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LEGISLATIVE HEARING ON 21ST CENTURY CURES

HEARING

BEFORE THE

SUBCOMMITTEE ON HEALTH

OF THE

COMMITTEE ON ENERGY AND COMMERCE

HOUSE OF REPRESENTATIVES

ONE HUNDRED FOURTEENTH CONGRESS

FIRST SESSION

APRIL 30, 2015

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[1] Dr. Woodcock did not offer an oral statement.

[2] Dr. Woodcock submitted a joint statement with Dr. Shuren.

[3] Dr. Woodcock and Dr. Shuren did not answer submitted questions for the record by the time of printing.

[4] The information has been retained in committee files and also is available at *http://docs.house.gov/meetings/IF/IF14/20150430/103400/BILLS-114pih-DiscussionDraft.pdf.*

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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, DC.

The subcommittee met, pursuant to call, at 10:01 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, Luján, Schrader, Kennedy, Cárdenas, 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 the Economy; Adrianna Simonelli, Legislative Associate, Health; Heidi Stirrup, Policy Coordinator, Health; John Stone, Counsel, Health; Traci Vitek, Detailee, HHS; 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.

OPENING STATEMENT OF HON. JOSEPH R. PITTS, A REPRESENTATIVE IN CONGRESS FROM THE COMMONWEALTH OF PENNSYLVANIA

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 dis-

covery, 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 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 three witnesses today for their assistance throughout this process and their testimony today.

[The prepared statement of Mr. Pitts follows:]

PREPARED STATEMENT OF HON. JOSEPH R. PITTS

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.

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On that note, I would like to specifically thank our three witnesses today for their assistance throughout this process and their testimony today.

[The discussion draft has been retained in committee files and also is available at *http://docs.house.gov/meetings/IF/IF14/20150430/103400/BILLS-114pih-DiscussionDraft.pdf.*]

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

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.

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.

Mr. PITTS. Chair thanks the gentlelady.

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

OPENING STATEMENT OF HON. GENE GREEN, A REPRESENTATIVE IN CONGRESS FROM THE STATE OF TEXAS

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 and for the countless hours of work they put in to help us in this effort. It has been 1 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 of effective, innovative treatments.

The NIH, the world's leading research institution, 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 life 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 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, and 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 Congressman Shimkus and I are working on to provide a streamlined approval 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 to the global crisis of antibiotic resistance. We are working hard to incorporate 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 on with Congresswoman Blackburn for a cou-

ple 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 is fundamental in realizing the goals of the 21st Century Cures Initiative, and an interoperable healthcare system can advance and facilitate research and dramatically improve patient care and safety.

I thank my colleagues for their commitment. 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:]

PREPARED STATEMENT OF HON. GENE GREEN

Good morning and thank you all for being here today.

I particularly want to thank our witnesses and their colleagues for their expertise, and for the countless hours of work they have put in to help us in this effort.

It has been 1 year since the 21st Century Cures Initiative was launched by my colleagues, Chairman Upton and Congresswoman DeGette.

Yesterday's release of a discussion draft marked continued progress toward boosting research and delivering hope to patients.

As we know, 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 the breakthrough designation for drugs—to facility the development effective, innovative treatments.

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FDA, and Dr. Woodcock in particular, has been an incredible partner on this issue.

I thank the agency for their continued commitment to combat the global crisis of antibiotic resistance.

We are working hard to incorporate recent feedback and will have a new draft of ADAPT to share in the coming days.

The draft also includes a new version of the SOFTWARE Act, which I have been working on with Congresswoman Blackburn.

This provision will provide clarity for developers of software products used in health management and care.

Dr. Shuren and his colleagues at FDA have been instrumental to this effort, and I look forward to continuing to work with you all to foster innovation, provide regulatory certainty, and promote patient safety.

The draft recognizes the importance of improving the interoperability of health IT systems.

Interoperability is foundational to realizing the goals of the 21st Century Cures Initiative.

An interoperable health care system can advance and facilitate research, and dramatically improve patient care and safety.

I thank my colleagues for their commitment to continuing this effort.

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 to advance our health care system and live up to the goals of the 21st Century Cures Initiative.

Thank you again to our witnesses, and I yield the remainder of my time to Congresswoman DeGette.

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 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, 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.

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.

OPENING STATEMENT OF HON. FRED UPTON, A REPRESENTATIVE IN CONGRESS FROM THE STATE OF MICHIGAN

Mr. UPTON. 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 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 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. Twenty-first 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 1-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:]

PREPARED STATEMENT OF HON. FRED UPTON

First, I'd like to talk about how we got here today. These two little girls [holds up photo with the girls], my friends Brooke and Brielle Kennedy, served as an inspiration for 21st Century Cures. They are battling SMA, and are two of the brightest stars I know.

At our inaugural 21st Century Cures roundtable last spring I commented, "I think we all agree that we can always be doing more to help biomedical innovation." We've come a long way, but those words still hold true. In fact, since our launch 1 year ago today, we have heard from our colleagues in the Senate that 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.

At that first roundtable 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 released yesterday, which makes meaningful investments and still will be fully paid for, includes a number of policies that seek to answer those questions.

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unlock the wealth of health data available so patients, researchers, and innovators can communicate and keep the cycle of cures constantly moving and improving.

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I want to thank all of my colleagues who have participated in this effort, thank the patients who have shared their stories with us, as well as the administration officials, staff, and other experts. Yes, we still have work to do, but it is important to recognize the incredible progress of the 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. And it is Brooke and Brielle's time.

Mr. PITTS. Chair thanks the gentleman.

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

OPENING STATEMENT OF HON. FRANK PALLONE, JR., A REPRESENTATIVE IN CONGRESS FROM THE STATE OF NEW JERSEY

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 am 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 a $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 devel-

opment 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.

[The prepared statement of Mr. Pallone follows:]

PREPARED STATEMENT OF HON. FRANK PALLONE, JR.

Thank you Chairman Pitts. And let me thank Chairman Upton, Ms. DeGette, and Ranking Member Green. Today's hearing will examine a draft released yesterday that is the result of months of discussions. 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 am hopeful we can bring this legislation to a successful conclusion.

Let me also thank HHS for the expert advice and help along the way. I know how many resources have been spent on this effort as well, and this draft is a better product because of their guidance.

Now I would have liked Members and their staff, and our witnesses, to have had more time with the draft before a legislative hearing. The ambitious timeline has been a challenge. Iwant to be clear that I am committed to ensuring that every Member is comfortable as this process moves forward so that a final product gains broad support.

There are a large number of policies in this draft—and not a lot of time to cover all of them. But let me highlight just a few things.

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 a $1.5 billion increase in NIH discretionary authorization over the next 3 years. 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.

While this a great development, I hope that we can also ensure that FDA has the needed resources to implement the many additional policies put forth in this draft. We cannot divert already scarce resources nor impede the progress FDA has already made to advance the development and review of medical products.

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. I appreciate the chairman's commitment to me to continue to discuss this policy and ensure that it is targeted only to where it is needed.

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.

As I've said since I became Ranking Member, I am serious about finding common ground on important issues. True bipartisanship is critical to achieving successful and broadly supported policies. I am confident that this much improved collaborative process can continue. There is still much more work to be done, but today's hearing is an important step and I look forward to our continued partnership on this initiative.

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

So I will yield now to the gentlewoman from Illinois.

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 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.

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 who 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 re-

duce 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.

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 appears at the conclusion of the hearing.]

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.

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

STATEMENT OF KATHY HUDSON

Dr. 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 Initiative.

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 re-

searchers, 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 Dr. Hudson follows:]

DEPARTMENT OF HEALTH AND HUMAN SERVICES

NATIONAL INSTITUTES OF HEALTH

21st Century Cures

Witness before the

House Energy and Commerce Committee

Subcommittee on Health

Kathy Hudson, Ph.D.

Deputy Director for Science, Outreach, and Policy, National Institutes of Health

April 30, 2015

Good morning, Chairman Pitts, Ranking Member Green, and distinguished Members of the Subcommittee. My name is Kathy Hudson and I am the Deputy Director for Science, Outreach, and Policy at the National Institutes of Health (NIH).

I want to thank the Members of this Subcommittee for your hard work over the past year on the 21st Century Cures Initiative and for holding this hearing today. It is an honor to appear before you, alongside my colleagues from the Food and Drug Administration (FDA), to discuss how we, as a nation, can accelerate the pace of medical breakthroughs in the United States and get cures to patients faster.

As the nation's premier biomedical research agency, NIH's mission is to seek fundamental knowledge about the nature and behavior of living systems, and to apply that knowledge to enhance human health, lengthen life, and reduce illness and disability. All of us at NIH believe passionately in this mission, and are dedicated to the pursuit of knowledge and, ultimately, cures.

NIH has been advancing our understanding of health and disease for more than a century. Scientific and technological breakthroughs generated by NIH-supported research are behind many of the improvements our country has enjoyed in public health. For example, our nation has gained about one year of longevity every six years since 1990.[1] A child born today can look forward to an average lifespan of about 78 years – nearly three decades longer than a baby born in 1900. NIH research is also making progress against specific public health threats. For example, cancer death rates have been dropping about 1 percent annually for the past 15 years; each 1 percent decline has been estimated to be worth $500 billion as a result of gains in life expectancy.[2] Meanwhile, HIV/AIDS treatment and prevention may now enable us to envision the first AIDS-free generation since the virus emerged more than 30 years ago. These are extraordinary strides—but we aim to go much further.

The President's Precision Medicine Initiative, a bold new research effort announced early this year to revolutionize how we improve health and treat disease. The proposed initiative included in the

[1] http://www.cdc.gov/nchs/data/nvsr/nvsr64/nvsr64_02.pdf.
[2] Murphy, K.M., & Topel, R.H. (2006). The value of health and longevity. Journal of Political Economy, 114(5), 871-904.

President's Fiscal Year 2016 Budget will pioneer a new model of participant-engaged research that promises to accelerate biomedical discoveries and provide clinicians with new tools, knowledge, and therapies to select which treatments will work best for which patients. Precision medicine takes into account individual differences in people's genes, environments, and lifestyles and gives clinicians tools to better understand the complex mechanisms underlying a patient's health, disease, or condition, to better predict which treatments will be most effective.

The President's Fiscal Year 2016 Budget includes $31.3 billion for NIH, an increase of $1 billion or 3.3 percent above the enacted FY 2015 level, to maintain the nation's leadership in the biomedical sciences. Other countries are expanding their support for medical research, and stable funding for NIH is an important element in America's leadership in medical research and innovation. Other crucial areas of focus and opportunity to work with this Committee include (1) facilitating scientific collaboration and innovation; (2) modernizing clinical research and data access; and (3) reducing administrative burden and increasing efficiency.

Facilitate Scientific Collaboration and Innovation

The NIH supports basic research that is fundamental to the discoveries that have long made our nation the world's leader in biomedical science. In addition, NIH funds translational research, which seeks to find ways to move basic findings toward the clinic, and clinical research, which involves the testing and evaluation of new strategies for disease management and prevention. It is crucial that scientists work together during all stages of research, and I would like to share a few examples of how NIH is encouraging collaboration and spurring innovation.

