinced they should do generic substitution. Recently, we have sponsored studies to show that there is no outcome difference between a generic and an innovator drug for seizures. So there is going to be concern and there is going to be ongoing concern regardless of what we do. But right now we do extrapolate often for regular drugs, whatever biologics for the brand drug. We may extrapolate to children based on dosing information if the disease is similar enough, rather than subjecting children to randomized clinical trials. If we have enough scientific data, we will extrapolate after finding out what the right dose with the equivalent doses are in children of different ages.

Mr. Butterfield. Thank you. I yield back, Mr. Chairman.

Mr. Pitts. The chair thanks the gentleman. I now recognize the gentleman from Illinois, Mr. Shimkus, for 5 minutes for questioning.

Mr. Shimkus. Thank you, Mr. Chairman. I know we are getting close to calling of votes, but I want to first of all address Mr. Cavanaugh.

I appreciate your answers, but I don't think they are totally accurate because I think under the law and especially the report language of the bill passed, the report language and the congressional intent was that there would be separate billing codes for reimbursement at the ASP of biosimilars. So I mean your comment saying I can't do this because of statutory intent we feel is inaccurate. So I am going to move most of my comments to there and I hope you would take that back because we think you do have the authority to do that.

And the other thing just as a comment, listening to testimony, biosimilars, the efficacy and the ability, the cost benefit, the return on investment, Dr. Woodcock, as you mentioned earlier as a rheumatologist and wellness versus treatment, there is a great return on that investment that somehow has to be put into this pricing decision, right?

But I want to get to a couple of questions in this whole process.

Let me start, Dr. Woodcock. I understand the FDA has not provided details on specifics of interchangeable products. Is it possible that FDA might approve an interchangeable product without first issuing guidance or interchangeability?

Dr. Woodcock. Yes, it is. In an interchange with Mr. Barton, the statute allows us to execute the statute without guidance is my understanding.

Mr. Shimkus. Under current law, a new biologic product can

be brought to market either by being approved as a new drug or being licensed a biological product. How, if at all, does a manufacturer's decision to use one pathway or another affect pre-market review of a product?

Dr. Woodcock. The body of evidence that is submitted for a biological license application, a standalone, right, is different than the body of evidence that is submitted for a biosimilar.

Mr. Shimkus. So how?

Dr. Woodcock. Okay, a biological --

Mr. Shimkus. So what is the answer?

Dr. Woodcock. I am sorry. A biological product, stand alone, must demonstrate free-standing safety and efficacy of that product. A biosimilar must demonstrate biosimilarity to a reference listed, already approved biological product. Those are conceptually, fundamentally, two different things.

Mr. Shimkus. So really the question is if they choose one pathway or the other -- I mean right now, how are they making the decision which pathway to choose or how can they?

Dr. Woodcock. We have around --

Mr. Shimkus. Let me just go to the next question because they are all kind of in line. What about the post-market obligations if they choose one pathway or another?

Dr. Woodcock. It is unlikely a biosimilar product would not

have additional questions that would be post-market commitments, but they have the same safety surveillance requirements as other marketed products.

Mr. Shimkus. Let me just go to another one then and I will tell you why I am asking these specific questions. Does the FDA consider the Purple Book to be part of a biological product's labeling?

Dr. Woodcock. Does it consider what?

Mr. Shimkus. Do you consider the Purple Book to be a part of the biological products labeling?

Dr. Woodcock. No.

Mr. Shimkus. Okay, so these are all questions asked by U.S. Senators in your testimony in November which they still have as they have asked me to restate these questions. Do you know why? Because you all haven't responded to their questions in writing.

Dr. Woodcock. Well, okay. Well, we will certainly do that.

Mr. Shimkus. And the point being is there is great confusion out there in the healthcare sector on how we are going to move forward. And this hearing is going to have follow-up questions and they just need to be answered. And so again, all these are follow-up, and there was a lot more. I have got four or five pages of them from bipartisan questions that need to be addressed so we can help -- that is actually the same questions that you are being asked by the stakeholders. And so I would ask you to respond

timely to the questions posed by my colleagues. And I yield back my time, Mr. Chairman.

Mr. Pitts. The chair thanks the gentleman. I now recognize the gentleman, Mr. Cardenas, for 5 minutes for questions.

Mr. Cardenas. Thank you very much, Mr. Chairman. Thank you, Dr. Woodcock, and Deputy Administrator Cavanaugh for joining us today.

