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LEGISLATIVE HEARING ON 21ST CENTURY CURES

THURSDAY, APRIL 30, 2015

House of Representatives,

Subcommittee on Health

Committee on Energy and Commerce

Washington, D.C.

The Subcommittee met, pursuant to call, at 10:00 a.m., in Room 2123 of the Rayburn House Office Building, Hon. Joseph R. Pitts [Chairman of the Subcommittee] presiding.

Members present: Representatives Pitts, Guthrie, Barton, Shimkus, Murphy, Burgess, Blackburn, McMorris Rodgers, Lance, Griffith, Bilirakis, Long, Ellmers, Bucshon, Brooks, Collins, Upton (ex officio), Green, Engel, Capps, Schakowsky, Butterfield, Castor, Sarbanes, Matsui, Lujan, Schrader, Kennedy, Cardenas, and Pallone (ex officio).

Also present: Representative DeGette.

Staff present: Clay Alspach, Chief Counsel, Health; Gary Andres, Staff Director; Sean Bonyun, Communications Director; Leighton Brown, Press Assistant; Noelle Clemente, Press Secretary; Paul Edattel, Professional Staff Member, Health; Gene Fullano, Detailee, Telecom; Robert Horne, Professional Staff Member, Health; Carly McWilliams, Professional Staff Member, Health; Katie Novaria, Professional Staff Member, Health; Tim Pataki, Professional Staff Member; Graham Pittman, Legislative Clerk; Krista Rosenthall, Counsel to Chairman Emeritus; Chris Sarley, Policy Coordinator, Environment and Economy; Adrianna Simonelli, Legislative Associate, Health; Heidi Stirrup, Health Policy Coordinator; John Stone, Counsel, Health; Traci Vitek, Detailee, HHS, Health; Ziky Ababiya, Democratic Policy Analyst; Jeff Carroll, Democratic Staff Director; Eric Flamm, Democratic FDA Detailee; Waverly Gordon, Democratic Professional Staff Member; Tiffany Guarascio, Democratic Deputy Staff Director and Chief Health Advisor; and Kimberlee Trzeciak, Democratic Health Policy Advisor.

Mr. {Pitts.} The Health Subcommittee will come to order.

The chair will recognize himself for an opening statement.

One year ago today, April 30, 2014, the Energy and Commerce Committee embarked on an ambitious, bipartisan goal to develop legislation that would bring the medical innovation cycle of discovery, development, and delivery into the 21st century, and speed better treatments and, hopefully, more cures to patients who desperately need them. Since then, this subcommittee has held over a dozen hearings and roundtables to educate members on topics ranging from modernizing clinical trials, to personalized medicine, to digital health care, to incorporating patient perspective into the development and regulatory decision-making process. We heard from government, academia, patients, providers, manufacturers, and stakeholders from across the spectrum. The consensus was clear. We can and must do more to help patients in need and to maintain our Nation's role as the biomedical innovation capital of the world.

Informed by the continued outpouring of feedback and constructive criticism from stakeholders across the spectrum, we have worked tirelessly on a bipartisan basis to develop

the second discussion draft that was released yesterday. While it remains a work in progress, it is the product of good-faith negotiations and a significant step forward in this process. While increasing accountability, this legislation would invest in the basic research so critical to equipping our Nation's best and brightest with the tools they need to discover the underpinnings of disease; it would streamline the development of new therapies and technologies which have--has become increasingly challenging and resource intensive; and it would foster a dynamic, continuously learning health care delivery system. Work continues on several complicated, yet critical issues, including the regulation of diagnostic tests and telemedicine.

With respect to diagnostics, we remain absolutely committed to developing a modernized regulatory framework for these innovative and increasingly important tests and services. Understanding this is a particularly unique and complex endeavor. We look forward to working in a deliberative manner over the coming weeks with Dr. Shuren and stakeholders to advance legislation.

On telemedicine, I continue to work with my colleagues in the Energy and Commerce Working Group on Telemedicine towards a bipartisan proposal that will encourage the use of telemedicine services to improve health care quality and

outcomes, increase patient access, and control costs.

I want to thank the Administration and CBO for their input, and look forward to our continued collaboration moving forward. On that note, I would like to specifically thank our 3 witnesses today for their assistance throughout this process and their testimony today.

And I yield 1 minute to Dr. Burgess at this time.

[The prepared statement of Mr. Pitts follows:]

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Mr. {Burgess.} Thank you, Mr. Chairman. I do want to thank you for holding the hearing today.

A lot of bold goals in the 21st Century Cures, but at the end of the day, it is all about patients. Doctors, of course, want to heal, and the good news is I really do feel like we are entering into a golden age of medicine. I think that the doctors who are in medical school today will have tools at their disposal to alleviate human suffering that no generation of doctors has ever known. And it is the work of this subcommittee that is bringing that possible.

I do have a number of proposals in the newly released draft, and I look forward to discussing those proposals with our agencies today. All of these things can be helpful in speeding the development of new therapies, and getting the needed information into the hands of health professionals.

I do want to highlight, since 2009, we have spent $28 billion to drive adoption of electronic health records, yet patient health data continues to be fragmented and difficult to access for health care providers and for patients themselves. So I am glad to have the Chairman's continue support in this area.

I yield the balance of the time to the vice chairman of the full committee, Mrs. Blackburn.

[The prepared statement of Mr. Burgess follows:]

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Mrs. {Blackburn.} Thank you. And I think we are also pleased to see this legislation coming forward and to discuss it with you.

One of the purposes is to spur innovation and to look for cures, to help individuals with disease management, and to focus on those outcomes.

Kind of shift the focus of where we are going a little bit. I think of it as our moonshot. President Kennedy didn't say we are going to go increase NASA's budget and go to the moon, he said we are going to the moon. And that indeed he did. So this is where we are aiming; to increase these cures and opportunities.

And I thank you for your time, and I yield back.

[The prepared statement of Mrs. Blackburn follows:]

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Mr. {Pitts.} Chair thanks the gentlelady.

Now recognize the ranking member of the subcommittee, Mr. Green, 5 minutes for an opening statement.

Mr. {Green.} Thank you, Mr. Chairman. And thank all our colleagues for being here today.

I want to particularly thank our witnesses and their colleagues for their expertise for the countless hours of work they put in to help us in this effort. It has been one year since the 21st Century Cures Initiative was launched by our colleagues, Chairman Upton and Congresswoman DeGette. Yesterday's release of the discussion draft marked a continued progress toward boosting research and delivering hope to patients. FDA-approved treatments are the global gold standard for safety and effectiveness. It is what physicians, patients, and families trust when making decisions about their health.

Recently, Congress has enacted additional tools like breakthrough designation for drugs to facilitate development and effective innovation--innovative treatments. The NIH, the world's leading research institutions is one of the great success stories of the Federal Government. Our investment in basic and translational research has led to advances that have profoundly improved the health and quality of the lives

of millions of Americans.

The 21st Century Cures Initiative nobly asked for what more can Congress do to further the public and private efforts to address today's most difficult science--scientific challenges and advance our health care system. Additional funding for NIH is tantamount to this effort. It is so important that the initiatives include increased funding for NIH, both through reauthorization and $10 billion over 5 years in mandatory funding. On the regulatory side, the draft includes policies to incorporate the patient perspective in development process, facilitate the use of biomarkers, break down barriers to collaboration and data sharing. The draft also includes provisions to modernize clinical trials.

I want to particularly highlight the ADAPT Act, which Congress and Shimkus and I are working on to provide a streamline approval and pathway for the next generation of antibiotics. FDA and Dr. Woodcock, in particular, has been an incredible partner on this issue. I want to thank the agency for their continued commitment in the global crisis of antibiotic resistance. We are working hard to include feedback, and will have a new draft of the ADAPT to share in a few days. The draft also includes a new version of the Software Act, which I have been working with Congresswoman

Blackburn for a couple of Congresses. This provision will provide clarity for developers of software products used in health management and care. Dr. Shuren and his colleagues at the FDA have been instrumental to this effort, and I look forward to continuing to work with you to foster innovation, provide regulatory certainty, and promote patient safety. The draft recognizes the importance of improving the interoperability health of IT systems, interoperability and fundamental in realizing the goals of the 21st Century Cures Initiative, and our interoperable healthcare system can advance and facilitate research, and dramatically improve patient care and safety.

I thank my colleagues for their commitment. The draft--the Cures draft is a work in progress. There is a lot of work left to do, but we will continue to move forward and iron out policies that advance our healthcare system, and live up to the goals of the 21st Century Cures Initiative.

And again, I want to thank our witnesses. And I would like to yield the remainder of my time to Congresswoman DeGette.

[The prepared statement of Mr. Green follows:]

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Ms. {DeGette.} Thank you so much.

In the year since Chairman Upton and I announced this 21st Century Cures effort, I have constantly been impressed by the engagement and consensus of people across the healthcare landscape. From the beginning, we sought suggestions from everyone, and we have worked diligently to reflect those ideas in the discussion draft we have before us. I also want to add my heartfelt thanks to everybody, both in this room and across the country, who have helped Chairman Upton and myself, and all of the members of this committee, work to deliver treatments and cures for patients.

The draft makes important improvements to our biomedical research system, and our process for assessing and improving new therapies, drugs, and devices for patients. After years of resource erosion and cuts, we deliver important new resources to the National Institutes of Health. We placed the patient perspective at the heart of the FDA's drug approval process. We will develop disease registries to pull information, and help researchers drill into the unique and sometimes subtle needs of patient populations. We will help new scientists begin their careers in research so that our great minds tackle great biomedical challenges. Any of these ideas would be worth doing on their own but, frankly, this

committee's ambitions stretch across the century, and so we want to do everything we can to improve the process of discovering, developing, and delivering new biomedical advances.

So in that spirit, as you can see, we have a great deal more work to do. This discussion draft has brackets around many sections of text, and we have many--much more work to do, but it is certainly not through lack of trying on all of our parts over the last year. One specific issue that deserves singling out is the fact that we are asking FDA to make many changes to its current operation. We need to make sure that the agency has the resources to carry out these duties.

Mr. Chairman, I want to thank you, I want to thank Chairman Upton, and I want to just reflect back to the time when we made that kind of hokey video launching this effort, but we have made tremendous progress. We have a lot more to do, and in that spirit, I want to thank you, Mr.--Chairman Upton, Chairman Pitts, Mr. Pallone, Mr. Green, all of the staff. It has really been a great effort, and I look forward to moving along this road so that we can actualize this important, important piece of legislation. Thank you.

[The prepared statement of Ms. DeGette follows:]

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Mr. {Pitts.} The chair thanks the gentlelady.

And now recognizes the distinguished chairman of the full committee, Mr. Upton, 5 minutes for an opening statement.

The {Chairman.} Well, thank you, Mr. Chairman.

First, I want to talk a little bit about how we got here today. These two little girls, my friends, Brooke and Brielle, of Mattawan, Michigan, served as an inspiration for the 21st Century Cures. They are battling SMA, and they are two of the brightest stars that I know. Their motto is, we can and we will.

At our very first 21st Century Cures roundtable last spring, I commented that I think that we can all agree that we can always be doing more to help biomedical innovation. We have come a long way, yes we have, but those words still hold true. In fact, since our launch a year ago today, we have heard from our colleagues in the Senate, and yes, they are interested in these same goals, and President Obama even included Precision Medicine as part of his State of the Union Address in January. There is clearly an opportunity to make a real difference. And we--all of us here have traveled the country to listen to as many stakeholders as we could to get more knowledge to make this bill as solid as we can.

At that first roundtable in this room last year, we asked what steps can Congress take to accelerate the discovery development delivery cycle in the U.S. to foster innovation, bring new treatments and cures to patients, and keep more jobs in the U.S.? The bipartisan discussion draft that was released yesterday makes meaningful investments and still will be fully paid for, includes a number of policies that seek to answer those same questions. We started this journey because all of us know patients and families who are desperate for hope. We have also seen and read about the incredible advances made in science as well as in technology. But it has become increasingly clear in recent years that our regulatory policies have not kept pace with innovation, and there is much more that we can do to be doing to provide that hope to folks, and that is what this bill does.

This discussion draft, the product of eight hearings, more than two dozen roundtables, and hundreds of discussions, a number of white papers, incorporates the patient perspective into the regulatory process. It will increase funding for the NIH. It modernizes clinical trials, including allowing for more flexible trial designs so that we can customize trials based on the unique characteristics of patients most likely to benefit. 21st Century Cures will unlock the wealth of health data available to patients,

researchers, and innovators, and can communicate and keep the cycle of cures constantly moving and improving.

We still have important issues to resolve over the next couple of weeks. One placeholder included in the draft is on rescuing and repurposing drugs for serious and life-threatening diseases and disorders. As we move through the process to markup, we will continue to work on a policy to provide incentives to develop drugs that, while they may have failed in trials for one indication, show promise to treat patients facing other serious or life-threatening diseases. We need to ensure the scientific promise to help patients play a more important role than patients in drug development. This policy also will include incentives for doing research on drugs that are FDA-approved, but can be repurposed to help patients with different types of illnesses.

On the important issue of diagnostics, we remain committed to developing a modernized regulatory framework for these products and services. We look forward to working with Dr. Shuren and stakeholders with hopes of having a legislative hearing in July. This hearing and the one-year anniversary of 21st Century Cures are important milestones, but much more work remains to get the bill to the President. Along with the wealth of ideas and support shared over the last year, we have heard repeatedly that patients can no

longer wait. We must get this done this year.

I want to thank all of my colleagues on both sides of the aisle who have participated in this effort, thank the patients who have shared their stories, administration officials, staff, and other experts. I particularly want to thank Ms. DeGette, Mr. Pitts, Mr. Pallone, and Mr. Green for their countless hours and, indeed, partnership. Ms. DeGette joined me in Kalamazoo just this last week where we gained valuable feedback from a number of great groups; innovators, medical students, community leaders, and I look forward to going to her district in the next month or so.

Yes, we still have work to do, but it is important to recognize the incredible progress of this past year and remain focused on our common goal of helping patients. We have a chance to do something big, and this is our time. It is Brooke and Brielle's time as well.

Yield back.

[The prepared statement of Mr. Upton follows:]

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Mr. {Pitts.} Chair thanks the gentleman.

Now yields to the ranking member of the full committee, Mr. Pallone, 5 minutes for an opening statement.

Mr. {Pallone.} Thank you, Mr. Chairman.

Let me thank you, Chairman Pitts, and also Chairman Upton, Ms. DeGette and Ranking Member Green. Today's hearing will examine the draft released yesterday that is the result of months of discussion. It has changed significantly from the draft the chairman released earlier this year. While it is by no means perfect, it does reflect hard work by staff, true collaboration between republicans and democrats, stakeholders, and the Administration, and I hopeful we can bring this legislation to a successful conclusion.

There are a large number of policies in the draft, and not a lot of time to cover them all, but let me just highlight a few. Most notable in the new draft, and the one that I am most proud to see, is $10 billion in mandatory funding for NIH over the next 5 years. It also includes $1.5 billion increase in NIH discretionary authorization over the next 3 years, and this is a real win for researchers, patients, and industry alike. I believe federal funding is the foundation of our biomedical ecosystem, and is one of the most promising ways to spur economic prosperity and

treatments and cures for the 21st century.

We also need to ensure that policies in this draft do no harm. I have said all along that broadly extending drug exclusivity will not solve the problems 21st Century Cures sets out to address, so I am glad to see that this new draft includes placeholder language for a much more tailored approach at solving a targeted problem. We are going to continue discussions on how we can incentivize development of a narrow class of drugs that have been abandoned because of inadequate remaining patent life. Dr. Collins has spoken about the need to provide limited additional exclusivity for drugs that have been found to be safe in clinical trials. Even though they failed the trials for effectiveness, it may be possible to repurpose them for a different indication, or for a different population for which they may be effective. If such drugs fill an unmet medical need for treating a serious or life-threatening disease, it may be appropriate to provide companies with limited additional exclusivity for companies to spend the resources needed to determine if they work. And I appreciate the chairman's commitment to continue to discuss this policy, and ensure that it is targeted to where it is needed. I do not want to undermine the balance between protection and competition that Hatch-Waxman has been so successful in achieving.

Mr. Chairman, with the hard work of staff, I believe we have come a long way, however, there are other complicated policies like interoperability and Telehealth which still need thorough vetting and further consideration. And I have said since I became the ranking member, I am serious about finding common ground on important issues. True bipartisanship is critical to achieving successful and broadly supported policies, and I am confident that this much-improved collaborative process can continue.