One way we are working to unravel life's mysteries is with the President's Brain Research through Advancing Innovative Neurotechnologies (BRAIN) Initiative announced in 2013. NIH is partnering with colleagues at the National Science Foundation, the Defense Advanced Research Projects Agency (DARPA), the Intelligence Advanced Research Projects Activity (IARPA), and the Food and

Drug Administration (FDA), in this effort to revolutionize our understanding of the most complicated biological structure in the known universe, the human brain. This multiyear initiative will produce a clearer, more dynamic picture of how individual cells and neural circuits interact in both time and space. By measuring activity at the scale of neural networks in living organisms, we can begin to decode sensory experience and, potentially, even memory, emotion, thought, and consciousness. Ultimately, the technologies developed within the BRAIN Initiative may help reveal the underlying pathology in a vast array of brain disorders and provide new therapeutic avenues to prevent, treat, and cure neurological and psychiatric conditions such as Alzheimer's disease, autism, schizophrenia, traumatic brain injury, and addiction.

Recent advances in genomics, proteomics, imaging, and other technologies have led to the discovery of more than a thousand risk factors for disease—biological insights that ought to hold promise as targets for drugs. But, drug development is a terribly difficult and failure-prone endeavor. To the dismay of researchers, drug companies, and patients, the vast majority of drugs entering the development pipeline never emerge as patient-ready therapies. The most distressing failures occur when a drug is found to be ineffective in the later stages of development—in Phase II or Phase III clinical studies—after years of work and millions of dollars have already been spent. A major reason for such failures is that scientists often don't know how to choose the right clinical pathway to target. If a drug is aimed at the wrong target, it won't work against the disease it was intended to treat.

With this in mind, we were thrilled to launch the Accelerating Medicines Partnership (AMP) last year. This unprecedented public-private partnership is using cutting-edge scientific approaches to identify and validate promising biological targets for therapeutics. Besides NIH, AMP partners include the FDA, ten biopharmaceutical firms, and a number of non-profits, including patient advocacy groups. This pre-competitive partnership is focusing initially on three areas of disease that are ripe for discovery: Alzheimer's disease, type 2 diabetes, and the autoimmune disorders, lupus and rheumatoid

arthritis. Costs are shared equally between NIH and the participating companies, and all data is openly shared. Through this truly innovative and collaborative approach, we believe we can learn how to treat and cure disease faster.

NIH is also working to streamline the therapeutic development pipeline through efforts at the National Center for Advancing Translational Sciences (NCATS). One example is the Tissue Chip for Drug Screening Initiative, a collaboration with the Defense Advanced Research Projects Agency (DARPA) and FDA, with a goal of improving the process for predicting whether drugs will be safe in humans.

More than 30 percent of promising medications fail in human clinical trials because they are found to have unacceptable toxicity, despite promising pre-clinical studies in animal models. The Tissue Chip for Drug Screening Initiative is developing 3-D human tissue biochips that model the structure and function of human organs, such as lung, liver, and heart. These chips will then be combined into an integrated system that can mimic complex functions of the human body. This technology will give researchers in both the public and private sectors the ability to predict more accurately how effective a therapeutic candidate will be in clinical studies, eliminating toxic and/or ineffective drugs much earlier in the development process.

Another way we are working to advance therapeutics development is through the Discovering New Therapeutic Uses for Existing Molecules program. This collaborative approach partners NIH researchers with industry to provide opportunities to reposition and repurpose partially developed therapeutic candidates for new disease indications. By using agents that already have cleared several key steps in the development process, scientists nationwide have a strong starting point to contribute their unique expertise and accelerate the pace of therapeutics development.

For example, through the New Therapeutic Uses program, a team at Yale recently partnered with AstraZeneca to obtain the drug, saracatinib, an experimental drug originally developed to fight cancer.

The drug is now showing promise against Alzheimer's disease – restoring memory loss and reversing brain problems in mouse models of Alzheimer's. Based upon these promising results, the researchers are testing saracatinib's effectiveness in humans.

Despite all the exciting work NIH is doing in the space, there are still challenges to innovation. One of the most important ways that biomedical researchers can learn about exciting breakthroughs and form collaborations is by attending scientific conferences. However, recent travel restrictions have made it difficult for NIH scientists to attend and contribute to these meetings. We appreciate this committee's interest in this issue, as well as this Congress's work to relieve NIH from some of these restrictions and help facilitate scientific collaborations that could lead to breakthroughs and cures.

Modernize Clinical Research and Data Access

The policies governing biomedical research, along with its translation and use, must be as innovative as the science we support. Today, I want to share two areas of ongoing policy evolution: enhancing data sharing and expanding the protections for participants in research.

Dissemination of research findings is fundamental to science and an inherent aspect of NIH's mission. NIH has always endeavored to ensure that, to the fullest extent possible, the results of federally-funded scientific research are made available to and are useful for the general public, industry, and the scientific community. Since 2003, NIH has articulated its commitment to data sharing through various policies and guidances, including the NIH Data Sharing Policy and, more recently, the Genomic Data Sharing Policy.

Last November, the Department of Health and Human Services and NIH released for public comment two proposals to increase the transparency of information about clinical trials through ClinicalTrials.gov, a publicly accessible database operated by the NIH National Library of Medicine (NLM). The first proposal was a Notice of Proposed Rulemaking (NPRM) that describes proposed

regulations to complete the implementation of Title VIII of the Food and Drug Administration Amendments Act of 2007, which apply to both publicly and privately funded trials of certain drug, biological, and device products regulated by the FDA. One key provision of the proposed rule is the expanding of the scope of clinical trials required to submit summary results to ClinicalTrials.gov to include trials of unapproved, unlicensed, and uncleared products. At the same time, NIH issued a draft policy that would apply the same registration and reporting requirements to *all* clinical trials funded by NIH, including both phase I trials that are not otherwise subject to the Title VIII requirements and trials of behavioral and other interventions not regulated by FDA. Both proposals aim to improve public access to information about specified clinical trials and to ensure that information about clinical trials and their results are made publicly available via ClinicalTrials.gov.

Increasingly, the scientific community and the public expect data generated with Federal funds will be shared to enable further insights to be gained, to help enhance the quality of research, to increase transparency in Federal research spending, and to improve the return on investment in research. Although data sharing is becoming a more integral part of the research process, NIH is stepping up its efforts on a policy front to advance data sharing. Explicit statutory authority allowing the NIH Director to require sharing of scientific data generated from NIH-funded grants would strengthen these efforts.

Medical advances would not be possible without the individuals who volunteer to participate in research. Patients, and their loved ones, need new and better diagnostics, treatments and prevention strategies. They want the research enterprise to move as quickly as possible. To speed the pace of research and increase efficiency, NIH has taken steps to modernize institutional review board (IRB) policies. IRBs play a critical role in assuring the ethical conduct of clinical research, and studies must be reviewed and approved by an IRB before they can begin. When the regulations for protection of human subjects were first published, most clinical research was conducted at a single institution. Since then, the research landscape has evolved, and many studies are carried out at multiple sites and within large

networks. Studies that go beyond a single site are often able to recruit more individuals from diverse populations. These multi-site studies can often generate important results in less time. However, working through IRB review at each site can delay initiation of the research without increasing the protections for the research participants. To help address that issue, NIH issued a draft policy in December 2014 to promote the use of single IRBs in multi-site clinical research studies. The draft NIH policy proposes that all NIH-funded, multi-site studies carried out in the United States should use a single IRB of record. Exceptions to the policy would be allowed if local IRB review is required by federal, state, or tribal laws or regulations or if necessary to meet the needs of specific populations. Increasing the use of single IRBs for multi-site studies will help reduce duplication of effort, speed the initiation of important research, and save time and taxpayer funds while maintaining the highest ethical standards.

Although NIH is taking many steps to protect research participants, there are some ways Congress can be of assistance. NIH supports strengthening protections for patient information, particularly individual level genomic data. Because individual-level genomic data are unique, new genomic technologies, when coupled with identifiable reference data, make it possible to identify an individual. Genomic data can reveal significant and sensitive personal information, including risks of developing conditions such as cancer or Alzheimer's disease. 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 President's Precision Medicine Initiative, move forward.

Reduce Administrative Burden and Increase Efficiency

The significant administrative burdens placed on researchers may jeopardize or delay scientific progress. In 2009, the Federal Demonstration Partnership found that, based on a survey of faculty on active federally funded research grants, these individuals spend 42 percent of their time on administrative tasks related to grant rather than on research. Given this significant finding, NIH is committed to reducing administrative burdens on researchers and appreciates the committee's interest in reducing the administrative burdens on our grantees. As a Federal research agency, we are acutely aware that to achieve our mission we must serve as effective and efficient stewards of the resources provided by the American people. We would like to work with you on ways to enhance transparency and accountability without adding new, burdensome requirements. Reducing administrative burdens and eliminating duplicative requirements will allow Federal agencies and the research community to focus resources on the most value-added activities in finding cures.

Today, I have provided you with a brief overview of some of the exciting science supported by NIH, as well as some of the challenges facing the biomedical research enterprise. With your support, we can anticipate a bright future of accelerating discovery across NIH's broad research landscape, from fundamental scientific inquiry to translational and clinical research.

This concludes my testimony, Mr. Chairman. The NIH looks forward to continue working with you as the 21st Century Cures Initiative moves forward and I welcome any questions.

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 oral statement.

Mr. PITTS. Dr. Shuren?

Dr. SHUREN. It is in the spirit of greater efficiency.

STATEMENT OF JEFF SHUREN

Dr. SHUREN. 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 anti-

biotics 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. Woodcock and Dr. Shuren follows:]

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration
Silver Spring, MD 20993

TESTIMONY OF

JANET WOODCOCK, M.D.

DIRECTOR, CENTER FOR DRUG EVALUATION AND RESEARCH

AND

JEFFREY SHUREN, M.D., J.D.

DIRECTOR, CENTER FOR DEVICES AND RADIOLOGICAL HEALTH

FOOD AND DRUG ADMINISTRATION

DEPARTMENT OF HEALTH AND HUMAN SERVICES

BEFORE THE

SUBCOMMITTEE ON HEALTH

COMMITTEE ON ENERGY AND COMMERCE

U.S. HOUSE OF REPRESENTATIVES

"Legislative Hearing on 21st Century Cures"

April 30, 2015

RELEASE ONLY UPON DELIVERY

Mr. Chairman and Members of the Committee, we are Dr. Janet Woodcock, Director of the Food and Drug Administration's (FDA) Center for Drug Evaluation and Research (CDER) and Dr. Jeffrey Shuren, Director, Center for Devices and Radiological Health (CDRH). Thank you for inviting us to testify before the House Subcommittee on Health, Committee on Energy and Commerce, regarding the Committee's 21st Century Cures (Cures) proposal. We share your desire to accelerate the development of safe and effective medical products.