Dr. Woodcock, under BPCIA, interchangeable biologic products must demonstrate that they can be expected to produce the same clinical result as the reference product in any given patient. A biosimilar determined to be interchangeable can be substituted. So therefore my question is will you discuss generally the types of questions the Agency is thinking through as they are considering guidance to industry in this space?

Dr. Woodcock. Certainly. This issue was before us even before the statute was passed which is one of the differences of biologics for most generics is something called immunogenicity. In other words, the ability to stimulate an immune response in a patient, a reaction. And this can be insignificant or it can cause serious problems. And one of the concerns is under a scenario of interchangeability, what we see in the generics world people get all different kind of products, generics, they switch at the pharmacy based on their plan. And the concern is if you are switching back and forth could you set up an immune response

that would be negative to the person? And how you have to address that concern for interchangeability is contingent on a number of factors. A number of these drugs are not -- biologic drugs -- are not that immunogenic. And some of them they are, but the consequences have not been severe. Others, the consequences could be catastrophic. So we take all those factors in in talking to companies about how much evidence they have to show that switching back, people back and forth, wouldn't cause terrible harm.

And the problem we have is the science isn't far enough advanced for us to predict this from say test tube experiments. We have to look in people because we don't understand the immune system well enough. So there has to be some human testing of switching ordinarily to give confidence that, in fact, if you switch the people back and forth, they are not going to have some unprecedented problem.

Mr. Cardenas. Thank you. This question is to Deputy Administrator Cavanaugh. I would like to reiterate the concerns voiced by my colleagues on both sides of the aisle and the final rule for the position fee schedule which places biosimilars that reference the same biologic into single HCPCS code. Biosimilars are not generic drugs because they are not the same copies of one another and I appreciate how hard you and your colleagues at CMS are working to create a robust biosimilars market.

However, because biosimilars are so complex, I fear that this may discourage manufacturers from entering the biosimilars market. How do you feel about those dynamics?

Mr. Cavanaugh. So thank you for the question. I think it is important to recognize a couple of things. As I said, the important therapeutic differences which the FDA has educated us about and we recognize them, but there are also regulatory and marketplace similarities. And in fact, the congressional history here shows that the approval processes were mirrored or paralleled off of generic approval processes. We expect them to work in the marketplace much like generics in that there will be a low-cost alternative to a higher cost innovative product. And for that reason and also for statutory reasons, the statute said that for products where there is multiple products under the same billing code, they should have the ASP average.

I want to emphasize why we think this is a successful policy. One, to Congress' credit from the physician perspective when they are ordering the drug, Congress created a payment where they are still getting the six percent markup from the innovative product. So the physician is not invested in a higher cost product. They are made whole either way and I think that was a terrific policy that Congress made.

But secondly, they are going to choose the therapeutically successful product at a fair price and we will have these

biosimilar products competing on price with each other. So the last thing I would say is even though they are using a methodology similar to generics, that doesn't mean the resulting price will be similar to generics. The biosimilar companies set their own average sales price. So they can set it at a market-bearing price that results their costs whether it is research or other input costs. So I don't think the fact that they are the same methodology means you will end up with the same price.

Mr. Cardenas. Thank you. With my 5 seconds I just want to remind the American public that what we are talking about here cannot be put in one page, so I want to thank my colleagues and also the process for understanding that laws aren't necessarily one or two paragraphs. These are very important issues and protection of the public is very important to our country.

Thank you so much, Mr. Chairman.

Mr. Pitts. The chair thanks the gentleman. We are voting on the floor, so we will continue as long as we can. The chair recognizes Mr. Collins for 5 minutes for questions.

Mr. Collins. I want to thank the chairman and I know we are now trying to hold the votes down so thank you for letting us cut into this just a bit. I thank both the witnesses, Dr. Woodcock, Mr. Cavanaugh, for coming.

I think what I have heard is some 30,000 foot Cliff Notes discussions on biosimilars, generics, biologics, etcetera.

And Dr. Woodcock, as you are sitting there, I know you must have some level of frustration because we are under time constraints to get the point out. And so in my time, I think we have pretty much established that generic is for all practical purposes, 99.999 percent the same as the brand name. It is a compound. It is usually a pill. It is the same and therefore doctors prescribe that.

But Dr. Woodcock, in what testimony you have given, in thinking about the differences in a biologics and a small molecule which are two different worlds and a biosimilar which will never, ever, ever be the same, and while you have explained and I think it is true, the FDA's emphasis is safety and efficacy and I guess you would say safety, safety, safety, safety, safety. And we have got to know it works as well. But safety is always first.