I would like to yield now a minute to--initially to Representative Schakowsky, and then the remaining minute or so to Representative Matsui.

So I will yield now to the gentlewoman from Illinois.

[The prepared statement of Mr. Pallone follows:]

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Ms. {Schakowsky.} Thank you, Congressman Pallone.

I want to highlight how vital it is that we provide additional funding to NIH, both mandatory and discretionary. For years, NIH has seen stagnant funding, a trend that simply must be reversed, and I am so pleased to see this legislation includes both $10 billion in mandatory spending, as well as an increase in their discretionary authorization over the next 3 years. I also am encouraged by removal of many of the patent exclusivity provisions that were initially included in the draft released by the majority in January. Added exclusivity is not needed to bring new cures to patients.

Lastly, I believe that we must have a serious conversation about the high cost of medications, and we must do more to address this growing problem. If we are spending billions of dollars to incentivize the development of new drugs, we need to ensure that patients have affordable access to those therapies. I am drafting legislation that would allow HHS to negotiate for better price--prices on certain specialty drugs and biologics. I strongly hope that giving HHS this authority would help to ensure that our healthcare system can sustain the treatments that we hope to advance this legislation.

I want to end by expressing my gratitude to all the

leaders of this effort for giving the rest of us the privilege of giving real hope to millions of Americans who are longing for cures.

And I yield back.

[The prepared statement of Ms. Schakowsky follows:]

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Mr. {Pitts.} Gentlelady yields to Ms. Matsui.

Ms. {Matsui.} Thank you. Thank you for yielding.

I believe in this 21st Century initiative to take advantage of innovation, and to get breakthroughs of cures and technology to patients faster. I believe many of us have friends or family members were too late to it, and so we should use their courage to spur us on forward.

This legislation really does serve to address the roadblocks, and we must continue to get it right. I would like to thank Chairman Upton, Ranking Member Pallone, and Subcommittee Chairman Pitts for working with a bipartisan working group on Telehealth. Technology has huge potential to both improve patient care and reduce healthcare costs. Our ultimate goal as a working group has been to advance quality Telehealth services within the Medicare Program, while recognizing that Telehealth can save the system money. We must continue to work with that.

And critical to the efforts of both Telehealth and Cures is the interoperability of health IT systems, which facilitate population health research and improve patient care. We need to continue to work on this as well.

Thank you, and I yield back the balance of my time.

[The prepared statement of Ms. Matsui follows:]

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Mr. {Pitts.} The chair thanks the gentlelady.

That concludes the opening statements. As usual, all the opening statements of members, if you provide them in writing, will be made a part of the record.

I have a UC request. I would like to submit the following documents for the record. Statements from the American Healthcare Association, Healthcare Leadership Council, Health Level Seven International, National Association of Chain Drugstores, National Marrow Donor Program, The Premiere Healthcare Alliance, The Alliance for Healthcare Common Procedure Coding System Reform, Senior Care Pharmacy Coalition, and The Cord Blood Association, and a statement from the Bipartisan Telehealth Working Group.

And without objection, so ordered.

[The information follows:]

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Mr. {Pitts.} We have on our panel today three witnesses, and I will introduce them in the order of their presentation.

First, Dr. Kathy Hudson, Deputy Director for Science, Outreach, and Policy, at the National Institutes of Health. Secondly, Dr. Janet Woodcock, Director of the Center for Drug Evaluation and Research, at the Food and Drug Administration. And finally, Dr. Jeff Shuren, Director of the Center for Devices and Radiological Health, at the Food and Drug Administration.

Thank you very much for coming today. Your written statements will be made a part of the record. You will each be given 5 minutes to summarize your testimony.

And so, Dr. Hudson, at this point, you are recognized for 5 minutes for your summary.

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^STATEMENTS OF DR. KATHY HUDSON, DEPUTY DIRECTOR FOR SCIENCE, OUTREACH, AND POLICY, NATIONAL INSTITUTES OF HEALTH; DR. JANET WOODCOCK, DIRECTOR OF THE CENTER FOR DRUG EVALUATION AND RESEARCH, U.S. FOOD AND DRUG ADMINISTRATION; AND DR. JEFF SHUREN, DIRECTOR OF THE CENTER FOR DEVICES AND RADIOLOGICAL HEALTH, U.S. FOOD AND DRUG ADMINISTRATION

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^STATEMENT OF KATHY HUDSON

} Ms. {Hudson.} Good morning, Chairman Pitts, Ranking Member Green, members of the subcommittee, Chairman Upton, and Congresswoman DeGette. I want to thank the members of the subcommittee, and especially your amazing staff for all the work that you have done over the past year to move forward this 21st Century Cures Imitative.

I am pleased to testify this morning alongside of my colleagues from the Food and Drug Administration. We work side by side every day to advance the issues that you are attempting to address in this important bill.

How can we accelerate the pace of medical breakthroughs in the United States? How can we get cures to patients faster? Too often, patients and those who love them run out of options. We don't know what the disease is, we don't have

effective interventions for them, we simply don't have the answers. Our shared goal is to usher in an era in which we have the answers, and we have effective ways to diagnose, treat, and prevent disease and disability.

Investments in the National Institutes of Health have resulted in dramatic increases in lifespan, and marked reductions in devastating diseases and disabilities. Take HIV/AIDS. When I was a graduate student in California in the early '90s, I was attending far too many funerals of friends, fellow classmates and family members who had succumbed to the HIV virus. Today, it is unlikely that young people will attend the funeral of someone who has succumbed to AIDS because of the remarkable advances in treatments and preventions that have been made possible by NIH-supported research. While we have much to do, this is a remarkable success story, but we need more.

Today, I want to talk about a few of the areas in which your draft bill can facilitate scientific innovation and collaboration, and increase efficiency through reducing administrative burdens on scientists.

First, you have proposed to increase the funding available to support NIH research. Thank you. Thank you. Thank you. Thank you. The research community is ecstatic to see this new provision in the bill, and we are deeply

appreciative. After a number of years of reduced ability to support research, and diminishing ability to pay for great ideas that are brought before us, this is a dramatic and important moment, so thank you very much. We hope that this increase in support for NIH will be undertaken as a part of broader efforts to support important programs across government.

Second, the draft bill includes a number of proposals to enhance accountability, and we support those. That is why Dr. Collins and his leadership team are undertaking a number of new ways to enhance our stewardship of the resources that you and the American people provide. These include investments in making sure we are investing in the highest research priorities, fostering creative collaborations, and making sure that we are sustaining the biomedical workforce.

Third, I think that we can all agree that scientists should be spending their time doing science and bringing cures to patients. Unfortunately, researchers are spending too much time filling out forms that benefit no one. Your effort to streamline the ability of NIH intramural scientists to attend scientific meetings is one important step. NIH is taking additional steps to reduce burden on our grantees, and we appreciate the inclusion in the draft bill of an exclusion for scientific research from the paperwork-inducing Paperwork

Reduction Act.

Fourth, on data sharing, and you mentioned this, dissemination of research findings is fundamental, and we are using all sorts of new technologies and opportunities to make sure that the results of our investments in research are made available to other researchers, to patients, and to providers. We appreciate very much the inclusion in this draft bill of a specific provision that allows the NIH director to require data sharing for NIH-funded research.

And fifth and finally, while we need to ensure the rapid, unencumbered sharing of data from biomedical research, we also need to protect the privacy of those who volunteer to participate in biomedical research. Although we have taken a number of steps to protect research participants, there are ways in which Congress can be of assistance. Specifically, a statutory change establishing that individual level genomic data are confidential would provide research participants with more robust privacy protections, and enhance public trust and confidence in medical research. This will be particularly important as major new research efforts, such as the Precision Medicine Initiative, move forward.

This concludes my testimony, Mr. Chairman. NIH looks forward to working with you and your staff as you continue to remove the brackets from the draft bill. And I welcome your

questions. Thank you.

[The prepared statement of Ms. Hudson follows:]

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Mr. {Pitts.} Chair thanks the gentlelady.

Now recognizes Dr. Woodcock 5 minutes for an opening statement.

Dr. {Woodcock.} Thank you. Dr. Shuren will be presenting our draft oral statement.

Mr. {Pitts.} Dr. Shuren?

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^STATEMENT OF JEFF SHUREN

} Dr. {Shuren.} It is in the spirit of greater efficiency.

So, Mr. Chairman, and members of the committee, on behalf of Janet and myself, thank you for inviting us to testify regarding the committee's 21st Century Cures proposal. We share your desire to accelerate the development of safe and effective medical products. We would like to thank Chairman Upton, Representatives Pallone and DeGette, other members of the committee, for reaching out to FDA over the past many months to ask for our insights on opportunities to reduce the costs and time involved in studying new medical products, while continuing to protect patients who use those products.

We also want to recognize Congress' critical role in establishing user-fee programs that have led to faster product reviews, and greater collaboration between the agency, companies, and our stakeholders. With your partnership, FDA has been successful in accelerating drug and medical device review times, even as FDA's regulatory review process has remained the gold standard worldwide.

While working together with the committee on the Cures

legislation, we are continually cognizant of the agreements made between the agency and the industry, and enacted by Congress under the Prescription Drug User Fee Act, the Medical Device User Fee Act, and appreciate the importance of assuring that new provisions not impede or conflict with the important ongoing work pursuant to those user fee agreements.

We appreciate the chance to provide input throughout the drafting of the legislation. As we have previously indicated to the committee, we believe there are opportunities to accelerate medical product development. For example, by supporting patient-centered medical product development, encouraging development and qualification of biomarkers, utilizing real world evidence in the review process, reducing barriers to the use of central IRBs for device trials, and strengthening FDA's ability to hire and retain highly qualified experts. We are encouraged that these things have been addressed in this legislation, and look forward to providing additional feedback on these proposals as we evaluate the details of the draft.

There are also several areas that we believe require further improvement to ensure that they do not compromise the safety and effectiveness of American medical products. For example, we appreciate that the committee has been working with FDA and stakeholders to encourage the development and

qualification of drug development tools. We look forward to continuing to work with you to ensure that this language does not divert from important resources, and take those away from drug review activities. We share the committee's goal on advancing the development of new antibiotics through a new approval pathway focused on drugs intended for limited populations of patients with few or no available treatment alternatives, and streamlining the process for updating antibiotic breakpoints.

We thank Representatives Shimkus and Green for their leadership on this important topic, and look forward to continuing to work with the committee on the remaining issues, including the inclusion of a branding element within the labeling of such products that will alert healthcare communities to these products that they are special, and should be treated as such, as well as provisions related to meetings and agreements. We recognize the interest of manufacturers in communicating with health insurers about healthcare economic information, and are evaluating this new language. We will provide feedback on this topic as soon as possible.

We thank Representatives Blackburn and Green, as well as the committee staff, for the opportunity to work with the committee and stakeholders to ensure that medical software is

regulated in a manner that ensures appropriate oversight of higher risk software to protect patient safety, while limiting requirements on other products. In many cases, software is essential to the safe functioning of medical devices used in the diagnosis and treatment of patients. Removing particular types of software from the statutory definition of medical device requires careful consideration to avoid unintended consequences.

We look forward to continuing to work together to address remaining issues, including avoiding the imposition of unnecessary burdens on the agency's effort to streamline its approach to device software that would delay rather than accelerate these actions. We look forward to providing you with additional feedback as we review this new draft, and to ensuring that it meets our shared goal of accelerating innovation, without jeopardizing the safety and effectiveness of medical products. The American public benefits from the efficient and expeditious development and review of innovative medical products, and the safety and effectiveness of those products depends on the high quality of the input and review of FDA.

Thank you, Mr. Chairman, and we look forward to your questions.

[The prepared statement of Dr. Shuren follows:]

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Mr. {Pitts.} Thank you. All right, we will begin questioning.

And the--I will recognize myself 5 minutes for that purpose.

We will start on patient center drug development for Drs. Woodcock and Shuren. Patients are the cornerstone of the 21st Century Cures Initiative, incorporating patient perspective into the regulatory process, and the benefit-risk discussion is a pivotal change to our regulatory structure. The patient focus drug development section builds on the work FDA started with FDASIA in 2012, and I know that both, Dr. Woodcock, Dr. Shuren, both your centers have made progress incorporating the patient perspective in different ways for drugs and devices. What have you done since the enactment of FDASIA in this regard?

Dr. Woodcock, we will start with you.

Dr. {Woodcock.} Certainly. We have held--we are supposed to hold 20 meetings. They are The Voice of The Patient. They are for specific diseases, and we hear from patients, and it is a facilitated discussion of the burden of disease, what is their experience of the disease, what are the various burdens, because really, there is a whole spectrum of burden in--for patients. One patient's

experience doesn't represent the experience of everyone how has a disease. So we hear from a spectrum of patients, and then we write a report called The Voice of The Patient. And then in some cases, we have issued guidance afterward on drug development, talking about, for example, with chronic fatigue syndrome, about how you would develop a drug for that condition.

So what we have really learned is that patients are experts in their disease, people with chronic diseases are experts, and we really need to hear from them, both the burden of their disease, and also how well the treatments that exist, if any, are doing, and what needs to be improved. And what we have learned though is we need a much more structured and organized way to incorporate this input into drug development. And we think that what is laid out in the discussion draft will really help with that.

Mr. {Pitts.} Thank you. Dr. Shuren?

Dr. {Shuren.} Well, in 2012, we put out a framework on the factors we consider, and benefits and risks, and weighing benefits and risks, and approving high-risk and innovative lower-risk devices. One of those factors that we would take into account is patient's perspective on benefit and tolerance for risk. We have been working on draft guidance about how patient perspectives would be included in premarket

review, and in support of device approvals. We have been working as a part of the Medical Device Innovation Consortium, a public-private partnership with industry, patient advocacy groups, nonprofits, and government, and that includes NIH, on a compendium of tools for assessing patient preferences, to then inform product approvals. They are also working on a framework for sponsors for what to take into consideration on patient preferences.

We have also worked with RTI to develop a tool for assessing patient preferences for patients with obesity and the treatments that would best benefit them. The results of that survey we use to inform our decision to approve the very first device treatment for obesity since 2007. So we are actually already incorporating such information into our decisions. And, of course, we attend the drug meetings as well.

Mr. {Pitts.} Thank you. Now, next question for all of you; one on interoperability, and one on pediatric clinical trials.

This legislation is based on the innovation cycle, the way medical products are developed through the regulatory system from discovery, development, to delivery. Some of the fundamental problems we have identified is the challenges of working together, but the committee has identified how

working together is critical for 21st century innovation, and a paramount piece of this is interoperability. Imagine a world where your cell phone would not work with a landline, or if my cell phone did not connect with other networks. Ridiculous. Well, that is the world of electronic health records, and that is the world of health data patients with devices such as diabetes patients, numerous devices collecting data that never get compiled or looked at by a physician.

We are not using this information to innovate and empower patients, and interoperability is the barrier, how interoperability and data collection could be used at your agency to accelerate the science and gain understanding of diseases. The first question, and then comment on how will a global pediatric clinical trial network help accelerate pediatric research in medical products? Dr. Hudson?

Ms. {Hudson.} So let me begin in addressing the question of interoperability. Our colleagues in the Office of the National Coordinator for Health IT are working very hard at fixing the problems of interoperability, and making sure that all of our healthcare providers, and we all have many, are actually able to communicate with each other, and equally importantly, able to share that information in a ready way with us.

I moved my mother from Texas to Minnesota in November, and I ended up carrying two boxes of paper medical records with me. I hope that that doesn't happen in the future, and I think we are moving quickly to solve that problem.

Certainly, interoperability for patient care is extraordinarily important, but having interoperable medical records is also vital for research. And so making electronic medical records, electronic health records, available and accessible for research will be important, especially as we move forward with the Precision Medicine Initiative.

Do you want to--

Mr. {Pitts.} So if you would supply in writing to us the response to those questions.

I will now recognize the ranking member, Mr. Green, 5 minutes for questions.

Mr. {Green.} Thank you, Mr. Chairman. Among the provisions, the draft includes key improvements to FDA's premarket program for medical devices. I believe most significant of these provisions is the establishment of an expedited pathway for breakthrough and innovative technologies. That has the potential to increase the efficiency and predictability of the agency's review process, and improve patient access.

Dr. Shuren, can you comment on the provision creating a

breakthrough pathway for medical devices? Is this complimentary actions at the FDA is already underway?