We would like to thank Chairman Upton and Representatives Pallone and DeGette for reaching out to FDA over the past many months to ask for our insights on potential opportunities to reduce the costs and time involved in studying new medical products, while at the same time continuing to protect patients who will use these 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 other stakeholders. 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 (PDUFA) and the Medical Device User Fee Act (MDUFA), and appreciate the importance of ensuring that new provisions not impede or conflict with the important ongoing work pursuant to those user fee agreements.

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. FDA's drug review times are consistently faster than all other advanced regulatory authorities

around the world, and American patients are the first to receive innovative new drugs more often than patients in other countries. In 2014, FDA approved the largest number of new drugs in almost 20 years, including more drugs for rare diseases and more new therapeutic biological products than ever before, and the greatest number of new drugs approved for "orphan" diseases since Congress enacted the Orphan Drug Act over 30 years ago.

Since 2011, FDA has made meaningful progress in reducing review times for devices approved or cleared through the 510(k) and Premarket Approval (PMA) processes, and continues working to further reduce the total time to review medical devices while maintaining standards for approval that the American public and the global population depend upon.

We appreciate the opportunity to provide input throughout the development of this legislation. As we have previously indicated to the Committee, we believe there are opportunities to accelerate medical product development by:

1. Supporting patient-centered medical product development. We are pleased that you have included provisions to help us incorporate patients' voices into FDA's decision-making regarding the benefits and risks of new products;
2. Encouraging development and qualification of biomarkers;
3. Utilizing real-world evidence in the review process;
4. Reducing barriers to use of central IRBs for device trials; and
5. Strengthening FDA's ability to hire and retain highly qualified experts.

We are encouraged that these themes have been addressed in this legislation and look forward to providing additional feedback on specific proposals as we evaluate the details of this draft.

We also intend to work with the Committee on the provisions of the draft that remain placeholders. Among the additional areas where we have appreciated the opportunity to work with the Committee on the draft provisions and look forward to our continued discussions is the issue of biomarker development. FDA supports the development and use of biomarkers in the review process. We appreciate that the Committee has been working with FDA and stakeholders to refine this section of the draft. We look forward to continuing to work with you to ensure that this language supports the qualification of biomarkers and other drug development tools without diverting resources from drug review activities.

FDA also appreciates 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, testing, and treatment of patients. In addition, FDA recognizes the interest of manufacturers in communicating with health insurers about health care economic information and is evaluating this new language. FDA shares the Committee's goal of 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 look forward to continuing to work with the Committee on issues, including the inclusion of a branding element within the labeling of such products that will alert the health care community that these products are special, and should be treated as such, and provisions related to meetings and agreements.

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, while ensuring the safety and effectiveness of products, allowing for FDA's efficient review of drugs and medical devices. 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 from FDA.

Mr. PITTS. Thank you. All right, we will begin questioning.

And 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 for patients. One patient's experience doesn't represent the experience of everyone who 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 for 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 were used 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 as 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?

Dr. 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. This 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 complementary to actions that the FDA has 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. OK. Basic research and translational research are critical to scientific advancement. Dr. Hudson, we heard that certain modifications to 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 Sciences', 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 Transactional Authority will help advance scientific research?

Dr. HUDSON. Thank you very much for the question. So NCATS, the National Center for Advancing Translational Sciences, 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. OK. 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 in the 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 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 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. OK. 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.

Mr. UPTON. 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, 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?

Dr. 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——

Mr. UPTON. Do you know about what share of that money comes back?

Dr. 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.

Mr. UPTON. We are just thinking that as we try to make sure that you have a steady stream, and one that is going up——

Dr. HUDSON. Yes.

Mr. UPTON [continuing]. 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 directly for research seems to me, a better thing.

Dr. HUDSON. Um-hum.

Mr. UPTON. 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.

Mr. UPTON. And I want to give you a backhanded 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, 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 de-

velopment initiatives, including efforts to improve effectiveness and to help ensure proper stewardship of antibiotics in our healthcare system?

Dr. 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 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 [continuing]. 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, in a 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 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?

Dr. 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——

Dr. HUDSON. Um-hum.

Mrs. BLACKBURN [continuing]. 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?

Dr. 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. Yes. 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.

Dr. 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. OK. Now, let me ask you this.

Dr. HUDSON. Yes.

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?

Dr. 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 Clinicaltrials.gov, which is now being upgraded and implemented in full force, there is a requirement——

Mrs. BLACKBURN. OK, my time is expiring, and I want a fuller answer on this, and I know——

Dr. HUDSON. I look forward to providing that.

Mrs. BLACKBURN [continuing]. You would like to give it.

Dr. HUDSON. But I think that what 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.

Dr. 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 review for everything because of 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 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 cre-

ates 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 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?

Dr. 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.

Dr. 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?

Dr. 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. OK. 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?

Dr. 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 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, 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?

Dr. 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?

Dr. HUDSON. Go ahead.

Dr. WOODCOCK. Yes. 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 [continuing]. 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 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. Yes, 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 regulated and any changes the manufacturer makes—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 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, 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 ½ 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.

Dr. 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 innova-

tive 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——

Dr. HUDSON. Yes.

Mr. KENNEDY [continuing]. 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 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, 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 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 [continuing]. Gentleman from Illinois, Mr. Shimkus, 5 minutes for questions.

Mr. SHIMKUS. Thank you, Mr. Chairman. 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, 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 market, getting to patients, but still be 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 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.

Yes, 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. Yes, we probably can't do enough to get this crisis addressed. We are doing more under GAIN. GAIN was very helpful. We thank you. 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, 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 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?

Dr. 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 CDER 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 targeted 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 precision medicine more broadly available by understanding the genetic basis of these.

Ms. CASTOR. OK, 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?

Dr. 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?

Dr. 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, as I have said many times, I believe there is somewhat of a gap between the basic discovery of these and 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 of 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.

Dr. 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. OK. We look forward to working 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?

Dr. 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.

Dr. HUDSON [continuing]. 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 Chairman Pitts and Chairman Upton and 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 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?

Dr. 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-reviewed 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 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 a half, 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 I only get ancestral information from 23andMe? It is great to know my mother was descended from Jesse James—I always suspected that—but actually it would be more useful if 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 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 break-

through populations, and will this be part of your addressing this bill?

Dr. 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.

Dr. HUDSON [continuing]. 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 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, many 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 [continuing]. 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 [continuing]. 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. Yes, 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. 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?

Dr. 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 appears at the conclusion of the hearing.]

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 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?

Dr. 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 physi-

cians. 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?

Dr. HUDSON. So thank you for your interest in Clinicaltrials.gov. 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 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?

Dr. 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?

Dr. HUDSON. So certainly, the inclusion of the ages that are sought for inclusion within clinical trials——

Mrs. CAPPS. Right.

Dr. HUDSON [continuing]. 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.

Dr. HUDSON [continuing]. 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 us, 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?

Dr. 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.

Dr. HUDSON [continuing]. 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?

Dr. 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——

Dr. HUDSON. Yes.

Mr. GRIFFITH [continuing]. 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.

Dr. HUDSON. Yes.

Mr. GRIFFITH. So we need to make sure it gets out there.

Dr. HUDSON. Yes. 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.

Dr. HUDSON. Yes.

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

Dr. HUDSON. Yes.

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

Dr. HUDSON. Yes.

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——

Dr. 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——

Dr. HUDSON. Yes.

Mr. GRIFFITH [continuing]. 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.

Dr. HUDSON. May I comment? So——

Mr. GRIFFITH. Yes.

Dr. HUDSON. So that provision specifically requires that NIH or the Secretary contract to an outside entity——

Mr. GRIFFITH. Um-hum.

Dr. HUDSON [continuing]. 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 havej 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——

Dr. HUDSON [continuing]. Particular subsets.

Mr. GRIFFITH [continuing]. 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 appears at the conclusion of the hearing.]

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 Cures 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 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 de-

vices and the statutes governing drugs were put in place a long time ago, and they didn't really contemplate, I think, these new products, which are probably part of the future of medicine. And so we are working very hard to try and 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 gulf 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 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 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?

Dr. 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, 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 might work—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? We will start with Director Hudson, and then Dr. Woodcock.

Dr. 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. Yes, 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 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 OK.

Dr. WOODCOCK. 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 in-

clude 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. It 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. 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. 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?

Dr. 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 [continuing]. 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 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. 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, it's just a factual statement about the drug approval and the indication.

Mr. LONG. OK. 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 regulating 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. OK.

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 reevaluating 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. OK, 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. OK, 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. As you said, there have been caps on the hiring authorities, there are caps on how much we can pay the people, there are actually caps on 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 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 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 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. Yes. 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 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, 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, and so there are so

many safeguards and everything, 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 [continuing]. 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 eternally 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, lo, 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.

Dr. 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.

Dr. HUDSON [continuing]. 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?

Dr. HUDSON. No.

Ms. DEGETTE. If you think of something, let us know. And keep——

Dr. HUDSON. We absolutely will let you know.

Ms. DEGETTE. And, of course, 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 there is a disease in adults, and then there should be a disease in children. But, in fact, in the targeted therapy, 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 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?

Dr. HUDSON. Just respond quickly.

Ms. DEGETTE. Yes.

Dr. 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.

Dr. HUDSON. Yes.

Ms. DEGETTE. Thank you.

Dr. 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. Yes, 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.]

[Material submitted for inclusion in the record follows:]

Statement from the American Health Care Association

21st Century Cures Initiative

4/30/2015

The American Health Care Association (AHCA) is the nation's largest association of long term and post-acute care providers with more than 12,000-plus members who provide care to approximately 1.7 million residents and patients every year. Members include not-for-profit and proprietary skilled nursing facilities, assisted living communities, and residences for persons with developmental disabilities.

AHCA commends the Committee for its work on the 21st Century Cures initiative to advance medical innovation and promote patient centered care. Unfortunately, the document contains a provision that has the unintended potential to harm the beneficiaries we serve in our facilities.

AHCA strongly recommends long term care pharmacies who serve Part D beneficiaries living in long term care facilities are exempted from the lock-in provision contained in the latest draft. Access to medications is highly controlled in long term care facilities. Long term care facilities are required by federal regulations to have medications packaged, labeled and stored according to specific conditions and medication therapy started on a timely basis appropriate for the patient's needs. Multiple medication management systems can lead to adverse events including medication errors and inefficient use of clinical resources that deters staff time from providing needed hands on care to long term care facility residents or patients.

Many pharmacies cannot meet the federal requirements for medication management in long term care facilities. Long term care pharmacies are adept at meeting these specialized regulatory requirements. Locking in a long term care facility resident or patient to a non-long term care pharmacy would present the following risks to the beneficiary and to the facility:

- Medication delivery by mail versus controlled delivery by long term care pharmacy.
- Medication delivery with significant processing time (ex. by mail) versus scheduled delivery by long term care pharmacy.
- Medications dispensed in bulk bottles versus unit dose medication management system that offers additional verification of medications that helps decrease errors.
- Medications dispensed in large supply such as 90 days' supply versus 30 days' supply from long term care pharmacy which presents risk and potentially unnecessary medication costs.

- Long term care facilities perform critical medication control procedures where nursing staff are required to count medications on a scheduled basis. Manually counting bulk medications in large quantities is very time consuming compared to unit dosed controlled substances in much smaller quantities that are typically dispensed by a pharmacy dedicated to servicing long term care facilities.