In 2 or 3 minutes, for the purpose of this committee educationally, what would you like to add as far as how the FDA is looking at and even maybe explain to the committee the basic difference in why a biosimilar will never be a generic and even the manufacturing process of a biologic, the sterility process or give us 2 or 3 minutes of education which I think would be helpful to this committee having heard already some of the questions.

Dr. Woodcock. Well, the biologics are very large molecules. In other words, it would be like maybe a little house compared

to the Empire State Building as far as the comparative size of a regular drug to a biologic. And if you just think about the brand biologics, most of them they are not exactly the same from batch to batch. I know that is very disappointing, but they are made by cells, usually.

Mr. Collins. The manufacturing process is unique.

Dr. Woodcock. Yes, they are biosynthesized by cells and those cells are subject to conditions we don't understand very well fully or can't fully control. And so they are slightly different from batch to batch to batch. Our job in regulating them is to make sure that those variabilities in the brand product, I am still talking about, don't affect safety, safety, safety or effectiveness, okay? So that they stay in a band where from the clinic, if you are a doctor treating patients, it doesn't make any different. But it isn't like the small molecule where every tablet we know exactly what is in there.

Mr. Collins. And to interrupt, isn't it also true that in the intellectual property protection, etcetera, a manufacturer is not required to tell the world exactly how they are making it?

Dr. Woodcock. Never, and that is true with the generics as well.

Mr. Collins. And that is what I think some people miss. You have to put enough information in your intellectual property to say I can replicate it, but you don't -- a manufacturer is going

to protect his price point.

Dr. Woodcock. Well, it is considered trade secret.

Mr. Collins. Right, and it will always be trade secret. So when a generic or a biosimilar comes out, they don't know the 16 steps that the brand name is doing to make that product.

Dr. Woodcock. Right.

Mr. Collins. And hence, in the case of a biosimilar, it can never be the same.

Dr. Woodcock. What they do and what we found, okay, is for the generics does this too. They have to buy the reference product on the market and reverse engineer it and then develop a process of their own that will replicate that avoiding any patents that might be preexisting that may be on process and so forth.

Mr. Collins. And it is just like reverse engineering anything. You don't know and don't have the prints of the original company to know the tolerances of every item and how they interact.

Dr. Woodcock. That is correct.

Mr. Collins. So I think in defense of the FDA and what they are doing in the safety, safety, safety, but also looking at efficacy, the folks making a biosimilar don't have the prints. They don't have the process.

Dr. Woodcock. That is correct.

Mr. Collins. They are trying to reverse engineer it and yet the FDA has to make sure in reverse engineering it safety is not compromised, efficacy is there. And that is why it is -- you have got a very, very, very difficult job in bringing biosimilars to market knowing that the person making it doesn't have the benefit of the drawings, the process. They are guessing. They are reverse engineering. They are hoping they get it right and you have to make sure at the end of the day it is safe and the efficacy is there.

Dr. Woodcock. And also what we found to your point, it is a very good point, what we found as we work with these companies they are going to have to get more lots of the reference drug because generics usually --

Mr. Collins. The variation.

Dr. Woodcock. Because they have to be within the variation. If you just pick a small sample, it might be outliers.

Mr. Collins. My time is up. I thank you for that. I think the education piece is very valuable.

Mr. Pitts. Thank you. I am sorry to rush you. We have 8 minutes left. So we are going to have to try to hurry as much as possible. Dr. Bucshon, you are recognized for 5 minutes.

Mr. Bucshon. Yes, Mr. Chairman, I will be brief and just make a comment and maybe a brief question. I am glad that you are both here. Thank you very much for your work. That

said, Mr. Cavanaugh, this is kind of directed at CMS. It appears to me, as a physician and I was a practicing cardiovascular surgeon, that CMS has become the rate-limiting step in getting innovative products to the marketplace and to patients. A brief example, a treatment for glioblastoma for brain tumors, for example; a bionic prosthetic to benefit veterans and others. And it also seems to me that frequently on reimbursement decisions that comments and recommendations of experts outside of CMS have been ignored. And unfortunately in my view, decisions are being based on financial reasons, not based on medical benefit of the products.

I am talking about products that have been approved by the FDA and other organizations around the world and subsequently are also reimbursed frequently by the private sector insurers and then are not reimbursed by CMS or it has been dragged out so long that some of the innovative companies have almost gone bankrupt because CMS hasn't approved their payment.

So the question I have is is it the role of CMS or the FDA to determine the safety and efficacy of medical products including biosimilars?