Dr. {Shuren.} Yes, it is. So we think this is a very important provision. It essentially codifies a program that we just launched the other week that we call the Expedited Access Pathway Program. It is something we have been piloting since 2011. This is an attempt to sort of speed access to very important medical devices. It includes greater collaboration and interaction with the sponsor who is developing the product, but also the opportunity, where appropriate, to shift some data we would otherwise collect premarket, to the post-market setting and gather it then.

Mr. {Green.} Okay. Basic research and translational research are critical to the science advancement. Dr. Hudson, we heard that certain modifications that give increased flexibility would help NIH to leverage funding and advance promising research. The discussion draft includes a provision that removes restrictions on the National Center for Advancing Translational Scientists', or NCATS, ability to utilize its authority and foster development. Can you explain how increased flexibility on the use and funding of NCATS and other transitional authority will help advance scientific research?

Ms. {Hudson.} Thank you very much for the question. So

NCATS, the National Center for Advancing Translation--Translational Scientists, is our newest center at the National Institutes of Health, and it ironically has this limitation on being able to pursue beyond Phase 2(a) clinical trials.

The way that NCATS works is largely in collaboration with other institutes at the NIH to pursue new innovative approaches, to design of clinical trials and the like, and so it having this restriction on being able to move forward in later-stage clinical trials has really limited its ability to do important research. So we appreciate very much the lifting of that restriction in the draft discussion.

Mr. {Green.} Okay. Thank you.

Dr. Woodcock, during our roundtables and hearings, we heard a great deal about the promise of biomarkers. The science is incredibly complex, and the scientific community has a wide variety of views on the issue. The discussion draft includes language on FDA's treatment of biomarkers, but outstanding policy questions need to be answered. We must ensure that legislation provides a clear and workable solution that recognizes the underlying science. Can you share with us your view of what additional authorities would be most helpful to the FDA to facilitate and advance the use of biomarkers and approval process?

Dr. {Woodcock.} I am not sure that additional authorities are needed. For those who are not experts in this, biomarkers are measurements that are made on people, and these measurements help us decide whether a person has a disease, whether giving treatment might help them or not, and also to monitor treatment once they are on therapy. And we have thousands of biomarkers that are now used in clinical trials, but clearly, the new biomarkers, the genetic biomarkers, proteomics, all these new technologies, are going to be very important in helping us do precision medicine and develop new cures. And their progress is slow, and their regulatory acceptance is slow, because not enough evidence is usually generated to decide whether they are worthy of making decisions about human lives. You have to know those biomarkers are reliable before you are willing to take a chance on a human life.

And so the question is what processes should be put in place that help develop these biomarkers and make them robust. The discussion draft codifies some procedures that we have been--have in place called the biomarker qualification process, and during that process, we give advice to developers who are usually consortia, because another problem is there is nobody really in charge of this, and so these consortia come together, patient groups, others,

come together and develop the evidence on these biomarkers. And we provide advice and--about what would be needed to get them to the stage where you would be willing to use them to make decisions about people.

So I think the discussion draft has made a lot of progress, and we really look forward to working with you on finalizing this very important issue.

Mr. {Green.} Okay. Thank you, Mr. Chairman. I am out of time, but I know we will have some other questions to submit. Appreciate it.

Mr. {Pitts.} All right, thank you.

The chair now recognizes the chairman of the committee, Mr. Upton, 5 minutes for questions.

The {Chairman.} Well, thank you again, Mr. Chairman. And, you know, as I reflect on this overall bill, one of the things that I am most proud of is the money for the NIH. And, Dr. Hudson, appreciate your kind words when I talked to Dr. Collins a couple of times over the last week or so, he was very excited. And I just want to read--there was a statement that Andy von Eschenbach, who has been very helpful as well, former FDA Commissioner, of course, he said, and I quote, ``I think it has the potential''--this bill is what he is referring to, ``has the potential of being one of the most transformational pieces of legislation that has come along

since the National Cancer Act of '71.'' And he praised the bill for looking at the entire ecosystem on medical product discovery, development, and delivery, and figuring out how to achieve more synergy between the groups involved, the basic medical research, drug development, approval, and reimbursement.

And I can remember the first roundtable that we had in this room, of course, it was Henry Waxman and myself that led the effort in the House to double the money for the NIH back in the '90s. We teamed up with Paul Wellstone and John McCain in the Senate to get it done. Had a lot of discussions since then, even yesterday with Cory Booker and Durbin, and, you know, it is something that Frank Pallone and Diana, then Joe and--we are all very much onboard to try and increase that money.

The question I have, Dr. Hudson, for you is, so--is the TAP Program, and as you know, the practice of taking away 2-1/2 percent of NIH's research budget through the evaluation TAP, Section 241 in the Public Health Services Act, I have to confess, must create some difficulties when planning.

Can you walk us through the challenges and added burdens that you face when dealing with TAP and its effect on the stability of NIH funding, and would it be in the public's best interests for the NIH to be exempt from that

requirement, as I understand we did in the Cromnibus piece of legislation last year?

Ms. {Hudson.} Well, first of all, I want to reiterate my deep appreciation on behalf of the entire biomedical research community, and also patients for the increase in the NIH budget that is proposed in this bill. It is a welcome change and really quite remarkable.

In terms of the TAPS, they are complicated. They were particularly complicated this year in the omnibus and how they were orchestrated. It requires somebody from the Budget Office to actually walk us through this, but it is-- basically, we still have the TAPS but they are rerouted into NIH with a reduction in the base budget of one of our institutes, the National Institute of General Medical Sciences. That is not an ideal fix for this situation. The TAPS are fairly predictable, and so we are able to base our projections of what we are going to be able to fund, taking into account that we know that these TAPS always come about, and that we account for them in our budgetary and programmatic planning each year.

So they are not unexpected, they support important programs, including programs at the National Institutes of Health. So some of those planning and evaluation dollars come back to us to support important programs--

The {Chairman.} Do you know about what share of that money comes back?

Ms. {Hudson.} I don't know off the top of my head, but we can certainly provide that to you. It is a nontrivial amount that comes back to us as P&E money for us.

The {Chairman.} We are just thinking that as we try to make sure that you have a steady stream, and one that is going up--

Ms. {Hudson.} Yeah.

The {Chairman.} --that that is a source that ought to be, you know, I think, for me, I would feel more--just think that--knowing that it is used for--directly for research is--seems to me, a better thing.

Ms. {Hudson.} Um-hum.

The {Chairman.} Dr. Shuren, you know that as we are developing legislation on a new diagnostics framework, and by the way, appreciate your help across the country as well as we have developed this legislation, we believe that that new framework could serve as a cornerstone to the advancement of the provision medicine and support development of diagnostic tests. And I just want to get your thoughts and continued commitment to work with us as we see this proposal through.

Dr. {Shuren.} Mr. Chairman, we would be happy to work with you. It is also our hope that we can all commit that

the final version on any legislation will have the support of the labs, of the device industry, of all of you, and of course, the FDA as well.

The {Chairman.} And I want to give you a backhanded comment--compliment as well, when Ms. DeGette and I were in Kalamazoo last week, the folks at Striker Medical said very good things about the role that you have been playing, and appreciate all that you do.

So with that, Mr. Chairman, I yield back.

Mr. {Pitts.} Chair thanks the gentleman.

Now recognize the ranking member of the full committee, Mr. Pallone, 5 minutes for questions.

Mr. {Pallone.} Thank you. I wanted to ask a question of Dr. Woodcock first.

It seems to me that we are asking the FDA to take on a lot of new responsibilities in this discussion draft, and the draft would require FDA to issue more than 15 guidance documents and implement a variety of new processes. For example, the section on antibiotic drug development would require FDA to create a separate approval process for antibiotics and antifungal drugs intended to treat serious and life-threatening infections for certain populations.

So can you talk about the time and resources that will be necessary to implement these provisions and issue these

guidance documents?

Dr. {Woodcock.} Well, I think there is a trade-off between putting out new guidances and implementing new programs, and actually getting the work done, giving advice to sponsors and reviewing applications in a timely manner. And I believe that the FDA Amendments Act, which had a large number of provisions in it that we had to implement, shows what can happen. This chart shows that right after--in the green is our performance of getting things done on time; drug applications, reviewing those new products and getting them out on the market. Immediately after the Amendments Act, and for many years after, we were not on time with our review work, and that was because we were implementing the provisions required under the Amendments Act, which were important, but we did not receive additional resources in many cases to do this other work.

So I would say, we have a saying in medicine which is, first, do no harm, and it is very important in, I think, in enacting new legislation to make sure that you don't break what is fixed. And currently, our drug review program is really going full-speed, we are making all our deadlines, and we would like to keep it that way.

Mr. {Pallone.} Well, as you know, the current draft does not authorize any additional funding for FDA to take on

these additional responsibilities, so can you talk about how implementation of these provisions will divert resources from the work that the Center for Drug Evaluation and Research is currently doing?

Dr. {Woodcock.} Well, to the extent that the requirements are statutory, and we have to get guidances out or do other work, set up new programs in a specific amount of time, those are directions from Congress, and those will come first. All right? And we do try to meet all our user fee goals and exceed them because those are the new products that need to get on the market. And, for example, the breakthrough therapy, we try to get those products out the door even faster than the goals because, really, those are products that are going to be life-changing for people. And it is no doubt though that statutory instructions will come first, and we will have to prioritize our resources toward getting what Congress has instructed us to do, done.

Mr. {Pallone.} Well, Dr. Hudson--thank you.

Dr. Hudson, with regard to NIH funding in antibiotic research, NIH funding has also been responsible for generating investment in dry development pipelines, particularly areas of critical public health need, and one such area that needs increased investments is that of antimicrobial development, which the World Health

Organization has named as a top public health threat. How could NIH use increased funding to support antibiotic research and development initiatives, including efforts to improve effectiveness and to help ensure proper stewardship of antibiotics in our healthcare system?

Ms. {Hudson.} So I appreciate the question. Certainly, there are opportunities to explore new--development of new antibiotics. In fact, there was recently, with the support of NIH, the discovery of a new antibiotic from a soil bacteria, as it turns out. So we certainly have opportunities to explore new--the development of new antibiotics, and also to explore the development of approaches to treat antibiotic-resistant microbes. That is a serious and growing problem across the country, and we need to focus additional resources on that serious concern.

Mr. {Pallone.} All right, thank you.

I am just trying to get one more question to Dr. Woodcock. In addition to increased NIH funding, which has long been a priority, one of the provisions in this discussion draft that is especially important is the FDA Grant Authority for studying the process of continuous drug manufacturing, and the conventional process of batch manufacturing is outdated, but continuous manufacturing will benefit patients and pharmaceutical companies by increasing

quality and efficiency.

Dr. Woodcock, can you talk about the difference between batch manufacturing, continuous manufacturing, and what advantages does continuous manufacturing provide, and what do you think--or why do you think it is more widely used in this country for drug manufacturing?

Dr. {Woodcock.} I--

Mr. {Pallone.} You have 7 minutes.

Dr. {Woodcock.} I don't know why--

Mr. {Pallone.} Seven seconds.

Dr. {Woodcock.} --it is not more widely used because if you think of batch manufacturing, it is like cooking, and instead of having like a little cake mixer, that you have a gigantic cake mixer. And then you take all that stuff and you put it into some other machine, and that is what they mean by batch. So you do one operation, then you transfer it to another operation, then you transfer it. There is a tremendous amount of waste, and there is a tremendous amount of opportunity for not getting things right when you do this mass mixing and so forth, and you want to get it into little pills at the end.

So continuous manufacturing at its best, you take the ingredients at one end, the chemicals, and you make the active and then add whatever else you are putting in it,

continuous stream. So it comes out at the end all done, one end to the other. And you can measure it carefully. Each tablet you can measure, whether you made it right or not, by computer. And so the--this is the future of drug manufacturing. It is much more efficient. It also can bring manufacturing back home because there is no reason to do that all around the world, like there is now with these gigantic factories that are needed.

So this cannot be accelerated enough in my opinion.

Mr. {Pallone.} Thank you. Thank you, Mr. Chairman.

Mr. {Pitts.} Chair thanks the gentleman.

Now recognize the vice chair of the full committee, Mrs. Blackburn, 5 minutes for questions.

Mrs. {Blackburn.} Thank you so much, Mr. Chairman.

And, Dr. Shuren, I want to say thank you to you and your team for working with my team and also Congressman Green, as he mentioned earlier, on our Software Act, which is a part of this legislation. We think we are in a better place on that, and we thank you for your participation.

Dr. Hudson, I want to come to you with some questions. The Cromnibus that we passed last December required NIH to do an NIH-wide strategic plan. I want to know where you all are in that process, when it is going to be completed, and are you incorporating some of the elements we are discussing

today?

Ms. {Hudson.} Thank you very much for the question.

So we are, in fact, in the process of developing that strategic plan. We have put together a group of NIH leaders that includes some of the directors of the institutes and centers across the NIH who have begun this process. The Cromnibus requires that we complete this strategic plan by December, and we intend to meet or beat that deadline. We are excited about integrating the overarching strategic plan for the National Institutes of Health with the strategic plans that are already required and provided by each of the 27 institutes and centers. And so those will be linked together in fundamental ways.

We appreciate some of the modifications that were taken into consideration in the revision of the discussion draft; removal of some of the more onerous requirements for the strategic plan and related provisions, but we are well on our way and look forward to sharing that strategic plan--

Mrs. {Blackburn.} Wonderful. We look forward to getting it. We think it is an important part--

Ms. {Hudson.} Um-hum.

Mrs. {Blackburn.} --what we are trying to do through the Cures legislation, that we be focused and strategic, and that we set some goals. And also we think that

accountability and transparency is an important part of this process, and in that, we want to make certain that you all are prioritizing your spending. And so as you go through this process of developing that plan, that is something we are going to be looking for. And I wondered, as we were looking at this, as you look at your spending, do you look at portfolio analysis and conduct that, and you want to speak to that for a second?

Ms. {Hudson.} I do. I do. I appreciate the interest. And we have been looking very carefully, in part because of the constriction and the available budget for the NIH, it has even been more important that we make sure that we get as much value of every dollar that we invest as possible, and that we are investing in the right opportunities to address the challenges that face us, and translating basic science into translation into the clinic. So we have--are in the process of enacting a series of stewardship reforms to make sure that we are looking carefully across the portfolio, and of course, we have the technologies today to be able to do that. It used to be with paper records we couldn't really do that. Now, with the press of a button and some new nifty tools, we can look across and see what are we funding in a particular area, what are other government agencies funding in a particular area, and where are there opportunities that

we need to focus more attention on. So those are great opportunities that we are looking at to make sure that we are spending all of our dollars very wisely.

Mrs. {Blackburn.} Yeah. I was recently at Vanderbilt Children's Hospital in Nashville, and we were discussing a little bit about some of the childhood diseases and research. So talk to me about what you are doing with children. As you look at this portfolio analysis about children benefitting from the cures and the scientific advances that are there through NIH funding.

Ms. {Hudson.} So we are going to be going down to Vanderbilt the--later in the month of May for our working group meeting on precision medicine. We are really looking forward to that. So we spend probably 10 percent of our budget focused specifically on pediatric research. That doesn't say that kids are not included in other studies, but about 10 percent are directly focused on children.

Mrs. {Blackburn.} Okay. Now, let me ask you this.

Ms. {Hudson.} Yeah.

Mrs. {Blackburn.} I am under the impression that you all do not have a method to track all children in all studies. Is that correct?

Ms. {Hudson.} So we do have mechanisms to be able to know that children are or are not included in the studies.

It is a question that is asked of applicants in the grant application. We also have means of being able to follow whether or not children were or were not included in trials in the course of progress reports, and in Clinicaltrial.gov, which is now being upgraded and put--implemented in full force, there is a requirement--

Mrs. {Blackburn.} Okay, my time is expiring, and I want a fuller answer on this, and I know--

Ms. {Hudson.} I look forward to providing that.

Mrs. {Blackburn.} --you would like to give it.

Ms. {Hudson.} But I think that what I--we would like to do is be sure that you have a better system for tracking children so that they are included in the appropriate studies, and I would look forward to working with you on that.

And I yield back.

Ms. {Hudson.} Likewise. Thank you.

Mr. {Pitts.} Chair thanks the gentlelady.

And now recognize the gentleman from Maryland, Mr. Sarbanes, 5 minutes for questions.