The lock-in provision would unnecessarily restrict access and delay obtaining needed medications for long term care facility residents or patients which could cause adverse events or unintended negative health outcomes for individual beneficiaries. Long term care pharmacies should be exempted from this lock-in provision. Thank you for your consideration.



STATEMENT FOR THE RECORD

House Energy and Commerce Committee, Subcommittee on Health:
"Legislative Hearing on 21st Century Cures"

April 30, 2015

Dear Chairman Upton, Representative DeGette, Ranking Member Pallone, Chairman Pitts, and Representative Green:

The Healthcare Leadership Council (HLC), a coalition of chief executives of the nation's leading healthcare companies and organizations, appreciates your efforts and the bipartisan work toward the release of the second draft of the "21st Century Cures Act" by the House Energy and Commerce Committee.

HLC strongly supports the effort to modernize the discovery, development and delivery of innovative treatments and cures to patients nationwide and is pleased to see this effort led on a bipartisan basis by Energy and Commerce Committee Chairman Fred Upton (R-MI), Oversight and Investigations Subcommittee Ranking Member Diana DeGette (D-CO), full committee Ranking Member Frank Pallone, Jr. (D-NJ), Health Subcommittee Chairman Joe Pitts (R-PA), and Health Subcommittee Ranking Member Gene Green (D-TX). HLC and its members have actively worked to support many sections of this draft legislation.

For example, HLC is pleased by the inclusion of provisions designed to speed the clinical trial process and reduce unnecessary administrative burdens. While the provisions on expanding the sharing of data generated through NIH-funded research are important, HLC strongly believes that any effort to accelerate treatments and cures must include robust data sharing from the Centers for Medicare and Medicaid Services (CMS), as well.

In testimony before the House Energy and Commerce health subcommittee last summer, on existing barriers to developing and communicating medical evidence, HLC testified that in order to advance health system improvements and medical research, health data held by the federal government should be shared more freely with organizations working to treat patients and develop new treatments and cures. Any

standard that restricts access to critical federally-held data is detrimental to our shared goals for medical and human progress. We hope future drafts include provisions encouraging greater data sharing with the private sector by federal agencies.

HLC is pleased to see the Energy and Commerce Committee address modernization of research restrictions related to the Health Insurance Portability and Accountability Act (HIPAA). We believe that medical privacy laws are serving the public well, but minor updates are necessary to enabling optimal use of today's health databases. HLC's testimony to Congress encouraged members to keep in mind that HIPAA was created at a time in which policymakers weren't thinking about the knowledge that could be gained by accessing data residing in large databases and the technological ability to process that data very rapidly. It is appropriate to adjust the authorization components of HIPAA to ensure that data can be used effectively for research.

Throughout the 21st Century Cures Initiative process, HLC has emphasized the importance of telehealth and electronic health record interoperability for inclusion in the draft legislation. We strongly support the Energy and Commerce Committee's continued work on these sections for inclusion in the final legislation.

HLC CEO members, who are leaders in every healthcare field, have agreed on the need for an interoperable health IT infrastructure constructed in a way that is both beneficial to consumers and realistic and sustainable for industry. In a September 2014 statement (attached), all HLC members endorsed a role for policymakers in encouraging the exchange of material and meaningful health data and in decertifying electronic health record products that knowingly block information. We will continue to work with Congress to finalize draft language addressing these critical issues in the 21st Century Cures Act.

We look forward to seeing the provisions on telehealth, and are pleased that work continues on a bipartisan basis. Telemedicine legislation is essential to equip healthcare providers with the tools needed to ensure they can meet the demands of an innovative healthcare system, dramatically changing patient demographics, and engaging patients in the prevention and management of their chronic diseases. HLC's multisector, consensus principles on workforce identify telehealth as a top priority, and stress the importance of significantly addressing the current restrictive reimbursement, licensure, and other regulatory barriers that make it challenging to employ telehealth effectively.

Thank you for your continued leadership on issues critical to millions of Americans. We appreciate the opportunity to work with you as you continue to develop this legislation.

The opportunity to accelerate cures and treatments for those most in need is one we all must embrace and advance.

Sincerely,



Mary R. Grealy
President

Attachment



STATEMENT ON INTEROPERABILITY AND EXCHANGE OF PATIENT INFORMATION

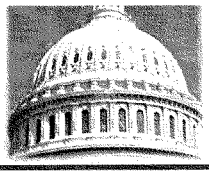
The Healthcare Leadership Council (HLC) has long served as the innovative voice of healthcare in the United States. HLC members recognize the increasing importance of efficient, timely transfer of patient information throughout the healthcare system, enabling care to be delivered to the patient more quickly and guided by meaningful data. We believe in a future in which health organizations work not as "silos" of information, but as an interoperating health system using shared data to accelerate progress in medicines, technologies, and healthcare delivery.

HLC CEOs, who are leaders in every healthcare field, have agreed upon the need for an interoperable health IT infrastructure that takes shape in a way that is both beneficial to consumers and workable for industry. It is our hope that these recommendations support the work of Congress, the administration, and other organizations working to create the health system of the future.

- We believe that policymakers should **encourage exchange of material and meaningful health data** through the use of technologies and applications that enable bidirectional and real-time exchange of health data currently residing in electronic health record (EHR) systems (e.g., open and secure API technology).
- Policymakers should also use appropriate authority to **certify only those EHR technology products that do not block or otherwise inhibit health information exchange**. The HHS Office of the National Coordinator should decertify "Meaningful Use" products that intentionally block the sharing of information, or that create structural, technical, or financial impediments or disincentives to the sharing of information.
- The federal government, in collaboration with the private sector, should **build on current and emerging best practices in patient identification and matching** to identify solutions to ensure the accuracy of every patient's identity, and the availability and accessibility of their information, absent lengthy and costly efforts, wherever and whenever care is needed.
- Any interoperability **requirements or incentives should be "technology neutral" and focused on outcomes**—active interoperation between and among systems—rather than on adoption or use of specified technologies. It is critical that future policies do not stifle potential innovations in health system connectivity.

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(Alphabetized by Company)



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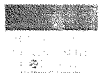
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House Energy and Commerce Health Subcommittee Hearing Statement
21st Century Cures
Charles Jaffe, MD, PhD
CEO, Health Level Seven (HL7)
April 30, 2015

Health care interoperability provides an important foundation for achieving the 21st Century Cures goal to accelerate the pace of cures in America and for creating a robust, patient-centered learning health system in our nation.

HL7 welcomes the chance to provide its suggestions on the 21st Century Cures Initiative today. Our organization -- founded nearly three decades ago -- has a wealth of standards experience to contribute to this effort. As the global authority on health care interoperability, HL7 is uniquely positioned to inform the interoperability debate and assist in achieving this critical goal.

- HL7 builds the interoperability highway for delivering 21st century cures. Over the past 30 years, our standards have become synonymous with the international roadways, the interchanges, and the bridges to arrive at healthy lives. Adopted worldwide, our standards have created innovation and cost-effectiveness for the continuum of health care from providers, to patients, and to public health.

- HL7 is continuing to develop and enhance standards at the heart of the health care system as well as those that offer the promise of a more efficient and information-rich future for providers and patients. Its new vehicle, FHIR (Fast Healthcare Interoperability Resources) is but one of them.

- HL7 originally created FHIR and Consolidated Clinical Document Architecture (C-CDA), both of which are cited in the Version 1.0 *Interoperability Roadmap* by the ONC as foundational for critical interoperability advancements in the near-term.

- And, HL7 is a cornerstone of Meaningful Use and interoperability, as well a key driver of the most promising of private sector innovations for achieving it.

Our organization is engaged in a sustained, bipartisan effort to educate Congressional leaders about necessary elements of workable and meaningful interoperability legislation. As part of this effort, HL7 submitted written comments on the first generation legislative draft that emphasized that the 21st Century Cures legislation must sufficiently address and support. These elements include:

Reliable and Sustained Funding: A reliable funding stream to support the private sector interoperability work of standards development organizations (SDOs) in order to meet increased and timely demands for the technical expertise to develop and refine interoperability standards. Sustained Federal support, over a period of four or more years, will be needed to ensure that

SDOs have sufficient resources to enhance the private sector's capacity to meet increased demands and to quickly ramp up for them.

Critical Interoperability Activities: Interoperability activities spearheaded by health care SDOs lead to clinical improvements and technological innovations in the health care marketplace. These include standards development, robust testing and piloting, and implementation.

Strong, Rational Standards Development Processes: Standards development processes provide the technical means to successfully achieve interoperability. A standards development process that is: (1) open, (2) transparent, (3) consensus-based and (4) subject draft standards testing for success and user acceptance including, but not limited to, piloting in appropriate venues and by the appropriate providers and other users.

Efficiently Leveraging Interoperability Experts: Consultation with representatives of relevant federal agencies and private sector clinical and technical experts with expertise provide the needed momentum to establish indispensable health care interoperability.

Equally importantly, HL7 is leading the way in private sector innovation. The highly regarded Argonaut Project was launched in December 2014 with the goal of accelerating the development and adoption of HL7's Fast Healthcare Interoperability Resources (HL7® FHIR®). HL7's FHIR is a next generation standards framework that leverages the latest web standards and offers enormous flexibility for patients and providers. Its versatility can be applied to mobile devices, web-based applications, cloud communications, and EHR data sharing. A list of current Argonaut project members can be accessed at: http://bit.ly/1zjMcWj.

As a global, national, public and private sector leader, HL7 welcomes the opportunity to work with House Energy and Commerce Chairman Fred Upton (R-MI), Ranking Member Diana DeGette (D-CO) and Health Subcommittee Chairman Joe Pitts (R-PA) as the 21st Century Cures initiative advances forward.

NACDS NATIONAL ASSOCIATION OF CHAIN DRUG STORES

Statement

Of

The National Association of Chain Drug Stores

For

United States House of Representatives
Committee on Energy and Commerce
Subcommittee on Health

Hearing on:

"21st Century Cures Bill"

April 30, 2015
10:00 a.m.
2123 Rayburn House Office Building

National Association of Chain Drug Stores (NACDS)
1776 Wilson Blvd, Suite 200
Arlington, VA 22209
703-549-3001
www.nacds.org

The National Association of Chain Drug Stores (NACDS) thanks Chairman Pitts, Ranking Member Green, and members of the Subcommittee on Health for the opportunity to share our perspectives the 21st Century Cures Bill. NACDS represents traditional drug stores and supermarkets and mass merchants with pharmacies. Chains operate more than 40,000 pharmacies, and NACDS' 125 chain member companies include regional chains, with a minimum of four stores, and national companies. Chains employ more than 3.8 million individuals, including 175,000 pharmacists. They fill over 2.7 billion prescriptions yearly, and help patients use medicines correctly and safely, while offering innovative services that improve patient health and healthcare affordability. NACDS members also include more than 800 supplier partners and nearly 40 international members representing 13 countries. For more information, visit www.NACDS.org.