Mr. Cavanaugh. To me, sir?

Mr. Bucshon. Yes.

Mr. Cavanaugh. No. It is the role of the FDA to determine that.

Mr. Bucshon. Okay, so once these are proven to be safe and show efficacy for patients, why is frequently CMS dragging its feet on reimbursing it? What is the reason?

Mr. Cavanaugh. Is the question specific to biosimilars or broadly?

Mr. Bucshon. Broadly.

Mr. Cavanaugh. First of all, as I said, on the first biosimilar, we acted very quickly and in fact, we had a billing code and coverage guidance before it was actually even marketed. You have raised other examples though that are broader than biosimilars.

Mr. Bucshon. I was just throwing those out there. Let me just tell you as a physician, many, many people talk to me about medical issues, right, because I was a practicing physician. And it is not just -- I mean I have literally heard from hundreds of people frustrated with CMS because there have been -- and these are coming from patients, from physicians, from companies, everywhere, telling me that these products are approved by the FDA. They are frequently reimbursed by the private sector and that CMS has either decided to not reimburse them or dragged their feet or put up a price that is not competitive for the production and maintenance of a company or the product to be actually on the market place at all. And it is very frustrating for me as a physician to know that there are products out there to benefit

patients.

And I know you have a tough job. I am just on my soap box a little bit here. But I just don't get it because if things are approved by the FDA, they are frequently approved by the same organizations in the European Union and around the world, and these patients are not available to people in the United States because not that they are not proven to be effective and safe, but Medicare hasn't decided how they are going to pay for it.

And so if you are not making decision on safety and efficacy, how can you decide not to pay for it?

Mr. Cavanaugh. Sure. I think it is a terrific question and allows me to talk about the Medicare process. FDA approves safety and efficacy. When it comes to the Medicare, there are two standards they need to meet, any product or service. One is, it has to meet one of the statutorily-defined benefit categories. So the statute has to say this is something -- this falls into a category that applies to Medicare coverage.

Mr. Bucshon. I am going to briefly interrupt you. That has already been determined by the FDA. There has been clinical studies that they have gotten that have shown efficacy, that have shown benefit to patients. So that seems like reinventing the wheel to me.

Mr. Cavanaugh. With all due respect, the benefit category is not about safety and efficacy. The Medicare statute specifies

the covered services and benefits. And what I am saying is no matter how safe and effective FDA finds it, it can only come into Medicare if it meets the statutory definition of something that Medicare covers. And after it makes it past that criteria, the secondary criteria is it reasonable and necessary for --

Mr. Bucshon. Understood. Since I only have 10 seconds, I just want to say that, in my view, every product that is approved by the FDA should be available to America's seniors and the limiting factor should not be the ability of CMS to stonewall and not pay for it. And I am just telling you it is a very frustrating situation. I yield back.

Mr. Pitts. The chair thanks the gentleman. There are 3 minutes left on the floor clock. The chair recognizes Ms. Ellmers for questioning.

Ms. Ellmers. Thank you, Mr. Chairman, and I will be very brief. I want to thank the panel. Thank you, Dr. Woodcock, for being here again, and Mr. Cavanaugh.

I actually just have two letters that I would like to submit and ask unanimous consent to do so.

Mr. Pitts. Without objection, so ordered.

Ms. Ellmers. One is actually from our Doctors Caucus. Twelve members of the Doctors Caucus submitted a letter on December 21st to the acting Secretary, excuse me, Commissioner Ostroff. And we have not yet received a response to those

questions.

And so Dr. Woodcock, we will be submitting questions for you and I know that this is an interim, but we would look forward to some of the answers. You both have answered many of the questions that we have already had.

Now the other letter that we have that I would like to submit under unanimous consent is from the Coalition of State Rheumatology Organizations. This is a letter that I would like to submit.

Mr. Pitts. Without objection, so ordered.

Ms. Ellmers. Thank you very much. And again, thank you to our panel. This is a very, very important issue to all of us and I know that we all agree that we need to get to the bottom of this so that we can help those folks out there that need the help. Thank you.

Mr. Pitts. The chair thanks the gentlelady. That concludes the questions that we have here. We have follow-up questions. We will submit those to you in writing. We ask that you please respond promptly. Members have 10 business days to submit questions for the record, so members should submit their questions by the close of business on Thursday, February 18th.

A very important hearing, thank you very much for your testimony today. Without objection, the subcommittee is adjourned.

[Whereupon, at 12:02 p.m., the subcommittee was adjourned.]