Mr. {Sarbanes.} Thank you, Mr. Chairman. Appreciate the testimony today, and I want to congratulate the members who have been working on this piece of legislation for some time now, obviously making tremendous progress with it.

I wanted to follow up a little bit on what Representative Pallone was asking about in terms of the resource challenge potentially for the FDA, Dr. Woodcock and Dr. Shuren. Obviously, I don't have the handle on the inner structure of FDA that you do, but just conceptually, I imagine that there is basically a main review process that exists, and then what seems to have happened over the last few years, for understandable reasons, is we keep pulling things out and creating priority reviews, and expedited processes and so forth. And I wonder if there comes a point at which, if you kind of expedited every last part of what the original main review process was, that you kind of slice the agency up into so many little component parts that you would stand back and look at it and say, well, if we had just gone ahead and expedited the overall main process, we would probably have a more efficient allocation of resources, and we might even have faster review in place.

So could you just comment on sort of, if you take this out to the nth degree, or to its logical conclusion in terms of constantly expediting what you have to do, whether you end up with some kind of structural distortion in the way you are supposed to operate, that even with additional resources, which I think are important, would mean that you couldn't get to the efficiency that you ultimately want to have, and that

the public and that we want to see you have. And it may be that that tension I am describing is really not as much of a challenge as it appears to me, but I would like to get your thoughts about it.

Dr. {Woodcock.} Well, basically, we have expedited sort of review for everything because under the Prescription Drug User Fee Act that Congress has passed multiple times, and then the Generic Drug User Fee Act. We have timelines for everything, all the applications we review, and under the PDUFA we have timelines for meeting with companies, and for getting minutes back to them. We track tens of thousands of different activities that we are supposed to do. And so it is all part of the review program. And the same people then have to do the pediatric program that Congress passed, and they have to do the breakthrough program, and they have to do many other programs that we have that, of course, people have been very interested in. And so I think these things from the drug center point of view could be accomplished with adequate resources, but we are at the point where we add more programs on, with the same people trying to implement them, and we slow the whole thing down, as happened in 2007.

Dr. {Shuren.} So it is a similar situation on the device side, and that is not a criticism about good things people want to do, it is just being--recognizing the fact

that our people are people and they have a lot of work on their plates, and we have commitments to meet, and the more things that get piled on, the more we are set up for failure. It is one of the reasons why I deal with a high turnover rate in our review divisions and in the center, because their workload is high and the more that goes on, the more challenging it is.

You know, when we looked at our budget--what we get for our budget authority for this year, compared to 10 years ago, even though there were some increases, and none since 2011, if you factor in increased inflation and mandatory pay increases, our purchasing power today is the same as it was 10 years ago, but our responsibilities went up. And our only real increases in funding come from industry. They pay for it, but they pay for services they get in return, not for the other things we do. And we are excited that NIH will get more support, but all those great things don't get forward out to the market and those assessments on whether or not they are safe and effective unless we are in the position to do our work.

Mr. {Sarbanes.} Well, and the other, I guess, the bottom line issue is that this effort for expedited review and processing of things creates expectations on the part of the public, and if you can't meet those expectations because

of resources then, you know, you end up creating a more kind of cynical public as a result. So I think it is really important that this resource piece be addressed and be robust.

And with that, I yield back.

Mr. {Pitts.} Chair thanks the gentleman.

Now recognize the chair emeritus of the committee, Mr. Barton, 5 minutes for questions.

Mr. {Barton.} Thank you, Mr. Chairman.

Before I ask my questions, I want to compliment you and Chairman Upton and Mr. Pallone, Ms. DeGette, and others for this discussion, for this draft that we released yesterday on the 21st Century Cures. It is literally transformational. Healthcare has been a priority of mine in the time I have been in the Congress. I helped lead the effort to reauthorize the NIH back in 2006. I have helped in bills to reform the FDA, but I would say this piece of legislation, if it goes forward, and hopefully it will, will be a landmark not just for this Congress, but for many, many Congresses. So I want to compliment you and all the people that have worked on it. I am very--I am extremely pleased with what is in the draft. Now, there are some things that are not that I wish were. I had hoped that my Ace Kids Act, which is bipartisan, bicameral, with over 120 cosponsors, was in the

discussion draft. It has been deleted from this draft. I hope to have discussions about that and perhaps get a hearing just on that piece of legislation because it is certainly worthy of being included, or moving as a standalone bill.

Dr. Hudson, you are the deputy director. I spent quite a bit of time with the director, Dr. Collins, out at the Milken Institute this past weekend in California. I was on a panel with him Monday morning, so I am very pleased that, if he couldn't be here today, that you are here. I am going to ask you some specific questions about what is in the draft, and hopefully you can make your answers succinct so that we can get through a number of questions.

The discussion draft creates a review--a new review panel called Biomedical Research Working Group, to identify and provide recommendations to the NIH director on ways to reduce the overhead burdens. You have existing at NIH a Scientific Management Review Board which is already set up, already established, and basically, either is doing or could do the same thing. In your opinion, could the Scientific Management Review Board that already exists do the function that the new Biomedical Research Working Group is tasked with doing in the draft?

Ms. {Hudson.} So it is certainly a possibility. Either the SMRB could undertake this review, or a working group of

the SMRB could undertake this task. Similarly, it could be a working group of the Advisory Committee to the director. There is also a National Academy of Sciences Study that has just been undertaken to look at scientific burden. This is an important administrative burden on scientists. This is an important problem we need to solve.

Mr. {Barton.} Well, I am certainly not opposed to there being a review of biomedical research, but in my opinion, to create a brand new group doesn't make sense when, as you just pointed out, you have several groups that are already in existence, and the overhead is there, the staff is there, we could just give them that task.

The draft has a creation of an Innovation Fund that it funds at $2 billion for 5 years. Again, I support the concept. In 2006, we created the Common Fund, and we set a minimum of 1.8 percent, which is about 6 or $700 million.

Ms. {Hudson.} Um-hum.

Mr. {Barton.} That Common Fund has done great work, but it has never been increased in funding. It stayed about 1.6 to 1.8 percent of the budget. It is authorized up to 5 percent. In your mind, could not we put this $2 billion that we earmarked for the Innovation Fund and put it into the existing Common Fund, because that was the whole purpose of the Common Fund which was give the director the ability to

move money where it would do the most good?

Ms. {Hudson.} So the Common Fund has been an amazing asset for the NIH, and I appreciate you having created that in the 2006 Revitalization Act. The--an Innovation Fund that is proposed in this discussion draft does include $2 billion, and has two specific purposes, and one other purpose that is yet to be defined. And we look forward to working with you on that.

The specific part of the Innovation Fund that I think is important is that it permits the distribution of those funds to the institutes and centers for innovative research. And so I think that we need the ability to be able to funnel those funds to important opportunities across the institutes and centers.

Mr. {Barton.} Okay. And finally, my last question. The discussion draft creates a biomedical--I mean in the discussion draft--it is not discussion, it is a draft now, a bill, we--it requires each institute director to look at biomedical research at the institution. Congressman Harris, who is on the Appropriations Committee, and myself have a bill that creates a biomedical research officer at OMB, because OMB looks at all the agencies. Which approach do you think is better; letting each institute director do this review, or having somebody at OMB who looks at all the

agencies and that is their only job?

Ms. {Hudson.} So I think that we need to have scientific decisions made by people with scientific expertise who have a focused disciplinary background. So I would prefer that those kinds of decisions remain at the NIH. The institute directors and their Advisory Councils have an important responsibility to not just consider the priority score that comes out of peer review, but also to consider other factors, and we are making sure that those best practices are shared across the institutes and adopted.

Mr. {Barton.} That is not the answer I wanted, but I got two out of three so I am going to declare victory and turn it back to the chairman.

Mr. {Pitts.} That was excellent. The chair thanks the gentleman.

Now recognize the gentlelady from California, Ms. Matsui, 5 minutes for questions.

Ms. {Matsui.} Thank you, Mr. Chairman.

Before I begin my questions about specific provisions, I would like to reiterate points my colleagues have made about how critical it is that we adequately fund agencies to do all the work that we expect them to do. I am pleased that we were able to include both strong discretionary and mandatory funding screens for NIH research in this legislative draft.

I urge my colleagues to provide similar financial support for the FDA as we move forward. We expect the FDA to make sure that our food and our drugs are safe and effective, and it is our responsibility as Members of Congress to ensure the FDA has the resources to do so.

There are several provisions in this legislative package that would help patients with rare diseases. I support the idea of incentivizing the development of new and existing drugs that will make a difference in patients' lives, especially rare disease patients who may not yet have the treatments or cures that they need. However, I am cautious to balance the incentives for development with the ability for generic competition to come onto the market, as that is a key aspect of drug access and affordability.

This bill isn't perfect and there are many pieces that still need to be worked on, but I would like to highlight a few pieces that have the potential to really get at the goal we are all after in an effective and balanced way.

Dr. Woodcock, as you know, patients with life-threatening conditions are often willing to try riskier treatments than other types of patients. The FDA has the Expanded Access Program to increase access to experimental drugs for these patients. 21st Century Cures includes a provision based on the Andrea Sloan CURE Act, which I

cosponsored with my colleagues, Representatives McCaul and Butterfield.

Dr. Woodcock, can you comment on FDA's Expanded Access Program and how the related provision will help patients who seek increased transparency in the program?

Dr. {Woodcock.} Well, currently patients in the United States can get access to investigational drugs if their doctor applies to the company. FDA facilitates these interactions and rarely, rarely turns them down. So thousands of patients--a 1,000 patients or patients every year get expanded access. However, there isn't transparency on company policies on whether or not they will be providing such access and how. And so the bill does urge companies or--to post a policy so that people would know.

We think that having a point of contact also would be helpful because sometimes we don't know who to call to find out how to arrange expanded access for a patient. So we believe that transparency would be helpful, and we believe that in our conversations with the community, that entities will step forward to help broker those connections between the healthcare professionals and the companies so that there is much more transparency in this.

Ms. {Matsui.} Thank you.

Dr. Hudson, a part of seeking cures for patients should

include collecting data about their conditions and current treatments in order to better understand their diseases. A couple of provisions of this package would enhance data collection. I want to ask about the Neurological Disease Surveillance System for diseases like Parkinson's and MS, since CDC is not here as a witness. But surveillance is an important public health function, and I support that provision.

Dr. Hudson, can you describe the idea in Section 1123 to establish a partnership between NIH, FDA, industry, and academia to establish or enhance an IT system to manage data on the natural history of diseases, especially rare diseases?

Ms. {Hudson.} So I believe that section actually provides the authority to the Secretary, and so it will be up to her to make the decision about how that is implemented. And I will turn to my colleagues at FDA to weigh-in on this as well.

There are a number of ongoing activities that provide information especially about rare and neglected diseases, both through the National Library of Medicine and through the Office of Rare Diseases at the National Center for Advanced and Translational Sciences, and what I would like to do as we move forward with this bill is to make sure that these new information systems are compatible and synergistic, in fact,

with existing systems so that we don't end up having many, many different places for information about rare disorders, so that when people are encountering a situation where they have a child, for example, without a diagnosis, that they don't have to go to multiple places to find the information they are looking for, but can readily find it.

Ms. {Matsui.} But I just want to ask how would NIH and FDA work with non-governmental organizations like NORD to incorporate existing disease registries?

Ms. {Hudson.} Go ahead.

Dr. {Woodcock.} Yeah. Well, we are very interested in and, in fact, have been working with NORD, and have talked to other stakeholders as well. When planning a trial of a new intervention into a rare disease, you have to know what happens to the people or you can't make a plan--

Ms. {Matsui.} Sure.

Dr. {Woodcock.} --and that is why we need to collect data over time on people with very rare diseases and what happens to them. And so we are very interested in these tools that will help patient groups actually collect the data, and have a repository so we can plan trials better and developers can understand what they need to do.

Ms. {Matsui.} I thank you very much.

And I yield back.

Mr. {Pitts.} Chair thanks the gentlelady.

Now recognize the vice chair of the subcommittee, Mr. Guthrie, 5 minutes for questions.

Mr. {Guthrie.} Thank you, Mr. Chairman.

Dr. Shuren, the provisions of Cures is--are both big and small, and they all were created to improve the way we develop access to cures. One provision which I have championed is Section 2218, which seeks to create more clarity around the CLIA Waiver process for both the benefit of industry and for the FDA. Can you tell me your thoughts on the benefits of clarifying the CLIA Waiver Program?

Ms. {Shuren.} Yeah, we think--we had put out guidance in 2008 to attempt to provide greater clarity, and we understand there really is more flexibility out there for what companies can do, but we haven't provided that sufficient clarity, both for them and, quite frankly, for our own staff. So we support moving forward to update that guidance and provide that level of clarity and, of course, work with the community on a final product.

Mr. {Guthrie.} Thank you, Dr. Shuren.

And, Dr. Woodcock, matter of fact, Mr.--Congressman Pallone kind of got into the continuous manufacturing, and I am a manufacturing background and so we are looking at this as we are moving forward, and going from batch to continuous,

if it is efficient and--it seems like that would develop naturally through the marketplace. But my understanding, and so I ask that question, is the regulatory uncertainty is what authority you have to grant, and what authority the manufacturers have if they change, does that change the whole process, so we put a provision in to have a grant program to invest in, so it is not just happens just like the marketplace outside because of the regulatory process. So why is it important that we invest, and why do you--why is this necessary to move to a more continuous manufacturing program?

Dr. {Woodcock.} Well, there have been many factors that have led to this industry making such valuable products actually having its manufacturing processes not be state-of-the-art. And some of that has been regulation, because the old manufacturing processes are so uncertain, because of the nature of the bulk efforts that they are doing, they are very strictly regulate and any changes the manufacturer--any substantive changes, they have to apply to us and get approval and so forth. And it takes quite a while. Not necessarily us, but doing all the documentation. And so that has been one factor that has held back innovation in this area.

Another factor though is that these products, I think,

are so valuable, but I don't think the industry, until recently, felt manufacturing was a competitive advantage. And so the R&D people got all the glory, and the manufacturing folks were told just get the product out the door and don't change anything. So now, because of various changes, that has--that is altering, and we are seeing applications with continuous manufacturing, and we are working with companies. We are not a barrier, but we need more of an academic base in this to feed ideas into the manufacturing sector. And that is where we would like to provide more grants and so forth, more funding of some sort, to enable academia to contribute to this revolution.

Mr. {Guthrie.} All right, thank you very much. I appreciate that answer.

And, Mr. Chairman, I--while representatives from CMS are not here today, I do believe it is important to touch on an area that will be addressed in Cures for which more work needs to be done. The national and local coverage discrimination process within CMS are the processes whereby new technologies gain entrance to the Medicare Program, and I have heard numerous concerns about the current processes, specifically for LCDs, that need to be addressed, and I certainly deeply appreciate the bipartisan support for the narrow provision that is included in this bill. However, I

believe there is still more to be done, and I plan on gathering more information on this topic and working with stakeholders to gather more ideas on ways to improve the LCD process.

I look forward to working with the committee and the Administration as I move forward. And thank you, Mr. Chairman, and I yield back.

Mr. {Pitts.} Chair thanks the gentleman.

Now recognize the gentleman from Massachusetts, Mr. Kennedy, 5 minutes for questions.

Mr. {Kennedy.} Thank you, Mr. Chairman. I want to thank the witnesses for your testimony today. Thank you for coming. I also want to thank the chairman of the subcommittee and ranking member, and Chairman Upton, Mr. Pallone, Ms. DeGette, for all their hard work in bringing this bill to this place where it is. It has obviously undergone an awful lot of work, and from somebody in Massachusetts who has a vocal constituency that is very much looking forward to the movement of this bill through. Excited to see the progress, and obviously, a lot of work that still needs to be done.

But I wanted to focus a little bit, if I can, back at funding mechanisms for NIH. And, Dr. Hudson, maybe to start with you. Obviously, federal investments in medical research

have, and continue, to transform healthcare, advance new treatments, therapies and screenings. Nowhere is this more evident than at NIH. In fact, the 2011 Health Affairs Studies found that nearly 1/2 of all patents for new drugs cite public sector patents or research in their applications. Increased investments in NIH yields groundbreaking research, fuels industry, serves as a foundation for this Nation's greatest scientists. Funding has obviously stagnated for years. And as I indicated, this is a huge--not at--certainly not a week goes by, and often not a day goes by when I don't have constituents that come into our office and indicate that this is a huge priority for Massachusetts.