NACDS supports the mission of the 21st Century Cures Initiative to accelerate the discovery, development and delivery process of new drug treatments for patients. As the face of neighborhood healthcare, retail pharmacies can play an important role in the delivery of new lifesaving drug treatments that result from the 21st Century Cures Initiative. NACDS member pharmacies play a leading role in expanding patient access to care and treatment, which positions our members to be key players in dispensing new drugs, as well as providing new services that include emerging cures. Within the context of the 21st Century Cures Initiative, community pharmacy's value is amplified through the growing efforts of the industry to provide health education and disease state testing and management. Through personal interactions with patients and face-to-face consultations, pharmacies are helping to shape the healthcare delivery system of tomorrow, in partnership with doctors, nurses and others. We look forward to working with the Subcommittee in developing policy and legislative ideas to ensure the success of the 21st Century Cures Initiative. We offer the following comments for your consideration.

Section 3151, "Establishing a PDP Safety Program to Prevent Fraud and Abuse in Medicare Prescription Drug Plans

NACDS urges a deliberative and thoughtful approach to implementing policies restricting access to prescription medications. While we share the legislation's goal of combatting prescription drug abuse and diversion, we also believe that any potential programs must ensure legitimate beneficiary access to needed medications is not impeded. Policies to reduce overutilization must be balanced with maintaining access to prescription medications by the beneficiaries who need them most. Protections should be in place to allow a pharmacy, in consultation with the prescriber, to fill legitimate prescriptions without needlessly delaying treatment for beneficiaries.

NACDS believes access can be maintained by ensuring patients have the ability to select one or more pharmacies that best meet their needs. In selecting their pharmacy, beneficiaries should have the option to select a location, or number of locations under common ownership that electronically share a real time, online database, which are licensed by the respective State Board(s) of Pharmacy to dispense prescription drugs to a beneficiary. This will allow a beneficiary to obtain their medications from a number of locations for a particular pharmacy, so long as that pharmacy uses a common database that would ensure medications are not inappropriately dispensed. Similarly, processes must be in place to ensure that beneficiaries can quickly change their selection when they move. We believe these beneficiary protections would reduce barriers to access while maintaining the integrity of safe pharmacy networks.

Conclusion

NACDS thanks the Subcommittee for consideration of our perspectives on the 21st Century Cures Bill. We appreciate the opportunity to work members of Congress, as well as other policymakers, to promote the health and welfare of our patients and all Americans.



Support Common Sense Regulation of Cord Blood Units:

21st Century Cures Legislation Should Require the FDA To Establish Good Collection, Storage, and Maintenance Practices that Recognize the Unique Nature of Cord Blood Banks

It is common for FDA regulations to be criticized for stifling innovation and adding unnecessary costs. In the case of umbilical cord blood banking, both criticisms are valid. But even more important, current regulations threaten public health by limiting access to cures for serious diseases. The regulations do this by limiting the growth of the national cord blood inventory and causing the needless destruction and disposal of viable tissues. The challenge is licensure requirements that are illogical or based on outdated science.

Our Credentials

The National Marrow Donor Program/Be the Match (NMDP) supports the Committee's efforts to improve the current federal regulatory framework. As the contractor for the C.W. Bill Young Cell Transplantation Program (Program), we understand first-hand the importance of ensuring an efficient and effective pathway for developing innovative treatments. With our partners throughout the world, we have sought to minimize the burdens that can make it difficult for patients to access bone marrow and cord blood transplants. Because of the efforts of physicians, patients and their families, researchers, and the support of the Congress, these cellular transplants have led to the development of treatments and cures for more than 60,000 patients with over 70 blood diseases and genetic disorders. But there is still more that needs to be done.

Cord Blood – It's Not a Drug

Many federal regulations have not kept pace with innovation, which has resulted in a gap between the science of cures and how it is regulated. This is especially true for bone marrow and cord blood transplantation. One of the most difficult barriers to access relates to the recent implementation of licensure requirements for public cord blood banks. The licensure process seeks to regulate cord blood units as if they were drugs, which they are not. Simply put, the current licensing structure does not recognize that the collection, storage, and maintenance of cord blood units is different than the manufacturing process used to create biologics, drugs, and other pharmaceutical products.

This disconnect between the science and the regulations creates a significant burden on cord blood banks, which has led to a slowing of growth of the national cord blood inventory, and has also significantly increased the cost of each unit that

is used in transplantation. Furthermore, it has resulted in many units being needlessly wasted each year either because of disqualification for reasons that do not affect the quality of the cord blood unit or for stability studies which, per the FDA, require use of actual clinical product.

Government Promotion of Cord Blood

Like bone marrow, cord blood can be used to treat and/or cure more than 70 malignant or genetic diseases. In 2005, the Congress formally recognized the importance of collecting, storing, and maintaining an inventory of publicly banked cord blood units by creating the National Cord Blood Inventory (NCBI). The units in the NCBI are listed on the national registry (known as the Be The Match Registry) and available for patients unable to find a matched related or unrelated adult donor.

Through the NCBI, the Health Services and Resources Administration (HRSA) provides grants to cord blood banks that meet certain qualifications to subsidize the costs of collection and storage of public cord blood units. Both the registry and the NCBI are part of the C.W. Bill Young Cell Transplantation Program. It is important to note that each cord blood unit represents a separate and unique 'batch' of product and each cord blood unit is a potential unique match for an individual patient in need. Thus, each banked cord blood unit is a highly valued product.

Currently, licensure is only a requirement for public cord blood banks. Five of the 13 banks in the NCBI have been granted license in the past three years. Others are in the process of applying for their licenses. The licensure regulations are more stringent than those that apply to other blood, hematopoietic stem cell and blood products. The applicable laws and regulation for cord blood licensing, include:

- Public Health Services Act, Section 351, which establishes the licensure requirements for biologic licensing;
- The Current Good Manufacturing Practice for Finished Pharmaceuticals regulations, 21 C.F.R. Pt. 211;
- The Biological Products general regulations, 12 C.F.R. Pt. 600;
- The Biological Licensing regulations, 12 C.F.R. Pt. 601; and
- The General Biologics Product Standard regulations, 21 C.F.R. Pt. 610.

In addition, the FDA issued final guidance in 2009 for "Minimally Manipulated, Unrelated Allogeneic Placental/Umbilical Cord Blood Intended for Hematopoietic Reconstitution for Specified Indications." The FDA updated this guidance most recently in 2014.

Regulation at Cross Purpose with Legislation

While the intent of requiring licensure is to assure the public that the cord blood units are safe and effective, the way it has been implemented has created significant barriers that increase the cost of cord blood units, stifled innovation, and made it more difficult for patients to access these types of cells for transplantation.

The primary problems with the regulation of cord blood banking relate to the FDA's conclusion that collecting and storing cord blood units is the equivalent of manufacturing a pharmaceutical drug. This conclusion imposes a greater burden on banks without meaningfully addressing safety and effectiveness. In essence, the FDA has tried to put a square peg into a round hole and it clearly does not fit. The FDA recognizes that cord blood units do not fit into the precise mold (labeling cord blood units as intermediate products), yet requires compliance anyway. "While there are no specific regulations governing the manufacture of intermediates, drug substances or what are termed active pharmaceutical ingredients, compliance with statutory cGMP (section 501(a)(2)(B) of the FDCA) is required."[1]

The following problems illustrate many that stem from this approach.

1. Barriers to making improvements. The cGMPs establish strict procedural and timing requirements before a manufacturer can implement a change or an improvement to its processes. Cord blood banks are not manufacturing cord blood units the same way that a pharmaceutical company is manufacturing a biologic. Because of the overly strict requirements, the cGMPs inhibit innovation by limiting the ability of the banks to make necessary adjustments in their processes to recognize the unique characterization of each cord blood unit and the rapid innovation in the field. And, applying these requirements to cord blood also makes it extremely difficult to respond to unpredictable shortages of materials/devices used to collect, store, and maintain the cord blood units.

2. Unnecessary, duplicative validation. The cGMPs also require manufacturers to validate their processes *and every product used in the process regardless of whether it was subject to prior validation and clearance.* The FDA has interpreted this requirement for cord blood banks to mean that they must validate products, despite the fact that they have already approved by the FDA for cord blood collection and banking and are purchased from approved vendors by the cord blood bank for processing and testing cord blood units. The interpretation also applies to FDA-approved product for human use, such as Hespan, a volume expander commonly administered to patients in shock, which is also used in preparing cord blood units for storage. This interpretation amounts to a revalidation process that duplicates what the actual manufacturers of the products have already done. The

[1]FDA, "Minimally Manipulated, Unrelated Allogeneic Placental/Umbilical Cord Blood Intended for Hematopoietic Reconstitution for Specified Indications," 19 (2009).

step is unnecessary and adds time and cost without providing any additional benefit or improved safety.

3. Overly burdensome environmental monitoring requirements. Unlike the manufacturing of pharmaceuticals, there are a number of options for assuring safety in the manufacture of a cord blood unit other than strict requirements applied to the entire facility. For instance, the preparation of cord blood is often done in a closed system on the bench-top. The environmental monitoring requirements that apply to pharmaceutical manufacturing are unnecessarily rigorous and simply increase costs without providing benefit.

4. Required creation of an expiration date. The cGMPs also require that all manufactured products to have an expiration date. To meet this requirement, each bank must annually destroy a small part of its inventory to demonstrate that there has been no deterioration in cellular quality even though separate clinical research supporting the use of cord blood units for transplantation has indicated that the cells do not expire. Thus, the requirement for an expiration date is simply not applicable to these cells. Yet, the FDA still requires it.

5. Wasteful stability protocols. The cGMPs also require cord blood banks to use units to comply with stability protocols. These protocols are meant to analyze product potency, integrity, and sterility. Yet, clinical studies have shown that proper storage does not result in a reduction in any of these areas. Applying this requirement means that cord blood units collected using federal dollars are again being taken off the registry and sacrificed for testing to meet this unnecessary requirement. Given that there are still thousands of Americans who cannot find a match today, it does not make sense to take cord blood units, each of which has a unique tissue type, that could provide that match and use them to meet a protocol meant to apply to pharmaceutical manufacturers.

Recommendations

As currently defined, the application of the cGMPs to cord blood units creates unnecessary barriers to accessing this unique and life-saving treatment. Prior to the application of these requirements, this was an area where a strong public-private partnership supported innovation and improved the speed at which this research has been translated to cures that save patients' lives. Removing these barriers is consistent with the intent of "The 21st Century Cures Act." Thus, we encourage you to include language in the next iteration of this legislation to solve this problem.

Specifically, we recommend that a provision be added to Section 5021 in "Subtitle B—21st Century Manufacturing." As currently drafted, this provision would require the Commissioner of the FDA to update the cGMPs. We believe that this Section should be expanded to require the FDA establish good collection, storage, and maintenance practices that apply specifically to cord blood units, which recognize the unique nature of cord blood banks, and remove unnecessary,

duplicative, and costly requirements. Importantly, FDA should be directed to implement this Section in collaboration with the subject matter experts in the hematopoietic stem cell transplantation and cord blood banking communities. We would welcome the opportunity to work with you to develop specific legislative text to authorize this work.

PREMIER

Statement for the Record

Submitted by

The Premier healthcare alliance

House Energy and Commerce Subcommittee on Health

"Legislative Hearing on 21st Century Cures"

April 30, 2015

The Premier healthcare alliance appreciates the opportunity to provide a statement for the record of the House Energy and Commerce hearing, titled "*Legislative Hearing on 21st Century Cures.*" We applaud the leadership of Chairman Joe Pitts and Ranking Member Gene Green for holding this important hearing.

Premier, Inc. is a leading healthcare improvement company, uniting an alliance of approximately 3,400 U.S. hospitals and 110,000 other providers to transform healthcare. With integrated data and analytics, collaboratives, supply chain solutions, advisory and other services, Premier enables better care and outcomes at a lower cost. Premier, a Malcolm Baldrige National Quality Award recipient, plays a critical role in the rapidly evolving healthcare industry, collaborating with members to co-develop long-term innovations that reinvent and improve the way care is delivered to patients nationwide.

HIT interoperability foundational to enabling 21st Century Cures

Cost-effective, interoperable health information technology (HIT) infrastructure is foundational to advancing and expediting health research. Being able to fully leverage robust data is critical to the discovery and development of new cures, modernizing clinical trials, effective surveillance of patient safety, and improving quality and efficiency of care. Despite its potential, the current HIT ecosystem

continues to be challenging for healthcare providers and researchers alike due to the lack of interoperability.

As this committee heard in previous hearings, the current market incentives are not aligned with open exchange of necessary healthcare data in cost-effective ways. As a result, data is locked in proprietary software systems across the care and research spectrum. The interoperability challenges created by these locked systems hinder their ability to connect and exchange information with other HIT assets including EMR/EHR systems, medical devices, sensors, monitors and other information technology tools necessary for improving research, patient care, safety and efficiency. The current lack of interoperability has enormous consequences not only for research, care and safety of patients but also in terms of cost to our healthcare system. Today, to build the bridges that connect disparate data sets necessary to provide comprehensive and informed decisions or care, researchers and/or providers are forced to either pay their original system vendors thousands of dollars to custom code links so they can "talk" to other HIT assets, or do it themselves via faxing or emailing. This comes at an enormous expense, both in raw dollars and manpower.

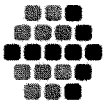
The goal should be to design and implement an HIT ecosystem that that enables secure exchange of health information in timely and cost-effective ways. It should promote collaboration among all stakeholders, creating a learning health system that focuses on improving healthcare quality, efficiency, safety, affordability and access through government and market incentives, while encouraging innovation and competition. To accomplish this goal, the federal government should incorporate the following policy principles and metrics in its implementation requirements to achieve interoperability of HIT system infrastructure:

- **Development of standards that promote interoperability and innovation:** The Office of National Coordinator, in collaboration with stakeholders and other federal agencies, should promote and facilitate the development and use of standards in key areas including: patient identifiers, terminologies, clinical data query language, security, open application program interfaces (APIs), and clinical decision support algorithms among others.

- **Transparent and public interoperability measures**: Transparent and public measures of interoperability should be developed in collaboration with standard setting bodies in consultation with the private sector and promoted through the ONC's certified technology program. These measures should be validated and tested in terms of standards, processes and within specific use case scenarios. Measures should include business and implementation approaches that deliver functional interoperability outcomes and include operational processes and implementation practices.

- **Standards and measures compliance:** Federal government should promote the enhancement of ONC's enforcement tools and certification program to ensure standards and measures compliance.

As this committee continue its work on the 21st Century Cures legislation, we urge the Members to require HIT interoperability as foundational to facilitate discovery, research, storage and use of health data in advancement of cures and patient care, and to enable further progress toward increased patient safety and higher quality care while bending the cost curve.



ALLIANCE FOR
HCPCS II CODING REFORM
[illegible]

March 12, 2015

VIA ELECTRONIC MAIL

Chairman Fred Upton
Committee on Energy and Commerce
2125 Rayburn House Office Building
Washington, DC 20515

Ranking Member Frank Pallone
Committee on Energy and Commerce
2322A Rayburn House Office Building
Washington, DC 20515

RE: Comments to 21st Century Cures Act: Suggested HCPCS Coding Process Reforms

Dear Chairman Upton and Ranking Member Pallone,

The current Healthcare Common Procedure Coding System (HCPCS) coding process for Level II alpha-numeric codes used by Medicare, Medicaid, and private health plans (particularly for durable medical equipment, orthotics, prosthetics and supplies [DMEPOS]) is not transparent, understandable or predictable. Over many years, this has created strong barriers to appropriate coverage and reimbursement for new technologies and products. The current process has a chilling effect on innovation that drives researchers and R&D investments away from DMEPOS, ultimately compromising access to quality care for millions of Medicare beneficiaries and other individuals. Although this process is administered by the Centers for Medicare and Medicaid Services, this badly flawed process impacts Medicare and all payers using the uniform code set. Reform is needed to ensure the goals of a meaningful code set are met, namely, uniformity in billing, appropriate coverage and reimbursement policies, and patient access to quality care.

Included below are recommendations for your consideration to be included in the 21st Century Cures Act when it is introduced in final form. Given the overall purpose of that proposed legislation, these recommendations for HCPCS Level II coding reform fit well within the confines of that proposed legislation. The members of the Alliance would be pleased to speak with you at your convenience about our concerns regarding the HCPCS coding process as well as about our recommendations.

The Alliance for HCPCS II Coding Reform ("Alliance") was formed in May 2008 to seek improvements to the HCPCS coding process so that it is fair, transparent, predictable, accurate, understandable, timely, accountable, efficient and independent of any individual payer's coverage and payment considerations. An improved HCPCS Level II coding process would allow meaningful consumer access to technology, regardless of payer. The Alliance is comprised of key law firms, lobbying firms, associations, coalitions, medical device companies and

reimbursement consulting companies with expertise in HCPCS coding who recognize the need to take action to reform the HCPCS coding system.

We have met over the years with the Centers for Medicare and Medicaid Services (CMS) senior staff; unfortunately, they have been reluctant to make the significant changes that would be meaningful to the process. This is why we believe that it is imperative to have legislative action on this important issue.

The fundamental problems we have identified with the current HCPCS decision process are as follows:

1. The current HCPCS Level II code set includes broadly defined codes that are ambiguous and imprecise, resulting in dissimilar technologies being lumped into the same code. This challenges coverage policy development and creates barriers to comparative effectiveness research that could provide evidence to inform improvements to coverage and policy decisions. In addition, it leads to improper payment determinations that oftentimes create barriers to access of medically necessary devices and technologies.

2. The coding process is not transparent, predictable, or timely. The criteria used to justify issuing or modifying codes are often undefined, have never been subject to public notice and comment, and seem to be applied inconsistently from year to year. In addition, there is no assurance that coding decisions give appropriate weight to scientific and clinical trial evidence that may distinguish an item or service from existing items or services with HCPCS codes. The composition of the HCPCS Workgroup at CMS has never been disclosed publicly, and the Workgroup has never included stakeholders in the decision-making process. CMS also does not allow for advance notice and stakeholder feedback when it decides unilaterally to delete or modify certain existing HCPCS codes outside the external application process. Finally, there is no reconsideration/appeal process other than resubmission of the application in the next annual coding cycle; this insulates the process from any form of accountability and causes delays of at least one year in patient access to these products.

3. The coding process improperly commingles Medicare coverage decisions with coding decisions. The factors involved in justifying creation of a new billing code are separate and distinct from the factors involved in justifying coverage of a particular device or technology to meet the needs of a specific payer's enrollees. In fact, this distinction is well-recognized in the laws and precedents that apply to the Medicare program. Nevertheless, the current process results in CMS making coverage decisions for all payers and often overlooks non-government-supported health plans that have coverage and payment policies that may be different from Medicare and serve different patient populations.

4. Outside of the HCPCS coding process (where existing codes are modified and new codes are created), the coding verification process administered by the Pricing, Data Analysis, and Coding (PDAC) contractor is also in need of reform in order for manufacturers, suppliers, and providers to obtain clear guidance on accurate coding. This coding verification process also needs to separate coverage from coding criteria and to eliminate the problems associated with the reassignment of HCPCS codes which may immediately result in change of coverage of products and technologies.

To address these significant problems with the HCPCS Level II coding process, we offer the following recommendations:

1. Recommendation: Increase Transparency of Coding Decisions.

i. HCPCS Workgroup Responsibilities: There should be a mechanism in place for each representative on the HCPCS Workgroup to obtain comments regarding HCPCS coding needs and information on the submitted applications so as to represent their constituency. Representatives should have the explicit responsibility to listen to stakeholder groups and individuals who wish to inform them of facts and circumstances involving coding decisions.

ii. Public Accountability: CMS should publish the names, affiliations, and titles of the CMS HCPCS Workgroup members. The identities of the Workgroup members should be a matter of public record and CMS should explicitly permit direct contact between coding applicants and Workgroup members throughout the year.

iii. Robust Representation on the HCPCS Workgroup: A more robust representation of Medicaid, Veterans Health Administration (VA), and commercial payers should be involved in the coding process to meet the needs of diverse populations. CMS should meaningfully engage, *throughout the entire coding process*, Medicaid, VA, and commercial payers to a greater extent to obtain their opinions on current HCPCS code applications and determine their HCPCS coding needs. CMS should clarify and formalize the process for Medicaid and commercial payers to ensure that their coding needs or program operating needs are identified and given adequate consideration by the HCPCS Working Group.

iv. Detail Reasons for Denial: Reasons for denial currently used by CMS in this process should be explained with greater specificity. To be fair, CMS has made improvements in this area over the past several years. The reasons for denial form the basis for the changes to the applicant's revised coding application for the following cycle and as a result these reasons therefore need to be sufficiently detailed to provide clarity and avoid unnecessary waste of time and resources. If CMS denies an application for a new HCPCS code, the letter should specify both the rationale for the decision not to issue a new code and explain what information the applicant needs to provide in future applications to achieve a favorable code result.

v. One-on-One Consultation: CMS should provide applicants with an opportunity to meet in person with CMS Workgroup staff before a preliminary decision is made to ensure that the HCPCS Coding Workgroup fully understands the devices and technologies being considered, and so that applicants may advance their rationale for a new code or codes.

vi. Mechanism for Applicant to Withdraw HCPCS Code Application. CMS should work with stakeholders to develop a timeline, process and circumstances under which an applicant may withdraw an application for the current HCPCS coding year.