Thrilled to see the increase in funding that is included in this bill. And wanted to dig in a little bit to your thoughts around the Innovation Fund. So the first priority there is precision medicine which, again, from Massachusetts, we have some great companies that are developing life-changing precision medicines to treat cancer, cystic fibrosis, Gaucher's Disease, and--just to name a few. There is a lot of progress there--or promise there. I think we have to work through some still--challenges as the process goes forward, but I was hoping you could dive into the precision medicine funding mechanisms a bit. Another priority there is young scientists which, again, comes on a

daily or weekly basis to me from our hospitals and provider communities saying that they are losing young, talented scientists to other industries, or even to other countries. Wanted to see if you could touch on that.

And the third piece that--I know it might be a bit premature, but--is that other bracket. So what do we think other might mean? And I don't mean to put you on the spot, but if you can flush that out a little bit, I would be grateful.

Ms. {Hudson.} Thank you very much. So on precision medicine, we are still in the early stages of trying to really sketch out a specific plan for the national cohort part of this in which we want to invite a million or more Americans to share with us, share with researchers their health information, genomic information, and environmental exposures, behavioral information and the like. And patients are eager to do that. They want to make sure that the best information is made available to advance their heath and that of their families and other Americans. So that plan is being developed. We are really excited about it, and hoping to use new innovative mechanisms of being able to fund that research, and also leverage the resources of others in the private sector to do some collaborative work together.

On emerging scientists, this is a substantial problem.

We need to reach sort of an equilibrium in the workforce pipeline so that we can attract new investigators in. Certainly, young people are going to see this $2 billion mandatory funding stream as an opportunity to--and encouragement to stay in and dig in, and stay with the biomedical research enterprise.

And then in terms of that other category, which is intriguing and we haven't had a lot of opportunity yet, since it has only been out for 24 hours, to talk about it with the leadership at NIH, but I think initial considerations are we would really like to be able to make sure that we are funding innovative investigator initiative research. The best ideas come from the best brains across America, and we don't necessarily anticipate what those ideas are going to be until they come before us. And right now, we are only paying 18 percent of the grants that come to us, and we know we are leaving great science unfunded. And so being able to pay more of that good science would--might be a priority as well as the brain initiative.

Mr. {Kennedy.} I have a minute left and so--

Ms. {Hudson.} Yeah.

Mr. {Kennedy.} --I wanted to get a brief discussion from the rest of the panelists as well.

You, Dr. Woodcock, I think indicated that basic tenet of

do no harm. We are putting a lot of exciting opportunities at your doorstep. Do you--as contemplated, does FDA have the resources to actually make these transitions and make these investments as effectively and as efficiently as possible, particularly when part of the challenge, at least that I hear, again, from my communities back home, is how long it takes to get some of these drugs and devices approved?

Dr. {Woodcock.} Well, I think we are very stretched. I think we are up against the wall always. We are always asked to keep doing more with less. We do not take a long time to get things approved. They take a long time to get developed. And it is our advice that is so important, and that would be one of the first things to go because that is more discretionary, but the--it has been shown that we can cut years off of company's development time by giving them--if they come in for timely advice and we--because we see across the board all the development programs. But yes, we are very stretched in our resources. And, of course, some of the hiring and assistance that is contemplated in this draft is--would be helpful as well because we are also below our ceilings.

Mr. {Kennedy.} Great. Thank you.

And, Dr. Shuren, apologies, but I am over time. So thank you very much for your testimony and thanks for coming

today.

Mr. {Pitts.} Chair thanks the gentleman.

Now recognize the--

Mr. {Kennedy.} Chairman, thank you.

Mr. {Pitts.} --gentleman from Illinois, Mr. Shimkus, 5 minutes for questions.

Mr. {Shimkus.} Thank you, Mr. Chairman. And just--since--it has been a long time since Mr. Green was asking his questions, but there is one point of what he was asking that I just wanted to build upon in the Subtitle K. So--and, Dr. Shuren, can you tell me the types of resources contained with the priority view for breakthrough devices section of this bill, and how important they can be to the FDA and industry when seeking approval of a breakthrough product?

Dr. {Shuren.} So we do think this is an important program. It is something we had launched. It can tremendously help important technologies getting to marketing, getting to patients, but still safe and effective technologies. Our challenge will be having the people to do this work. We know from piloting the innovation pathway in 2011 it requires a lot more people to do it. I think Janet and her program on the drug side found it requires a lot more people to handle breakthrough drugs.

When we proposed our program, we said we would do it

resources permitting, because we do not want to jeopardize the commitments we made under the User Fee Act or the other work we have to do. With the statutory provision, the challenge we have is this is mandated, we have to do it, and the law says so. And we are concerned that when we move forward on this, we will not have the people to succeed at all the things we have to do, and the things that are important to do for patients.

Mr. {Shimkus.} So in going to Subtitle L, which contains a number of regulatory improvements for both the FDA and industry, for instance, Section 2201, the third party quality system assessment can lower the burden on both FDA and the industry when such actions are warranted.

I am wondering if you can spend a few minutes and tell us how the FDA sees this section improving the Cures delivery cycle.

Dr. {Shuren.} So this program is--pertains to modifications that are made to high risk devices under PMA, and moderate devices under a 510K. And it looks at a subset of modifications that, if we had assurances the company had what we call a good quality system, it is essentially their system for designing, making changes, supplier controls, manufacturing, that we would not need to see those modifications. We could rely on a third party assessment of

that quality system for those device types. And we think that would be very helpful to industry. We looked at it, will this be an efficiency for us, and it turns out probably not, and here is why. It will cost us money to set up the program and maintain it, to have the people that go out training the third parties and auditing them. At the same time, we might free-up some of the work we do in reviewing these submissions. They tend to be the work--less work for those kinds of submissions for modifications. On the other hand, we lose all of the user fee revenue we would have gotten. So when we crunched the numbers, this may actually cost us money.

We still think if we can work this through it could be a very good thing to do, but we have to be cognizant about the resource implications.

Mr. {Shimkus.} Thank you. That is very helpful.

Yeah, and for the chairman and the ranking member, I know Mr. Green and I are pleased that adapt language in the draft is in this current draft, and give credit to Dr. Gingrey, former member, who was really a pusher of that in the last Congress. And I have been pleased to take a lead with Mr. Green on this process. It is reported, as you know, over two million Americans each year get sick due to antibiotic resistant bacteria, and tens of thousands die as a

result. And I can go over all the stats, we all know them. I guess getting just to the question, it is really--I still--even though I am happy with the draft, there is still, I think, a need, if we want to respond and we want to expand immediately and more appropriately for continued incentives.

So, Dr. Woodcock, would you want to speak on that issue?

Dr. {Woodcock.} Yeah, there is probably--we probably can't do enough to get this crisis addressed. We are doing more under GAIN. GAIN was very helpful. We thank you. This--the--we think that a limited population approach will be very helpful as an incentive because it has fewer patients and fewer costs associated with it, and it will be faster. We still believe, of course, we don't think we need a new program, and we would really like to see a logo or some kind of statement in the label. However, even if this program is enacted, I think it will attract investment because it is a very limited development program, and so the bar is lower. However, I don't know that that will be enough.

Mr. {Shimkus.} So, Mr. Chairman, just--so you are saying probably additional incentives might be needed?

Dr. {Woodcock.} Well, we can't do enough to address this crisis in my opinion.

Mr. {Shimkus.} So you are saying additional incentives might be needed.

Mr. {Pitts.} Chair thanks the gentleman.

And now recognizes the gentlelady from Florida, Ms. Castor, 5 minutes for questions.

Ms. {Castor.} Well, thank you, Mr. Chairman, for calling the hearing today.

I am very pleased with the progress on the 21st Century Cures Initiative by the committee, and want to thank Chairman Upton and Ranking Member Pallone, and my good friend Congresswoman DeGette, and Congressman Green and Chairman Pitts as well. I think it is moving in the right direction.

One of my top priorities as a Member of Congress has been to ensure steady and robust funding for the National Institutes of Health. Today, medical research in America is entirely discretionary. So that means that it is at the mercy of all of the congressional budget battles and sequester, and that brings on a lot of uncertainty. And I know all of my colleagues hear the same thing from research institutes and scientists in their own district. We will only save lives unless we have robust funding of medical research in America. And I think Dr. Hudson really said it in a very kind way, that we have a diminishing ability to pay for the treatments and cures of the future. We have really fallen behind. There was a recent Journal of American Medicine that went into how we are at risk of losing our

competitive edge to other countries around the globe. And, in fact, in the last 2 years, I have offered amendments in the Budget Committee to the federal budget to shift medical research funding from the discretionary category into the mandatory section because I don't believe that medical research in America anymore is discretionary. This is something that we have to demonstrate a commitment to. But I--you know, those amendments were always voted down on a party line vote, but the dialogue was very interesting because there was a great sense of--that something needed to be done. So I think it is appropriate that it is the Energy and Commerce Committee and the authorizing committee that begins to take that step towards moving research funding into the mandatory section.

I am also very pleased with the precision medicine portion and the Innovation Fund. Under what is currently happening at NIH, I know $200 million of that will go to expand cancer genomics research. And there is a very exciting collaboration underway at the Moffitt Cancer Center in Tampa, along with Ohio State and the new partners of University of Colorado, New Mexico, University of Virginia. And what they are going to do is launch a database with more than 100,000 patients who have consented to contribute tissue and clinical records for research to understand cancer at the

molecular level. They are going to use the total cancer care protocol to create a collaborative environment.

I know, Dr. Hudson, you had mentioned that before, and it appears you believe that this bill continues to give NIH the flexibility that you need to move forward on those kind of initiatives, is that right?

Ms. {Hudson.} It does, and we deeply appreciate the new investment in NIH, or proposed investment in NIH. We agree that investments in medical research really are mandatory. We must invest in medical research in order to bring cures to patients.

Ms. {Castor.} Thank you. And, Dr. Woodcock, on the precision medicine provisions in this draft bill, is the same true for FDA? I know the Center for Drug Evaluation and Research has been actively working for a number of years with a particular focus on pushing for the development of targeted therapies. I understand CEDAR has approved 30 such therapies since 2012. This new section in the draft is intended to help you, but tell us, does it help, is it counterproductive, does it need additional work?

Dr. {Woodcock.} Well, the basic research that underlies understanding disease can only help in developing treatments for those diseases. So, yes, I think that investing in biomedical research to understand diseases will generate a

new level of understanding that will lead to more target therapies for a wide variety of diseases.

Right now, it is concentrated in cancer, in rare diseases, and in a couple of other areas, and the goal here, I think, is to make it more--make precision medicine more broadly available by understanding the genetic basis of these.

Ms. {Castor.} Okay, that is very helpful.

And I would also like to add my concern for not having the ACE Kids Act included in 21st Century Cures, and I look forward to working with my good friend and colleague, Congressman Barton, to work on that. That is the Advancing Care for Exceptional Kids Act to improve how we deliver care to children with complex medical needs. And I thank Congressman Barton, Chairman Emeritus, for raising the issue today.

Thank you, and I yield back.

Mr. {Pitts.} Chair thanks the gentlelady.

Now recognizes the gentleman from Pennsylvania, Dr. Murphy, 5 minutes for questions.

Mr. {Murphy.} Thank you, Mr. Chairman. It is great to see this panel here. Thank you so much for your valuable input.

Couple of quick questions. Dr. Hudson, in the bill on

page 65, you don't have to look it up, but the draft version of the 21st Century Cures legislation it states, and I will read it for you, medical research consortia consisting of public-private partnerships of government agencies, institutions of higher education, patient advocacy groups, industrial representatives, clinical and scientific experts, and other relevant entities and individuals, can play a valuable role in helping develop quality biomarkers.

Can you give me some input on what you see is the value of these public-private partnerships as laid out in the legislation for biomarkers?

Ms. {Hudson.} So there certainly are opportunities for representatives from different sectors to come together to explore what are the challenges and opportunities in being able to develop biomarkers. And as Dr. Woodcock mentioned, biomarkers are really measurements of something that is going on, and those are used sometimes in preclinical research, and are extraordinarily valuable, but the ones, of course, that are of highest interest are those biomarkers that are used as surrogate endpoints in clinical trials that are related to drug development. And so we can certainly work collaboratively together, and are. There is a biomarkers consortium that involves FDA and NIH and others. There is the Critical Path Institute that is involved with multiple

stakeholders and looking at biomarker issues. The Accelerating Medicines Partnership, a great new public-private partnership that was launched just over a year ago that includes us, FDA, and a number of pharmaceutical companies and patient groups. It is also looking at biomarkers development, especially in Alzheimer's Disease.

Mr. {Murphy.} I think I am going to come back to Alzheimer's in a moment.

Dr. Woodcock, I want to ask both of you this question too. Consortia like this are key in biomarkers for mental illness, it seems to me. In July of 2014, the Psychiatric Genomics Consortium identified 128 independent associations spanning 108--that are common in schizophrenia. It was a major, major breakthrough. So how will the 21st Century Cures legislation help translate some of these insights derived from this research to new medical treatment such as drugs to treat serious mental illness? Either of you comment on that?

Ms. {Hudson.} Well, certainly, the increased investments in NIH will allow us to support additional research, particularly at the National Institute of Mental Health. And I know you have had many conversations with Dr. Insel about the investments and their importance. So that would be the primary benefit of the new 21st Century Cures

legislation for us and moving that field forward.

Dr. {Woodcock.} Well, I--as I have said many times, I believe there is somewhat of a gap between the basic discovery of these and what you need to--the evidence you need to generate to understand which one of them is actionable. We would really like to be able to subset schizophrenia. We would really like to be able to do earlier diagnosis. Right? We would really like to be able to do early intervention, but how do you get from identifying these genes and actually to something you can take action on? And that is evidence generation that some of the things that consortia are doing, but I feel that enough of it is not occurring.

Mr. {Murphy.} Well, let me add to this, you know, we are dealing here also with really alleviating a lot of pain and suffering from patients and their families. We heard from the President's Council on Science and Technology on the costs imposed by major chronic illnesses like Alzheimer's, and stunningly, the President's Council noted that Alzheimer's imposes a huge financial burden on America's economy with an annual cost of about $200 billion. The National Institute of Mental Illness, Dr. Insel, I think he wrote that there is about $57 billion cost also, which is equivalent to the cost of cancer, just for treating severe

mental illness, but those numbers are probably way low. NAMI estimated that for bipolar alone, the costs were $45 billion per year. And yet I am frustrated, as I am sure NIH and NIMH are, that we spend only about $900 million a year on researching mental illness, this devastating brain disease.

So do you see, I would like to ask this panel, do you see this bill in helping us move forward then, and do we need to tweak anything in getting more funding, more research, more focus on these devastating brain diseases such as Alzheimer's and severe mental illness? I will let you go across the panel.

Ms. {Hudson.} So I think that mental illnesses are particularly challenging. We don't understand very much about how the brain actually works, and understanding the normal function of the brain and the abnormal function of the brain is going to be critical in order for us to make breakthroughs in terms of treating many of these devastating mental illnesses.

One opportunity and where we can certainly have increased investment is in the brain initiative in order to understand the networks and circuitry in the brain, both in the normal human brain and in the abrupt, misfiring human brain. That will help in a whole host of mental illnesses and in neurological diseases as well. And so that is an area

where I think is ripe for investment. The Blue Ribbon Panel that set forth the spending plan for that, we have not yet made those budgetary targets, and we would be happy to move those numbers up.

Mr. {Murphy.} I recognize, Mr. Chairman, my time is up, so perhaps the rest of the panel could submit the questions for the record--their answers for the record. I would appreciate that.

Mr. {Pitts.} Chair thanks the gentleman.

And now recognize the gentlelady from Illinois, Ms. Schakowsky, 5 minutes for questions.

Ms. {Schakowsky.} Thank you, Mr. Chairman. I just want to say I feel a sense of bipartisan mission here, some excitement that we are standing on the brink of some very important discoveries. It is a wonderful feeling that we seem to be in agreement, and the--all the gratitude that has gone to the leaders is certainly well deserved to bring us to this point.

I wanted to specifically follow up on a question on the--on Representative Castor's line of questioning. And so I wanted to ask you, Dr. Woodcock, given the efforts that FDA has already taken to advance precision medicine, do you believe you need additional authority from Congress? Do you need new authority to pursue the goals laid out in the

President's Precision Medicine Initiative?

Dr. {Woodcock.} We don't believe we need new authorities for precision medicine. Actually, diagnosis, you know, is the foundation of medicine, and for hundreds of years doctors have been getting diagnosis more and more precise. And the precision medicine, we are really trying to use new molecular knowledge, like gene knowledge, to get even more precise. But that is sort of how drugs--drug regulation works. We figure out what patient population could benefit, and then they are treated. And so we have been doing this--we perceive a great groundswell of activity, we hope--we all hope, over the next few years in precision medicine, but it is an extension of the way drugs have been used for a very long time, and we just hope to get a lot better at it.

Ms. {Schakowsky.} So that is helpful. And as you know, there is a new precision medicine section that is in this draft. I believe it is intended definitely to further your efforts in this area. Can you tell us if you think it will accomplish that goal, this new section, recognizing that it may still need some tweaking? I think we all want to be helpful here and don't want to do anything that might be counterproductive.

Dr. {Woodcock.} Okay. We look forward to working on--with the committee on this. The version that was in

yesterday was changed from previously, and we need to take a close look at that, and we really look forward to working with you on it.

Ms. {Schakowsky.} Very good. I wanted to--while we are all forward-looking today, I think it may be helpful to just look back on what happens a little bit when we don't adequately fund NIH. I know that over--between 2003 and 2015, NIH actually lost about 22 percent of its funding. So, Dr. Hudson, I know--I remember Francis--Dr. Francis Collins talking about how we may have been more advanced in Ebola research, for example, and even some sort of vaccine had we had the funding to do it. I wonder if there are other examples of things that maybe we can do now that we couldn't do because of the lack of funding?

Ms. {Hudson.} I think probably one of the most devastating effects of the budget constrictions over the last several years has been the lack of appeal for careers in biomedical research--

Ms. {Schakowsky.} Um-hum.

Ms. {Hudson.} --for young people. So as I go to scientific meetings and conferences, and often with Dr. Collins, we hear repeatedly the sort of chronic depression of youngsters who are questioning whether or not it is worth pursuing a career in biomedical research, and that is

particularly true for MDs or MD-PhDs who could instead be in clinical practice where there is a more secure career trajectory, rather than in biomedical research where the success rate right now, and we hope now to see this rise, is 18 percent. And so people are spending a lot of time writing grants and not getting them funded. I had a meal this weekend with a girlfriend of mine who I went to graduate school with who won a Nobel Prize, and she was talking to me about how she has been really desolated by the budget cuts and by young people now not being interested in coming to work in her lab to pursue important research questions. So I think we are--we have gone from a very--we are potentially going from a very dreary phase in biomedical research to a much brighter phase, and for that we are very grateful.

Ms. {Schakowsky.} I hope so. The--also start and stop in terms of research funding makes it difficult, so I hope this is the beginning of continued funding going forward.

Thank you so much. I yield back.

Mr. {Pitts.} Chair thanks the gentlelady.

Now recognize the gentleman from Texas, Dr. Burgess, 5 minutes for questions.

Mr. {Burgess.} Thank you, Mr. Chairman. And before I start, I just want to underscore that the interoperability of electronic health records is a top priority for me. And I

know reading in the press this morning that my bandwidth has been exhausted by finally achieving success on the sustainable growth rate formula, I just want to assure everyone that I have good minds working in my office on this issue of interoperability, and it will remain a top priority. I am, of course, relieved that the--Chairman Pitts and Chairman Upton, the Ranking Members Pallone and Green also have made a similar commitment to this issue, and it is my sincere hope to have this issue advanced by having this markup--to have this issue advanced by the time we get this draft to markup.

So I have talked in the past about my own frustrations with electronic health records, and here we are years later and I am still hearing from doctors that electronic health records failed to deliver on the promise. Patients seen in the emergency room with chest pain, follows up with their cardiologist, that doctor should be able to review the patient's health information recorded by the hospital without the patient having to request that it be faxed, without the secondary doctor having to pay an exorbitant fee, without having to agree to use the same electronic health record vendor as the hospital, and yet many times that is the way our world is working. And it is frustrating for doctors, and it is bad for patients. Doctors and hospitals have invested

time and money to make this switch to electronic health records, and we in this committee, under the Stimulus Bill and to some degree under the Affordable Care Act, have invested 28 billion taxpayer dollars to support this transition. Developments in the technology have far outpaced the capabilities of the systems. This is not a tech problem, this is a bureaucracy problem, and we can fix it.

So, Dr. Hudson, let me ask you, if people were able to seamlessly share their health information in electronic form with the National Institute of Health, would it improve researchers' ability to identify patterns in diseases?

Ms. {Hudson.} Yes.

Mr. {Burgess.} Thank you. Thank you for being succinct.

Another issue, and I am very committed to protecting First Amendment rights of clinicians, to share and receive truthful medical information. The current draft, in my opinion, must do much more in this area.

So, Dr. Woodcock, given that approximately half of the medicines prescribed to treat cancer patients in oncology centers are used by physicians off-label, and over 60 percent of pediatric prescriptions are off-label, wouldn't it benefit patients if the manufacturers of these medicines could provide physicians and payers with the most up-to-date

truthful, non-misleading information about drugs with no delay?

Dr. {Woodcock.} Well, there are multiple pathways, of course, that clinicians can get information from manufacturers, they can talk to them, there are scientific meetings, there are publications, and so forth, and there are downsides to establishing essentially a market for a drug before it has been tested for a given indication. Now, for economic purposes, for payers, formulary committees, we understand that a free flow of information is needed, and we look forward to working on that.

Mr. {Burgess.} Right. There are First Amendment considerations here, but it seems like the FDA should allow a company to distribute to a physician. The peer review New England Journal of Medicine article, for example, that may have been important in getting this product approved in the first place.

And before my time has expired, I do--really do appreciate, Mr. Chairman, you holding this hearing today and I appreciate our witnesses being here. And I know it is a long hearing, and to some degree, we are all somewhat longwinded and drawn out.

On the issue of precision medicine, on the issue of personalized medicine, I do worry that some of the things

that have happened recently, within the last year and 1/2, have kind of put the brakes on what should be happening in that space, and specifically, I am referring to genomic information which should--why is my genomic information that 23andMe has, why is it locked up and why is it locked away from me now. Why can only--I get ancestral information from 23andMe. It is great to know my mother was descended from Jessie James, I always suspected that, but actually it would be more useful I knew whether or not I was at risk for multiple sclerosis, for example. And on the concept of precision medicine, we have dealt with laboratory-developed tests before. The ability of a doctor to get a more precise diagnosis is--sometimes hinges upon getting those laboratory-developed tests and not impeding their development. And then finally, the whole concept of medical apps. It is one that has exploded since really we have begun having some of these hearings, and I very much look forward to the day where medical apps, laboratory-developed tests, and consumer-directed genomic information can help direct that precision medicine.

Mr. Chairman, I will yield back.

Mr. {Pitts.} Chair thanks the gentleman.

And now recognize the gentleman from Oregon, Mr. Schrader, 5 minutes for questions.

Mr. {Schrader.} Thank you, Mr. Chairman.

Go back to maybe a little more basic questions, as a new member of the committee and stuff. What--how does both FDA and NIH prioritize the research, trying to juxtapose that research that gives the biggest bang for the greater population at large versus making sure that there are these opportunities for subgroups and breakthrough populations, and will this be part of your addressing this bill?

Ms. {Hudson.} So the way in which priorities are selected, and funding decisions are made is a combination of factors. First, we want to fund only the very best, most meritorious science, and that is determined through a process of peer review, which is sort of the gold standard. But that is only one measure of--one input for our funding decisions. Another is what are the diseases and disorders that are most profoundly affecting our population. And so that certainly weighs into our considerations as well. What is our existing portfolio of investments, and where are there potential gaps that we need to fill. And then lastly, where are there specific scientific opportunities. And sometimes that comes because there was a breakthrough in another area that shined some light on another unexpected area--

Mr. {Schrader.} Um-hum. Um-hum.

Ms. {Hudson.} --and then we need to chase after that,

and we need to do that with some alacrity. And so those are really the 4 basic mechanisms. And we are able to go out to the community and say we are interested in looking in these specific categories of research. They are high priority to us, come in with your best ideas. At the same time, leaving open the door for people who have their own ideas of the next best thing, that they can come to us with their great innovative ideas, investigator-initiative research, often basic research that is vital to our entire portfolio.

Mr. {Schrader.} FDA, same question.

Dr. {Woodcock.} Well, for the Center for Drugs, we have really a miniscule research budget. We are not really a research institution, all right, and we do testing--a lot of testing, say, counterfeit drugs and things like that. We also do applied research on matters that relate to regulating drugs, like how would you establish that a biosimilar drug is biosimilar.

Mr. {Schrader.} Um-hum.

Dr. {Woodcock.} And so we have to have scientists who actually do that hands-on in the lab, so they are capable of evaluating an application when it comes in.

Mr. {Schrader.} So both of you have strategic plans then to address how you prioritize the testing and/or the things you actually research.

If I could get a--my office could get a copy of that just so we have some idea of how to approach.

I guess the second question would be on the continuous manufacturing opportunity. The question I have is, you know, are there cost differences between that and the batch manufacturing that has been traditional within the industry?

Dr. {Woodcock.} There is going to be sort of an entry cost that will be high to switch over to this technology, and so we expect that, say, generic manufacturers may not switch over for quite a while because it needs to get established, the equipment manufacturers need to have stable offerings, and so forth. Once you get into continuous manufacturing, we would expect it generally to be less expensive because it has a much smaller footprint, much less waste, much fewer failures, and is higher quality actually. So--but getting into it is a radical departure from the way it is done now--

Mr. {Schrader.} Sure.

Dr. {Woodcock.} --and so will take investment.

Mr. {Schrader.} Would the, you know, would the pharmaceutical companies and device manufacturers agree with that?

Dr. {Woodcock.} Well, I don't know that it is relevant to devices so much, Jeff can speak to that, but yes, I think now the innovator industry really understands the opportunity

for them--

Mr. {Schrader.} Sure.

Dr. {Woodcock.} --and so they are moving very briskly into this area, whereas the generic industry, which actually supplies most of the drugs that Americans take every day, operates on smaller cost margins, their profit margins, and so I think they will be slower to enter this area.

Mr. {Schrader.} Yeah, I just wanted to make sure, you know, the manufacturers in our country, by and large, do a very good job. We have, I think, some of the safest drugs in the world, and you and others make sure that that occurs, which I appreciate. So I was just trying to get to the cost benefit type of playback that would be there.

I guess the last question would be for our NIH folks, Dr. Hudson. Are we--how do we--how do you work with pharmaceutical companies on the antibiotic, antifungal research, make sure you are not duplicating--many of them have huge R&D budgets, how do you make sure you are not duplicating what they are doing?

Ms. {Hudson.} So there is a network of investigators who specifically work on antibiotic research, and they are closely coordinating and communicating with the private sector on where our research investments are, and I would be happy to provide additional information on that for the

record.

Mr. {Schrader.} Great, thank you very much.

I yield back.

Mr. {Pitts.} Chair thanks the gentleman.

Now recognize the gentleman from New Jersey, Mr. Lance, 5 minutes for questions.

Mr. {Lance.} Thank you, Mr. Chairman. I would like to submit for the record a letter from the chief executive officer of the Parkinson's Action Network here in town regarding the legislation, especially regarding the integrated electronic health records with the Clinicaltrials.gov, and I would ask that this be submitted for the record.

{Voice.} Without objection.

Mr. {Lance.} Without objection.

Mr. {Pitts.} Without objection, sure.

[The information follows:]

********** COMMITTEE INSERT **********

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Mr. {Lance.} Thank you.

I was pleased to see in the latest iteration of the legislation a placeholder to incentivize and advance the repurposing of drugs to address serious and life-threatening diseases, and I have been working on this for quite some time. I am glad that there is a bipartisan agreement that this issue deserves our focus, and ultimately real policy solutions as part of the larger legislation.

Dr. Collins alluded to some of the some of the challenges in bringing cures and treatments to patients during one of our many roundtables last year, and I am deeply appreciative of that. Dr. Collins noted specifically that this was a problem where compounds failed to gain approval, but researchers later discovered potential new uses for cures and treatments for patients.

Director Hudson, can you give us a sense of how NIH has encountered and observed some of these challenges through its drug repurposing initiatives?

Ms. {Hudson.} I would be happy to, and thank you for the question.

So at our newest center, the National Center for Advancing Translational Sciences, one of the first programs that we started in that program--in that institute, and I was

honored to be the deputy--acting deputy director there at its onset, was a drug reuse program. And it is a wonderful partnership between a number of pharmaceutical companies, ourselves, and academic partners. And really, it is intended to take compounds that have proven to be safe in humans, but have failed in efficacy or have been abandoned for business reasons, economic reasons. And companies have been willing to share those compounds and provide them to us, and then they are offered up for academic researchers to see whether or not those molecules might actually be effective for a new use. And there was a recent paper that was quite dramatic in which a drug that had originally been developed by AstraZeneca for cancer, a researcher at Yale was looking at the available compounds. He had done some research on Alzheimer's and found that there was a particular kinase that was activated in Alzheimer's. He saw this kinase inhibitor that was available from AstraZeneca through our program, got it, used it in mice, restored neuronal synaptic activity, and restored some memory loss in these mice models. And it has moved very briskly into clinical trials in humans. So in 18 months, we have moved a compound that had failed in cancer, into phase two studied in humans. It is a pretty remarkable progress, and more programs like that would be very beneficial. We need to make sure at the end of the day that

somebody is going to commercialize those. And so we look forward to working with you on the specific provision in the bill.

Mr. {Lance.} Thank you, and I hope that this is included in the legislation that reaches the subcommittee, the committee and on the Floor of the House.

I would like to discuss briefly a different provision of the legislation that I have been working on with my colleague, Mr. Griffith, related to Clinicaltrials.gov. Last year, a constituent of mine contacted me expressing his deep concern and frustration with Clinicaltrials.gov. His young son had recently passed away from brain cancer, and over the course of his son's treatment, my constituent looked to Clinicaltrials.gov in the hopes of finding a trial for his son. Not only did the site lack a significant amount of information, but it was confusing and ultimately unusable. The legislation we have been working on aims to correct this by clarifying and streamlining the information included in Clinicaltrials.gov, and making the site an effective resource for both patients and physicians. And it conforms to what others are already doing, and I urge NIH to support this effort and make these meaningful changes.

Dr. Hudson, in your testimony, you stated the scientific community and the public expect data generated, that federal

funds will be shared to enable further insights to be gained. This is exactly why we are supporting these provisions, and why I hope that this is in the legislation. Would you please comment on your views on this?

Ms. {Hudson.} So thank you for your interest in Clinicaltrials.gov. I have to--I have a particular passion about this database and making sure that it is exceptionally useful to patients and providers and to researchers. I have to say that when I started getting engaged with Clinicaltrials.gov, I learned that it was very difficult for researchers to try to submit their trials into the database, it was difficult for patients and families and providers to easily search the database, and as a result of that, we have made specific targeted investments to increase the usability of Clinicaltrials.gov. We have a notice of proposed rulemaking, we have gotten comments back, we will be finalizing those rules to make sure that every single applicable clinical trial under the regulation, and all NIH-funded clinical trials, are registered and their data are submitted, and that the data is available.

There are some specific provisions in the draft where data--structured data elements are suggested, where I think they may be less than helpful at the end of the day. And we would be interested in working with you to make sure that

there are ways in which people can get the information without placing inordinate burdens on the researchers, and without actually trying to box up information in ways that ultimately it is less useful for being able to retrieve it. We have sophisticated search functions, we can be able to provide this information. I think we received the same letter that was sent to you from your constituent, and we are going to do better.

Mr. {Lance.} Thank you. My time has expired. This is an important issue and I hope to continue to work on it.

Thank you, Mr. Chairman.

Mr. {Pitts.} Chair thanks the gentleman.

Now recognize the gentlelady from California, Mrs. Capps, 5 minutes for questions.

Mrs. {Capps.} Thank you, Mr. Chairman. And thank you to all our witnesses for your testimonies.

I am so pleased we are here discussing investments in critical research and innovation, and want to commend the committee staff who have worked so hard to improve the latest draft of this bill.