2. **Recommendation: Clearly Separate the Criteria Used to Establish a New HCPCS Code from Criteria Used to Establish Coverage Policy.**

   i. Purge Coverage Criteria from Coding Decisions: Revise CMS's current *coding* "Decision Tree" to reflect that coding decisions are based on criteria that are separate and distinct from the criteria used to make *coverage* decisions for the same device or product. We recommend the following criteria to establish a new code. The device or product:

      1. Performs a different function (does something clinically different for the patient) than a previously coded product; OR

      2. Operates differently; OR

      3. Is a distinct technology (e.g., components, materials of construction, structural features, size, mechanism of action are distinctly different from existing technology); OR

      4. Meets a distinct patient or clinical need (e.g., there is a distinct patient population that benefits from the use of this device, or there are significant clinical indications or uses that are distinct from existing codes.)

   ii. Conformity with New Coding Criteria: CMS should be required to revise its HCPCS Coding "Decision Tree" to conform with the criteria listed immediately above and the additional suggestions below:

      1. Provide a clearer definition of what constitutes a "national program operating need" (in order to establish a new billing code) by commercial payers, Medicaid programs, as well as other payers and stakeholders by developing specific criteria to be met. We recommend revising the definition of the term "national program operating need" so that if one sector (defined as a payer, i.e., one Medicaid program, one commercial plan) supports the issuance of a new code, a national program operating need shall be recognized. To validate this request, the applicant would submit one letter from the one payer to CMS as part of the HCPCS application. In addition, the current requirement that an applicant demonstrate significant therapeutic distinction should be removed because it often comingles coverage with coding considerations; instead, the new decision tree criteria described above should be substituted.
      2. Add additional objective data to support the sales volume criteria that would demonstrate significant product demand in the marketplace such as sales trend reports and product feasibility studies. (*See* new definition for sales volume criteria.).
      3. Restrict the current practice of revising code descriptors to expand the scope of an existing code; this practice makes the coding system inaccurate and/or imprecise, leading to opportunities for abuse.

3. **Recommendation: Establish an Appeals Process to Provide Independent Review/Reconsideration of Coding Decisions.**

   i. Establish the Right to Appeal Coding Decisions: HCPCS coding applicants who receive adverse coding decisions should have a right to appeal the decision to a HCPCS Coding Appeals Board. The applicant should be granted an informal, in-person hearing with the appeals board within the 90-day period and prior to a final decision being made, providing the applicant with an opportunity to discuss the application, answer any questions, and address CMS' previous decision rationale. The appeals board should be comprised of a representative sample of individuals who serve on the HCPCS Workgroup, including Medicaid, VA, and private insurance representation as well as either the Director or Deputy Director of the CMS Chronic Care Policy Group to provide historical context and expertise to the coding decision. The board should be required to solicit external physicians and other health care professionals and suppliers with expertise in the specific subject of the coding application at issue to assist the appeals board in rendering a final coding decision. If the coding decision is changed as a result of the appeal, the new or revised code and fee schedule would be implemented in the next HCPCS quarterly update.

4. **Recommendation: PDAC Coding Verification Process Must be Improved**

   i. Proper Notice and Comment of All Coding Changes: All revisions, deletions, consolidations and changes to code criteria of HCPCS codes announced by the PDAC must first be published on the DME MAC websites and supplier publications in draft form with reasonable time for public comment before any HCPCS coding change becomes final and effective. This would not rise to the level of public notice and comment procedures under the Administrative Procedures Act.

   ii. Greater Access to the PDAC: PDAC officials should meet with coding verification applicants to discuss the product(s) at issue. In addition, key PDAC decision makers should be required to keep periodic office hours at CMS central in Baltimore, Maryland in order to permit small businesses and manufacturers to more easily engage the PDAC in coding verification discussions.

   iii. Pediatric Coding: CMS should develop a mechanism for coding verifications for pediatric products or otherwise work with Medicaid programs to eliminate the requirements for obtaining PDAC code verification. (For example, the PDAC currently declines to conduct coding verification for pediatric products.)

   iv. Coverage Information Separate from Coding: Consistent with our recommended standard for separate consideration of coverage and coding for new and revised codes, the PDAC should never use coverage information in the code verification process.

   v. Independent Reviewers for Reconsideration Appeals. Independent reviewers should be engaged during the appeals process. External physicians and other health care

professionals and suppliers with expertise in the specific subject of the coding re-verification could serve as advisors in rendering a final coding decision.

The Alliance for HCPCS II Coding Reform appreciates the opportunity to submit these comments to you for consideration of inclusion in the 21st Century Cures Act. We stand ready to meet with you to discuss these issue in more depth at your convenience. Thank you.

Sincerely,

Marcia Nusgart R.Ph.

Marcia Nusgart R.Ph.
Alliance for HCPCS Coding Reform Participants who include but are not limited to:*

John Broughton; Medela, Inc.
Grant Bagley; ADVI (formerly HillCo Health)
Kim Brummett; American Association for Homecare
Donald Clayback; National Coalition for Assistive and Rehab Technology
Jennifer Hutter; J.D. Hutter and Associates LLC
Seth Johnson; Pride Mobility Products Corp.
Stuart S. Kurlander; Latham & Watkins LLP
Robert C. McDonald; Aledo Consulting, Inc.
Marcia Nusgart; Coalition of Wound Care Manufacturers
Lynn Shapiro Snyder; Robert Wanerman; Epstein Becker and Green
Rita Stanley; Sunrise Medical
Peter Thomas; Powers, Pyles, Sutter and Verville PC
David Vermeulen; Halyard Health
Debra Wells; Wells Health Group

CC: Representative G.K. Butterfield
Representative Diane DeGette
Representative Renee Ellmers
Representative Gene Green
Representative Joseph Pitts



FOR THE RECORD

Statement
on
"Legislative Hearing on 21st Century Cures"

Senior Care Pharmacy Coalition
805 Fifteenth Street, NW
Suite 615
Washington, DC 20005

Submitted to the
House Committee on Energy & Commerce
Subcommittee on Health

April 30, 2015

I. Introduction

Established in 2014, the Senior Care Pharmacy Coalition (SCPC) is the national association for independent long-term care (LTC) pharmacies. Our members provide care and services to patients in long-term care facilities in more than 40 states and serve patients in approximately 350,000 beds across the country. The SCPC advocates for public policies that protect patients, improve the quality of healthcare across a shifting care continuum, and strengthen the economic viability of independent LTC pharmacies crucial to and their ability to serve medically-compromised seniors.

LTC pharmacies—sometimes called "closed door" or "institutional" pharmacies—are a distinct subset within the pharmacy community. All skilled nursing facilities (SNFs) and many assisted living facilities (ALFs) contract with a single LTC pharmacy to provide prescription drugs and an array of consulting pharmacy and care planning services required by Medicare, Medicaid, state licensure laws, and professional standards.

Requirements imposed on LTC pharmacies are significantly more stringent than those imposed on retail pharmacies. These include intensive pharmacist involvement in medication and patient care management, which is crucial to the continuity of care and to the quality of care that patients receive. The average LTC facility resident takes between 11 and 13 medications each day. Prescriptions change frequently, particularly within 30 days of admission to a facility and any time a resident undergoes a significant change in condition. The level of pharmacist involvement and oversight, combined with the fragile physical state of LTC facility patients, provide little opportunity for these patients to "pharmacy shop" or "physician shop" in an attempt to abuse prescription drugs.

II. PDP Drug Safety Program

The SCPC supports the Committee's goals of improving Medicare Part D through fraud and abuse prevention efforts and of reducing prescription drug abuse and diversion among Part D beneficiaries. However, we are concerned that the PDP Drug Safety Program established in section 3151 does not recognize the specialized capacity of LTC pharmacies to prevent potential abuse of controlled substances. Due to the substantial differences between retail and LTC pharmacies, the provision as drafted would pose significant quality of care and compliance issues for both LTC pharmacies and LTC facilities, particularly SNFs. More importantly, section 3151 inadvertently could prevent or delay patient access to needed medications and could undermine Medicare beneficiary choice in selection of a LTC facility.

LTC pharmacies already provide greater oversight of prescription drug dispensing and usage than section 3151 would require, and are in a unique position to ensure the integrity of the Part D program. As contracted pharmacies servicing LTC facilities, our members' pharmacists already have oversight of patients' entire drug regiments. The statutory and regulatory requirements imposed by Medicare on LTC pharmacies—as well as the methods of packaging, dispensing, and tracking medications and monitoring usage in LTC facilities—mean that **LTC pharmacies**

already satisfy higher standards than those section 3151 would impose on pharmacies in any safe pharmacy network. These requirements include, but are not limited to:

- Extensive pharmacy operations and prescription services;
- Around-the-clock delivery;
- Twenty-four hour on-call pharmacists, including many pharmacies that open and staffed 24 hours a day, seven days a week;
- Emergency medications;
- Specialized packaging;
- Comprehensive inventory; and
- Capacity to comply with the reporting requirements necessary to provide these services.

In addition, Medicare and Medicaid Conditions of Participation for LTC facilities require that the pharmacy provide oversight and management of all medications for each patient receiving care and services in the facility. Of particular note with respect to narcotics, which undoubtedly will be determined to be highly susceptible to abuse:

- LTC pharmacists and licensed LTC facility staff use count sheets to track every dose of narcotics prescribed and administered;
- LTC pharmacists and licensed LTC facility staff conduct regular narcotic audits to ensure compliance; and
- Orders and reorders of narcotics are handled by LTC pharmacists and licensed facility staff, not the Medicare beneficiaries themselves.

A comparison of these requirements with those of the safe pharmacy networks demonstrates that LTC pharmacies already provide greater oversight and protection than those proposed under the PDP Drug Safety Program.

The typical LTC facility resident, moreover, is ill-equipped to engage in the type of physician-shopping or pharmacy-shopping that creates substantial risk of substance abuse or diversion. He or she typically suffers from multiple chronic conditions, often is in the midst of intensive rehabilitation therapy, has impairments in multiple activities of daily living and suffers from cognitive impairments. Moreover, patients do not handle their own prescriptions; rather, licensed or certified facility staff administer each dose on every medication directly to each individual patients. This patient population simply is not likely to abuse or divert controlled substances.

The SCPC is concerned that the section 3151 does not require PDPs to include LTC pharmacies in their "safe pharmacy networks." Were it to include such a requirement, however, there is no guarantee that the LTC pharmacy contracting with the LTC facility would be in the safe pharmacy network or that the LTC pharmacy included in the safe pharmacy network would be able to service the LTC facility the patient selects. If no LTC pharmacy contracting with a particular LTC facility is part of the relevant safe pharmacy network, a Part D beneficiary residing in that LTC facility may not be able to access needed medications because no pharmacy in the network legally would be able to provide medications to patients in that facility. This could subject these Part D beneficiaries to delay or denial of needed medications, serious

medical complications and increased overall costs to the Medicare program, particularly the risk of an increase in unnecessary hospital admissions and readmissions.

The SCPC also fears that the PDP Drug Safety Program could severely limit Medicare beneficiary choice in selecting LTC facilities. Patients consider a variety of factors when choosing a SNF or ALF. However, it is their choice to make—not the choice of their PDP. Under the provision as written, once a PDP chooses the pharmacy members of its safe pharmacy network, it also effectively determines the beneficiary's choice of LTC facility if the beneficiary is required to participate in a safe pharmacy network.

III. Preventing prescription drug abuse while protecting LTC facility residents

The SCPC urges the Committee to exempt Part D beneficiaries receiving care in LTC facilities from the lock-in provision.