Early on in my time in Congress, that was over 50 years--15 years ago, I was very proud that we were able to work across the aisle to nearly double the budget of the National Institutes of Health. I think it was a high-water mark for

this Congress. We continually see how vital these federal research dollars are to medical innovation. NIH supports the best research in the world, and has contributed to dramatically improving the lives of so many Americans, but there still is much more to be done. That is why it is so crucial that this bill provides an increase of $10 billion for NIH research. It is important that we provide the necessary support that NIH requires to continue to be the gold standard in research and development. I have always believed that supporting NIH is one of the smartest investments that this Congress can make. As we all know, NIH is driven by innovation, however, we still face significant barriers in turning scientific knowledge into new therapies and effective treatments.

Last Congress, the National Pediatric Research Network Act was signed into law. This legislation was led by myself and Congresswoman McMorris Rodgers, and it targeted the difficulties in pediatric disease research, especially for research on rare diseases. The low prevalence of these diseases makes them particularly hard to research, but for those affected, a new cure or treatment could mean a world of difference.

So my first question, again, Dr. Hudson, I am kind of--we are picking on you today. Can--could you talk briefly, I

have three questions for you, but first, how the National Pediatric Research Network Consortia--Consortium described in the bill might have an impact on the study of rare pediatric diseases or birth defects?

Ms. {Hudson.} So there are a number of pediatric research centers and networks that already exist, close to 100 different research centers and networks, and those networks already provide important infrastructure for being able to do critical research on pediatric diseases, especially rare diseases. So we have newborn research network, we have a number of networks that are already in place. We look forward to building this new network and making sure that it is complimentary to, and not duplicative with, the existing research networks that we have in place.

Mrs. {Capps.} Thank you. My colleagues have heard me talk before about a family in my district with spinal muscular atrophy, and you know these rare diseases affect not just the person who is involved, but the entire family, and many times a wider network of folks as well. That is why devoting resources toward gaining better understanding of treatments of these particular diseases is so crucial to entire communities. As NIH takes on this critical research, we must ensure robust funding for this important program. That is my pitch, myself and my colleagues.

Another question for you. We know children also have unique healthcare experiences. Treatment needs research challenges. Children are not just little adults, and medical discoveries that apply to adults don't necessarily apply to children. NIH has had a policy in place for almost 20 years requiring that children be included in NIH studies unless there is a good reason not to do so. While I applaud this policy, I believe that we can do a better job of not only tracking the number of children in research, but also distinguishing between subgroups like infants and teens where there are tremendous differences. As many of you know, NIH tracks specific populations such as the number of women and minorities who are enrolled in the studies of funds, and this information is available on Clinicaltrials.gov. But now my question is to you, Dr. Hudson. I believe NIH should track the number of children it enrolls in studies and their ages on these Web sites as well because there are such major differences between them. Adding to this Clinicaltrials.gov could achieve--adding this to Clinicaltrials.gov could achieve the goal of more robust data regarding children in NIH studies. Do you agree?

Ms. {Hudson.} So certainly, the inclusion of the ages that are sought for inclusion within clinical trials--

Mrs. {Capps.} Right.

Ms. {Hudson.} --is being included in the registration information for Clinicaltrials.gov, and then when the summary data is reported, the ages are also included in that but in an aggregate form. I think we could also do more, especially with new technologies, electronic technologies and data technologies, to extract more information earlier in the process so when we are looking at the grant applications, when we are looking at the progress reports, that we would be able to monitor in a more robust way the inclusion of children before the study is already awarded and the trial is underway. And so we look forward to working with you to make sure that we are--

Mrs. {Capps.} Great.

Ms. {Hudson.} --paying close attention, using all the technologies that we have.

Mrs. {Capps.} And, Mr. Chairman, I realize my time is up, but I have one more additional question to you, Dr. Hudson. Perhaps I will submit it in writing. Thank you.

Mr. {Pitts.} Chair thanks the gentlelady.

Now recognize the gentleman from Virginia, Mr. Griffith, 5 minutes for questions.

Mr. {Griffith.} Thank you, Mr. Chairman. I would be happy to yield a minute to the gentlelady if she has one more question.

Mrs. {Capps.} Well, that is really thoughtful of you. Thank you very much.

The question--because it follows in a line with these others, I wonder if you could describe how this data sharing might increase our understanding of potential differences in the way medical treatments affect women and minorities as well. I mean this kind of provision would help up, would it not, better understand the effects of treatments on differing populations and subsets? I hope NIH continues its work to include more women and minorities in clinical research as well as children, and look forward to working with you. But is it just perhaps an extrapolation.

Ms. {Hudson.} And we are, in fact, looking forward to being able to have these kinds of data so that we can draw conclusions of data in sets rather than individually, to draw important conclusions about disparities in health and health outcomes--

Mrs. {Capps.} Great.

Ms. {Hudson.} --that would direct us for future research. So we have the tools now to be able to deploy to really ratchet up our attention to these issues.

Mrs. {Capps.} Thank you very much. And I yield back.

Mr. {Griffith.} Taking back my time. Let's stick with Clinicaltrials.gov. You heard both the gentlelady before me

and Congressman Lance talking about some of the concerns from some of the folks there, and I don't want to put words in your mouth, but I gathered from some of the comments you made back to Congressman Lance that you are not completely supportive of Section 1102 that deals with making sure that there are certain data points in there. How would you improve--we certainly want to work with you on it, but we also--I feel very strongly, and I know others do too, that we continue to improve this to make it easier for patients and others to get the data they need. What particularly do you have a problem with in 1102, and what would you think that we needed to add to it?

Ms. {Hudson.} So there are a number of elements there that the draft suggests be provided a structured data field, and they are pretty straightforward and we can certainly do that. We certainly have proposed that in the notice of proposed rulemaking. We are currently evaluating the 800 or so comments that came in in response to that, largely overwhelmingly positive. So we are excited about that and getting a final rule out, and we want to do that soon.

In terms of the elements where we have more concerns about whether or not you can actually put it into a discreet category really concerns the eligibility and exclusion criteria. For clinical trials, often the inclusion and

exclusion criteria are complex and aren't easily definable into subunits, and so by forcing investigators to put inclusion and exclusion criteria into structured data elements may actually lose some of the wealth of information that we would want to have available to patients, providers, researchers, research reviewers, et cetera. So that is really the area that we have the largest concern, and we would be happy to sit down and talk to you in more detail about that specific provision.

Mr. {Griffith.} Well, I certainly hope that we can work on that because--

Ms. {Hudson.} Yeah.

Mr. {Griffith.} --we don't want to exclude folks, but we also want to make sure the data is out there, and right now, as you have heard, there is a lot of concern about whether or not the data is really out there.

Ms. {Hudson.} Yeah.

Mr. {Griffith.} So we need to make sure it gets out there.

Ms. {Hudson.} Yeah. We--

Mr. {Griffith.} Because that is one of the things we see as very important with this, and with the next section in the draft bill, which is 1121, the clinical trial data system. And I believe the more that we can make that data

available, the more likely we are--obviously, you have to make sure that you take away the personal identifiers, but there have been all kinds of studies that say that we can do that.

Ms. {Hudson.} Yeah.

Mr. {Griffith.} And I think that means that we are going to find better ways to move forward.

Ms. {Hudson.} Yeah.

Mr. {Griffith.} You were talking about a drug recently that there had been a failure in in one area, but it worked somewhere else.

Ms. {Hudson.} Yeah.

Mr. {Griffith.} That is the kind of data, I think, if we can enact this section, and again, it is a draft proposal, we can tweak it, but if we can get this section drafted where we can get that information out there to as many researchers as possible and to as many people as possible, I think we are going to be able to find, just like that researcher, and I have forgot the university, was it--

Ms. {Hudson.} Yale.

Mr. {Griffith.} Yale. Who suddenly said, hey, I think this will work over here, when it didn't work for cancer, it did work perhaps--

Ms. {Hudson.} Yeah.

Mr. {Griffith.} --for Alzheimer's. I think that is the beauty of that particular section. I feel very strongly about that section staying in this bill as it goes forward because I believe that the more people who look at the data, somebody is going to have an ah-ha moment, a eureka, and jump out of the bathtub exclaiming that they have suddenly figured out how to solve the problem.

Ms. {Hudson.} May I comment? So--

Mr. {Griffith.} Yes.

Ms. {Hudson.} So that provision specifically requires that NIH or the Secretary contract to an outside entity--

Mr. {Griffith.} Um-hum.

Ms. {Hudson.} --who would then collect patient-level data from clinical trials that are supported by the NIH. It is not clear to me, frankly, that having us contract with an outside entity is the most effective way to get data available, and we are already experimenting with a number of mechanisms of making patient-level data available from specific programs where, in the RFA, we say we want to do it and then we do it, and we--there are different models that have been tried by different institutes. And I think we need to look carefully at what we are learning from that experience to--before we sort of jump into a statutory mandated requirement for all NIH clinical trials. This is

going to be a burden on our investigators, and we have not yet established the value for all clinical trials, as opposed to--

Mr. {Griffith.} What we want to try to do--

Ms. {Hudson.} --particular subsets.

Mr. {Griffith.} --is to ease the burden on patients and ease the burden on those who are trying to find cures for the patients' diseases. And I think it is important that we move forward with the taxpayers' money to make sure that as many people as possible can have access to that information.

And my time is up, so I will yield back.

Mr. {Pitts.} Chair thanks the gentleman.

Now recognize the gentleman, Mr. Butterfield, 5 minutes for questions.

Mr. {Butterfield.} Chairman Pitts, I thank you for holding today's hearing on the most recent legislative draft of the 21st Century Cures Initiative. I certainly appreciate the hard work of members, and particularly our staff. I look forward to continuing to work with you and our colleagues to see that 21st Century Cures meets and crosses the finish line.

I understand, Mr. Chairman, that our staffs have worked beyond the call of duty, and I just wanted to personally thank each one of them on both sides of the aisle.

By all accounts, Mr. Chairman, this has been a bipartisan process. I have had the pleasure of working with my colleagues on this committee, Congresswoman Renee Ellmers and Congressman Gus Bilirakis, and even with Congressman Mike McCaul, who is not on this committee but we all know him very well, on advocating for our shared priorities that span political parties. I am appreciative of the inclusion of some of my priorities in today's draft, including Subtitle D on disposable medical technologies. I must say, however, that I was very disappointed to learn that H.R. 1537, the Advancing Hope Act, was not included, nor was language that would achieve the same goal. The Advancing Hope Act would permanently reauthorize the Pediatric Priority Review Voucher Program, which has proven to be tremendously successful. Since its introduction, I have received overwhelming support from biopharmaceutical innovators and over 140 patient groups and rare disease organizations who have urged this committee in writing to include provisions in this initiative that would make the Pediatric PRV Program permanent.

And so I would ask unanimous consent, Mr. Chairman, that these letters dated March 30 and April 13 be inserted in the record.

Mr. {Pitts.} Without objection, so ordered.

[The information follows:]

********** COMMITTEE INSERT **********

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Mr. {Butterfield.} Mr. Chairman, the Pediatric PRV Program addressed the market failures we have seen as rare pediatric disease drugs have struggled to market by creating financial incentives for rare pediatric disease drug development in the form of vouchers. The PRV Program cost taxpayers absolutely nothing. Let me repeat, nothing. While at the same time helping to speed treatments and potential cures to pediatric rare disease patients who desperately need them.

So, Mr. Chairman, I hope that this committee will seriously consider including legislative language that would make the Pediatric PRV Program permanent in any subsequent 21st Century Cure drafts. I respectfully make that request of you, Mr. Chairman, and to all of my colleagues, and I look forward to working with you to see that that happens.

I have several questions, Mr. Chairman. In the interest of time and because I have an ambassador sitting in my office waiting for me right now, I will submit my questions for the record, if that would be acceptable.

Mr. {Pitts.} That is acceptable.

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

Mr. {Pitts.} Chair thanks the gentleman.

And now recognizes the gentleman, Mr. Bilirakis, 5 minutes for questions.

Mr. {Bilirakis.} Thank you very much, Mr. Chairman. I appreciate it. Thank you folks for your testimony this morning.

Dr. Woodcock and Dr. Shuren, anticipating more combination products in the future, can you tell the committee what steps FDA is taking to refine its current approach to facilitate the development of these innovative combinations?

Dr. {Woodcock.} Well, we have a combination product office that carries out the directions of Congress in trying to figure out--in figuring out whether there is a drug lead or a device lead for products. The device center and the drug center work very closely together in working on these products, but I must say that the statutes governing devices and the statutes governing drugs were put in place a long time ago, and they didn't really contemplate, I think, these new products, they--which is probably part of the future of medicine. And so we are working very hard to try and put these--make these two statutes congruent.

Dr. {Shuren.} That is a place that does require probably further discussion, and whether or not there are changes to be thought about to make that intersection work

better than it currently does.

Mr. {Bilirakis.} We might have some suggestions for you, so I would love to--

Dr. {Shuren.} We would be happy to have the conversation.

Mr. {Bilirakis.} Thank you.

Second question. During the 21st Century Cures roundtables, we often heard about the cures gap, the enormous golf between approved therapies and known diseases, which leave many patients with no treatment to turn to. Patients in the rare disease community understand this challenge, where market realities often make it more difficult to develop therapies for diseases with smaller patient populations. I believe there is a great--there is great promise in repurposing drugs. In fact, earlier this year, I introduced the Open Act with my colleague, Representative Butterfield, who had to leave to see the ambassador. It would foster research to increase the number of safe, effective, and affordable rare disease medicines for patients by incentivizing drug manufacturers to repurpose their approved products for rare disease indications, by providing an additional 6 months of market exclusivity when a product is repurposed and approved by the FDA for the treatment of a rare disease. Ninety-five percent of rare diseases have no

FDA-approved treatments.

My first question is to Dr.--Director Hudson, and of course, to Dr. Woodcock. Can you comment on how repurposing already approved drugs may hold therapeutic promise for rare disease populations?

Ms. {Hudson.} So I think there are a number of examples where drugs that were initially approved or pursued for one indication have proven to be effective for other indications. And in some cases, those have been rare and neglected diseases. We appreciate very much your interest in this area, and really look forward to working with you to come up with a provision that would be appropriate for being able to actively pursue this area where there is such opportunity to accelerate the delivery of new medications for patients that really need them.

Mr. {Bilirakis.} Thank you. Dr. Woodcock?

Dr. {Woodcock.} Well, I think we need--in rare diseases, you need to understand something about the disease, and then, of course, having a range of therapies that you can try, and being able to pick from those because you understand something about what--which is the example Dr. Hudson just gave about Alzheimer's. So obviously, there is a whole range of treatments out there, and those that have not made it to the market would expand that universe of things that could be

tried. So I think as disease understanding improves in rare diseases, there is an opportunity to try many compounds.

Mr. {Bilirakis.} Thank you. My next question. What incentives are currently available that encourage research into rare and orphan applications in drugs that are already approved by the FDA for a separate indication? Maybe for the--we will start with Director Hudson, and then Dr. Woodcock.

Ms. {Hudson.} So there are specific research programs at the NIH, including the Office of Rare Diseases, the Therapeutics for Rare and Neglected Diseases, there are a number of programs that are specifically focused on supporting research for diseases that affect a small number of people in the population. And then in addition, and Dr. Woodcock can address this, there are incentives and a poll from her end as well.

Dr. {Woodcock.} Yeah, the Orphan Drug Act was a very successful program that has brought many, many treatments to rare diseases, and it includes incentives during the development, as well as exclusivity provisions after a drug is marketed for that indication.

Mr. {Bilirakis.} Thank you. Sir, would you like to comment as well?

Dr. {Shuren.} So we have a program, the Humanitarian

Device Exemption, to facilitate and incentivize the development of devices for rare disorders, and I actually want to compliment the committee because there is a provision in this bill that will now change the cap for HDEs, and I think potentially provide greater incentives for device development in this area.

Mr. {Bilirakis.} Very good. Thank you very much.

And, Mr. Chairman, I will yield back. I do have another question, but I will submit it for the record. Thank you.

Mr. {Burgess.} [Presiding] Chair points out the gentleman's time has expired.

The chair would identify--recognize the gentleman from New York, Mr. Engel, 5 minutes for questions please.

Mr. {Engel.} Thank you. Thank you very much, Mr. Chairman.

Throughout my time in Congress, I have been a very strong advocate for those suffering from rare diseases. I authored the ALS Registry Act and the two most recent Muscular Dystrophy Act reauthorizations. I know the 21st Century Cures Initiative holds great promise for the patients and families afflicted with rare diseases if it is done well, and I am encouraged by the progress made with the latest discussion draft, and hope that continued refinements will lead to legislation that we can all support.

Dr. Woodcock, one of the concepts I am pleased to see included in the latest discussion draft is the section related to biomarker development qualification. I know that the FDA utilizes biomarkers often in making drug approval decisions, but to date there is not, I believe, a formal process to put in place to qualify biomarkers. So while I understand that FDA approves many products based on surrogate endpoints, I have also heard that the FDA has only qualified only a handful of biomarkers. So could you explain how the FDA currently uses biomarkers, and what the difference is between qualified biomarkers and surrogate endpoints?

Dr. {Woodcock.} Sure, although it may take your whole 5 minutes.

Mr. {Engel.} That is okay.

Dr. {Woodcock.} The--generally speaking, drug developers, during their development program, can come into FDA under the user fee agreements, and they can get agreement that is more or less binding with the FDA on their pivotal trials. And those trials might include a surrogate endpoint, which is not a clinical measurement like do you feel better, but is your tumor stable, all right, not--or it could include selection criteria which might be by biomarkers. Do you have a certain tumor marker or do you just have certain genetic mutation that would match with this therapy. All right? And

we can agree with that, but that whole process is confidential. And that is how most of these have gotten on the market, for rare diseases and regular diseases, is the companies have gone through a process which is confidential, we agree with their use of the biomarker, they use it, and then the review process occurs.

To use biomarkers more generally, a number of years ago we started a qualification process which was considered to be different. I would be public. And there we would want everyone to be able to use the biomarker, not just the company within its development program. So those are different kind of biomarkers usually, and the groups that have come into us are consortia, patient groups, and so forth, because they are looking, say, at safety biomarkers, something that an individual company might not be interested in developing, but this would apply to all drugs. For example, we are going through qualification now for drug-induced kidney injury and markers of that. It will be much better than the markers we currently have if they are accepted.

So we have actually approved 12 separate biomarkers through our qualification process, we have qualified those, but they were in five different programs. So people say we had five different biomarkers, but we have really had 12.

All right? But there are many more in the process. They are not under review by us, they are--we are giving them advice on how to develop these biomarkers, and generate the evidence needed to make decisions about human lives or human kidneys, or whatever. So we have a robust qualification process going on right now. It is not in a statute, it is something that we put out in guidance, and that we manage. And the European Medicines Agency, we also worked with them, and they have a parallel process. We often do this qualification together.

Mr. {Engel.} Thank you. And you didn't take up the full 5 minutes, so I can get in one more question.

And let me ask this question for anybody who cares to answer it. I am fully supportive of the goals behind the 21st Century Cures Initiative, but I think that we really know it won't be possible to achieve the ambitious goals set forth in the discussion draft without providing adequate resources to the FDA, CMS, and NIH. I didn't vote in support of the Budget Control Act, but I know that all of our witnesses have faced significant cuts to their budgets over the last several years as a result of sequestration. And I know that our witnesses have not had a lot of time to review the discussion draft released yesterday, but can each of you, or whoever cares to do this, share in broad terms what kind of staff and financial resources you believe will be

necessary to meet the requirements outlined in this discussion draft?

Dr. {Woodcock.} I--we would be glad to get back to you on that. I don't think we have had time to analyze this draft, but we do feel it will have significant resource implications for the FDA.

Mr. {Engel.} Do the others agree?

Ms. {Hudson.} So the discussion--the draft includes a significant increase in funding for NIH, which we think we can spend in effective ways, although we are concerned about other agencies and making sure that, as we address resource issues, that we also address resource issues for FDA and other agencies across government.

Mr. {Burgess.} All right--

Mr. {Engel.} All right.

Mr. {Burgess.} --gentleman's time has expired.

The chair recognizes the gentleman from Missouri, Mr. Long, 5 minutes for any questions please.

Mr. {Long.} Thank you, Mr. Chairman. And thank you all for being here today in this important hearing.

And, Dr. Woodcock, does the FDA have a Twitter page and a Facebook page?

Dr. {Woodcock.} I don't know whether the FDA does, but I know that my staff has--does things on Twitter.

Mr. {Long.} It is my understanding that they do have a Twitter page and a Facebook page, and when the FDA puts out Tweets about new drug approval, it is limited to 140 characters, so generally, they don't include the safety information and warnings about a drug within the Tweet itself. Is that--if you don't know they had one, I don't know how you can answer this, I guess, but let's assume they do have one.

Dr. {Woodcock.} Well, generally, they--just a factual statement about the drug approval and the indication.

Mr. {Long.} Okay. So in a social media post, the agency does not include the information in the body of the message which, again, in Twitter is 140 characters, and instead notes the new approval, and then provides the rest of the safety and effectiveness information in a detailed link. So the question that I have is, when regulative manufacturers use of social media, wouldn't a similar commonsense approach make sense to let the manufacturers do the same thing?

Dr. {Woodcock.} Well, I think the reasoning that has been pursued is that manufacturers have a different stake in presenting the information than does the agency.

Mr. {Long.} A different what?

Dr. {Woodcock.} Stake.

Mr. {Long.} Stake?

Dr. {Woodcock.} Yes.

Mr. {Long.} Okay.

Dr. {Woodcock.} In other words, that we are, you know, we are presenting this information as a factual matter from a government agency that does not market the drug.

Mr. {Long.} So would it be unreasonable for a company to use the name of the drug and have proved indication in a Tweet?

Dr. {Woodcock.} We have issued some draft guidance on this, and I think we would be glad to get back to you. We are currently re-evaluating our policies on regulation of drug advertising in light of recent jurisprudence, and we would be happy to discuss that further with you.

Mr. {Long.} But doesn't it benefit patients in discussions with their doctors to know about new medical advances, including the names of new drugs and their approved indications? Wouldn't that be beneficial to the patients?

Dr. {Woodcock.} Yes, and there are multiple pathways for that information to get out there now.

Mr. {Long.} Okay, well, don't you think the FDA should encourage this type of communication, rather than making it more difficult, assuming that the information is accurate, to be able to do the same thing that the FDA does as far as getting out the information and linking to other things?

Dr. {Woodcock.} We can get back to you on what our current guidance says about this on social media, and what we, you know, and the--

Mr. {Long.} I know what your current guidance says, but I would like to have your word that you will work with the committee and work with my office as far as trying to put these commonsense approaches into place, because I think that it is beneficial to the patients and to the doctors. So I just would like to have your word that you will look and work in that direction, as I have been told off-the-record that the FDA will be able to--

Dr. {Woodcock.} Yes, we will be happy to work with you on this.

Mr. {Long.} Okay, I appreciate that. And thank you all for being here today.

And with that, Mr. Chairman, I yield back.

Mr. {Burgess.} Chair thanks the gentleman.

Chair recognizes the gentleman from New York, Mr. Collins, 5 minutes for your questions please.

Mr. {Collins.} Thank you, Mr. Chairman. This has been a great hearing, and I want to thank Dr. Woodcock for taking the time earlier this week to meet with me and talk about some issues, and certainly my bill on the Bayesian statistical model for adaptive trials, and I appreciate your

support of that. I think--this is the 21st century, not 1950, and I think that is going to be good for all of us.

I was also very impressed with your knowledge and your dedication to safely getting new drugs to market, and that is what we are all about. But with all the novel and the complicated issues that we are asking the FDA to analyze and approve, I do worry that the FDA may not have the latitude and the government hiring process to hire the best and the brightest minds in the field. Now, HHS currently works under a cap on the number of senior biomedical researchers, that applies to the NIH and the FDA, and also salary caps. Now, the good news is the draft that we have now eliminates the cap on senior biomedical researchers. It also substantially increases the pay, I think it is to the level of pay up to that of the President of the United States, which is substantially more than we have now, and hopefully will make you competitive. But I do worry that there are 2 other barriers and, Dr. Woodcock, I would like you to maybe speak to those. The first one is the hiring process itself, where these are unique individuals, these are very high-paid individuals with very specific traits that are necessary for you to do the job that we are asking you to do, but yet, as I understand it, you are stuck in the traditional hiring process. It can take you 9 months, you may not even get the

name of the person you want to hire on the list. So if you could speak to that, and hopefully, what we can do here is eliminate that and allow you to have, for these levels of folks, the ability to hire the people you need. And then the other one is the little nuanced issue of one of these folks coming out of big pharm, Pfizer, something like that, with stock, and that, while they are willing to put them in a blind trust, which I am thinking is all we should ever ask, that is not currently allowed in your hiring process, and that could stop you from hiring someone. So if you could speak to those two issues and, frankly, give us your recommendation how we can still, in this draft, make changes.

Dr. {Woodcock.} Thank you. Yes, I am sure that Dr. Shuren has this same challenge, and I know it occurs across the FDA. The science right now is exploding, the new products are extremely innovative. That is wonderful, but we need to have some good scientists who can go toe-to-toe with the best in industry, and industry can afford the best scientists. And we have great difficulty hiring at that senior level. There--as you said, there are caps on--there have been caps on the hiring authorities, there have been caps--there are caps on how much we can pay the people, there are actually caps on how much we can promote--how much we can give them to promote them, that create tremendous disparities

internally in how people are paid, depending on when they came into the government. And we can't--we have extreme difficulty hiring senior people who have worked outside the government because of their holdings, and the conflict of interest rules, and we can't use blind trust for them to deal with their stocks. So recently, I had someone who said, you know, I really want to come, this was a very senior doctor, he said I really passionately believe in the mission, but I can't give up my family's future for--to do this, and I just can't do it. And we have heard that again and again. So we have major barriers to hiring senior people.

Dr. {Shuren.} I would add we have the exact same problem. I have lost great people as a result. On the flipside, we have great people at the center, but because I can't pay a competitive salary, we essentially are the training ground for industry. That is what the American taxpayer is paying for. And so we train them, they are terrific, they leave, they take that knowledge with them, and that disrupts our reviews, it makes it much harder for us to have the good people, and it ultimately--it hurts patients.

Mr. {Collins.} So I mean let's go back to the specifics. We have addressed two of the issues in this draft, but I am assuming you would like us to also get language in there that allows you the discretion to hire the

people you need without going through the bureaucratic hiring practice, and number two, allow these senior folks to put their holdings in a blind trust, and therefore, be able to come to work for HHS. Is that correct, those two would be very helpful?

Dr. {Woodcock.} Yeah. I don't understand the rules about financial arrangements well enough to know, you know, how that would be done, but it is clear that it is a huge barrier right now, and we can't get people who are experienced from all these industries we regulate. And so that--and direct hire is a kind of authority that is very helpful to us when we have it. We can just identify people and bring them in. I mean, as you know, people are worried about--the federal hiring system is worried we are all going to hire our relatives, but I don't have too many relatives who are PhD neuropharmacologists, you know what I--and so it is--and so there are so many safeguards and everything, we end up--we can't reach the people who we need. And that would be tremendously helpful. I am not sure how that should be done--

Mr. {Collins.} Well--

Dr. {Woodcock.} --but it would be helpful.

Mr. {Collins.} I think that is one of the things we can try to work through as this draft moves along, and I thank

you all for your testimony today.

And I know my time has expired, but I still yield back.

Mr. {Burgess.} Gentleman's time has expired.

Chair now recognizes the internally patient Ms. DeGette for 5 minutes for your questions please.

Mr. {Green.} Mr. Chairman, before you let her time start, I would like to say, Congresswoman DeGette, like Chairman Upton, has worked so hard on this for the last year, I want to thank her, but her patience was shown today, not only working on this legislation but also sitting here. And by the way, former Congresswoman Karen Thurman, who came in with me a few years ago and--from Florida, has been here also very patiently, along with a lot in our audience. Thank you, Diana.

Ms. {DeGette.} Thank you. Thank you very much. Well, actually, I have a leg-up, having sat through this whole hearing today because now I know what everybody thinks. That is very useful as we move forward. And I kind of consider myself to be the clean-up batter here at the end of this hearing.

Mr. Chairman, I really want to thank you and Mr. Pitts, and I want to thank Mr. Green and Mr. Pallone again. Mostly, I want to thank all of our staffs who have been really working night and day. And as I said, the best time to work

is really the weekends because there are no distractions. So it has been really great.

And, Dr. Hudson, Dr. Woodcock, and Dr. Shuren, you and your staffs have just been tremendous in giving us technical assistance. So that is the good news. The even better news from my perspective is we are going to have a lot more work to do here moving forward in the next few weeks, but I think the amount of consensus that we have is striking and positive. We still have a lot of those brackets in our discussion draft, and a lot of that is just hammering out language that we still need to agree on, but I am here to report that Chairman Upton is planning subcommittee and full committee markups soon. He wants to keep the momentum of this bill going, and so we really are going to have to redouble our efforts to get everything worked out. We have to get it scored, we have to find the money to do what we are going to do. I know a lot of people ask me, well, how could we possibly spend the money, and I said, because we need to. And I think that is the general view on both sides of the aisle, it is the general view in the patient community, and among the Administration, and, low, we are doing it here. We still need to find a way to fund the FDA for the things that we are asking you to do, and we know that. So we are going to do all of that. We also, as we learned today, need to

continue to work with members on language for issues that they care deeply about, and we are going to do that.

And so in these last few seconds that we have, I want to ask the administration, aside from resources, which we know we need to get you, what else do we need to consider that is not in this discussion draft? Dr. Hudson, I will start with you.

Ms. {Hudson.} Well, first of all, congratulations on this triumph really to get us to today, and the route ahead is really exciting. Your--the--many of the issues that we wanted to have included within this bill have been addressed. The ability of the NIH director to require data sharing, for example, the increased level of resources. There are a number of the specific provisions that we really wanted to see into the bill that are now here. There are a couple of places where we have some concerns. I mentioned some of those with the--with regard to individual patient-level data sharing mandates this early in the process, but we are very happy with where this bill stands--

Ms. {DeGette.} Great.

Ms. {Hudson.} --and I am not sure that we have any outstanding--we--probably some technical--small technical fixes, but nothing major that we are--

Ms. {DeGette.} Nothing that we have left out?

Ms. {Hudson.} No.

Ms. {DeGette.} If you think of something, let us know. And keep--

Ms. {Hudson.} We absolutely will let you know.

Ms. {DeGette.} And, of course, you will--you know, we look forward to having your input on those other issue.

Dr. Woodcock?

Dr. {Woodcock.} Well, one thing I think that I am somewhat concerned about is that children with cancer, most childhood cancers are very rare, and they are currently being left out of the precision medicine, or whatever you want to call it, targeted therapy revolution because the way we have looked at pediatric disease is we have said it is--there is a disease in adults, and then there should be a disease in children. But, in fact, in the targeted therapy, it is--there is a pathway that is targeted in adults, and then is there a pathway that is the same in children. And I think we should think about that because that is not--there is no current way to bring that about.

Ms. {DeGette.} And I will tell you, Dr. Woodcock, that is--pediatric cancer, that is an issue we have really been talking about. It is not in here because we haven't gotten to yet, and so we need help getting to that.

Dr. Hudson?

Ms. {Hudson.} Just respond quickly.

Ms. {DeGette.} Yeah.

Ms. {Hudson.} So in the Precision Medicine Initiative, there is a cancer section, and in that cancer section there is adult clinical trials and understanding resistance to oncology drugs, and there is a pediatric section for that. And we would be happy to have--

Ms. {DeGette.} So let's do some work on that.

Ms. {Hudson.} Yeah.

Ms. {DeGette.} Thank you.

Ms. {Hudson.} Absolutely.

Ms. {DeGette.} Dr. Shuren?

Dr. {Shuren.} Well, I will just say on behalf of the agency, you know, we just got the draft, we are going to go through it, and we appreciate the opportunity and would like to put that placeholder in of coming back if there are additional things that--

Ms. {DeGette.} Yeah, and that is why I said this is not just for the agency, but also for others, if they have suggestions of what they are not seeing in here, please bring them forward, again, expeditiously, because we are moving on this.

And thank you again, Mr. Chairman.

Mr. {Burgess.} Gentlelady yields back.

Chair thanks the gentlelady, and again thanks her for her patience.

I want to thank all of our witnesses today for your testimony. It has been a long morning, but I think it has been an important morning.

I do want to remind all members they have 10 business days to submit questions for the record. And I ask the witnesses to respond to the questions promptly. Members should submit their questions by the close of business on Thursday, May 14.

Without objection, the subcommittee is adjourned.

[Whereupon, at 12:52 p.m., the Subcommittee was adjourned.]