Since the inception of Medicare Part D, the Centers for Medicare and Medicaid Services (CMS) has recognized the national practice of a single SNF facility contracting with a single LTC pharmacy to best assure quality of care.[1] An exemption for LTC facility patients ensures that nursing home residents, who already receive all their prescription drugs from a single pharmacy, are not inadvertently prevented from access to needed medications when they transition into or out of the LTC facility or reside there but require changes in medications based on changes in the individual's condition. This simple, no-cost and non-controversial clarification will ensure that LTC facility residents, who already are well protected from prescription drug abuse through the use of a single pharmacy for their facility, are not adversely affected by the provision.

We look forward to working with the Committee and with other interested parties to seek effective ways to combat fraud and abuse within the Medicare program while protecting beneficiary access to needed medications in LTC facilities.

[1] While Medicare beneficiaries receiving care and services in SNFs and other LTC facilities maintain the freedom to choose pharmacies, they overwhelmingly choose not to do so in SNFs and frequently choose not to do so in ALFs.

Legislative Hearing on 21st Century Cures on April 30, 2015
Statement of Joanne Kurtzberg, M.D.
President, Cord Blood Association

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

Chairman Pitts, Ranking Member Green and Members of the Subcommittee:

As President of the Cord Blood Association, I want to thank you for the opportunity to submit testimony for the record for your hearing entitled, "Legislative Hearing on 21st Century Cures." I also am on the faculty of Duke University School of Medicine's Department of Pediatrics and work as a Distinguished Professor of Pediatrics and Pathology. In addition, I am the Chief Scientific Officer of the Robertson Clinical and Translational Cell Therapy Program. I also serve as the Co-Director of the Stem Cell Laboratory and the Director of the Carolinas Cord Blood Bank. I have dedicated my professional career to cord blood research, banking and transplantation. Along with my fellow CBA members, I am seriously concerned that FDA's licensure requirements for public cord blood banks are hampering innovation and restricting transplantation for treatment and cures, which is the subject of this written testimony.

Overview of the Cord Blood Association

The Cord Blood Association (CBA) was created and incorporated over this past year. It is an international, non-profit organization that promotes public and private cord blood banking and the use of umbilical cord blood and related tissues for disease treatment and regenerative therapies. CBA's members are public and private banks, as well as providers in the cord blood community and their patients. The CBA's mission includes promoting the work of the cord blood community, saving human lives and changing medicine. Cord blood transplantation can treat and often cure some types of blood cancers and hereditary conditions, and recent studies have suggested that cord blood transplantation holds promise as a future treatment for other conditions, such as autism, traumatic brain injury, stroke and cerebral palsy.

Concerns Regarding FDA Regulation

With regard to the 21st Century Cures draft that is being considered by the Energy and Commerce Committee, we want to raise an important issue that has caused difficulties for the cord blood banking industry. In 2014, the Food and Drug Administration (FDA) finalized licensure requirements for public cord blood banks to ensure the safety, purity and potency of cord blood units for transplantation. Although the CBA shares these critical goals, a number of these licensure requirements are more applicable for drugs than for cord blood units and impose significant cost and administrative burdens on cord blood banks. More importantly, these requirements threaten public health by stifling innovation and restricting growth of the national

cord blood inventory, which in turn limit access to life-saving transplantation for serious diseases and conditions.

The main issue with the regulation of cord blood banking relates to the application of current Good Manufacturing Processes (cGMPs), developed primarily for pharmaceutical manufacturers, to cord blood banking. The practical outcomes for such application include:

- Barriers to making improvements. The cGMPs delay the ability of banks to make timely adjustments to their processes, which may be necessary to enhance safety and effectiveness or to promote innovation.
- Duplicative validation. The FDA has required cord blood banks to validate a number of processes and products used in the process, although some are an unnecessary duplication of tests and validations.
- Burdensome environmental monitoring requirements. The preparation of cord blood is in no way comparable to the manufacture of pharmaceuticals in scale or scope; however, the FDA licensure requirements have not modified the environmental monitoring requirements to reflect this difference.
- Required creation of an expiration date. Prior to licensure, cord blood banks would test a unit to ensure high quality before use for transplantation. FDA now requires annual, regular testing of units, which leads to fewer units for actual patient use. At this time, there is no science indicating the shelf-life of stored cord blood. Therefore, it is difficult to understand why expiration dates are required in the labeling of these units. Units that are identified for therapeutic use should be tested before release but it makes little sense to annually test all units, especially when many cord banks have limited resources.
- Required stability testing. The cGMPs require cord blood banks to sacrifice units for stability testing, although such testing has not been shown to improve potency, integrity or sterility. Again, this testing leads to fewer units being available for patient use.

The sacrifice of units is problematic because, unlike a pharmaceutical, each unit of cord blood is biologically unique and cannot be replicated. A lost unit might have been the best biological match for a future patient.

Recommendations

As described, the application of the cGMPs to cord blood units creates barriers to cord blood transplantation, and removal of such barriers is consistent with the intent of "The 21st Century Cures Act." Thus, we request that you include language in the next iteration of this legislation to help resolve this issue.

Specifically, we recommend that Section 5021 in "Subtitle B—21st Century Manufacturing" be expanded to require the updated cGMPs specifically address good collection, storage, and maintenance practices for public and privately banked cord blood units, reflecting the cord blood banks' real world experience with complying with current requirements. The cGMPs should be updated in collaboration with leading experts in the hematopoietic stem cell transplantation and cord blood banking communities in an open and transparent process. We would welcome the opportunity to work with you to develop specific legislative text to authorize this work.

Conclusion

The CBA commends the Committee on its work on the 21st Century Cures legislation and it is our hope that as this legislation moves forward in the Energy and Commerce Committee, our concerns will be seriously considered in the final legislation. Mr. Chairman, thank you for the opportunity to submit written testimony on this important issue and we look forward to working with you, Members of the Committee and the FDA to ensure that these important issues are addressed in an appropriate manner.

Mr. Chairman:

The Telehealth Working Group is thankful for the opportunity to provide brief comments during this "Legislative Hearing on 21st Century Cures" and for the continued support of the Energy and Commerce Committee throughout this process.

Since its inception, the Telehealth Working Group has been deeply committed to bringing about patient-centered change that lowers costs, improves outcomes, and expands access to telehealth in the Medicare population. Innovative strategies like telehealth are paving a new way forward and focusing on what matters most: connecting patients with quality care. New technology is broadening patients' access to care regardless of distance and is ultimately reshaping how healthcare is delivered.

The Telehealth Working Group is appreciative of the Committee's recognition that increasing the availability of telehealth services and removing barriers to accessing care is an immediate need. There exists ample information in the telehealth landscape which supports the concept that telehealth offers not only increases access to critically needed care, but significant cost savings, particularly when substituted for in-person care. The progress made thus far by the Telehealth Working Group seeks to ensure that Medicare enrollees have access to this care as patients in the commercial, Medicaid, and many in the Medicare Advantage environment do. Our efforts are focused on creating a safe and cost-effective path to achieve this.

The critical concept that drives the Working Group is that telehealth should be treated as a modality to deliver health care services, with the same or higher expectations of quality and standard of care that a patient would receive in person.

We applaud the numerous telehealth stakeholders for their involvement throughout this process and for their substantive contributions in helping to craft legislation. We look forward to continuing our bipartisan discussions with the goal of putting forth good policy that achieves all of our stated goals.

###

The Energy and Commerce bipartisan Telehealth Working Group, is made up of Chairman Fred Upton (R-MI), Ranking Member Frank Pallone (D-NJ), Subcommittee Chairman Joe Pitts (R-PA), Rep. Greg Walden (R-OR), Rep. Doris Matsui (D-CA), Rep. Peter Welch (D-VT), Rep. Bob Latta (R-OH), Rep. Gregg Harper (R-MS), Rep. Bill Johnson (R-OH), and Rep. MarkWayne Mullins (R-OK)

PARKINSON'S ACTION NETWORK

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The Parkinson's Institute
Davis Phinney
The Davis Phinney Foundation for Parkinson's
The Honorable Janet Reno
Cokie Roberts

April 29, 2015

The Honorable Fred Upton
Chairman, Committee on Energy & Commerce
2125 Rayburn House Office Building
Washington, DC 20515

The Honorable Diana DeGette
Committee on Energy & Commerce
2322A Rayburn House Office Building
Washington, DC 20515

Dear Chairman Upton and Representative DeGette,

Thank you for your continued work on the 21st Century Cures Initiative. I write today to urge you to take action to integrate electronic health records (EHRs) with ClinicalTrials.gov in order to increase clinical trial recruitment and retention.

The potential to gather data on thousands – even millions – of patient encounters provides an unprecedented opportunity to make the connection between research and healthcare delivery. By requiring that clinical trial opportunities posted on ClinicalTrials.gov include pretrial screening information using standardized technical vocabularies, EHR systems will be able to compare relevant trial requirements to a patient's clinical and claims data without exposing the patient's private information. EHRs can enable clinical decision support functionality when a patient exhibits certain diagnostic factors that match pre-trial eligibility requirements for relevant clinical trial opportunities. By examining clinical indicators for potential participation in research, providers will be able to easily identify, as well as provide information on, relevant trials that may be beneficial to an individual's care. Patients and doctors could then decide whether participation in a trial makes sense for them.

We are supportive of the provision (Sec. 1102) included in the discussion draft of the *21st Century Cures Act* released today but do hope the language can be strengthened to require trial sponsors to submit trial eligibility criteria – inclusion and exclusion criteria – as coded values so EHRs and other technology can easily match patients to trial opportunities. The current language says NIH shall, to the extent feasible, give consideration to health care terminology and eligibility criteria for electronic matching to coded data. NIH should revise ClinicalTrials.gov to accept and present this information and make its database accessible by provider EHRs.

This provision could help address a large barrier in the discovery of new treatments – low recruitment and retention rates in clinical trials – and the costs that flow with these barriers. One contributing factor to the slow drug-approval rate for Parkinson's is the community's low clinical trial participation rate. At least seventy-one percent of people with Parkinson's report they are unaware of available clinical trials in their area.

We encourage Congress to consider requiring such standards as part of the ClinicalTrial.gov database and to provide funding to the National Institutes of Health to achieve an expansion of the database's architecture to achieve this goal. We believe this is a low cost way to positively impact the entire discovery, development, and

delivery process. Thank you again for your leadership. If you have any questions, please contact Jennifer Sheridan Palute, PAN's director of policy, at jpalute@parkinsonsaction.org or 202-638-4101 ext. 112.

Sincerely,



Ted Thompson, J.D.
Chief Executive Officer

March 30, 2015

The Honorable Fred Upton
Chairman
Committee on Energy & Commerce
U.S. House of Representatives
2125 Rayburn House Office Building
Washington, D.C. 20515

The Honorable Frank Pallone
Ranking Member
Committee on Energy & Commerce
U.S. House of Representatives
2322A Rayburn House Office Building
Washington, D.C. 20515

Dear Chairman Upton and Ranking Member Pallone:

The undersigned represent biopharmaceutical innovators focused on providing life-transforming therapies to patients with severe and life-threatening rare diseases. We join with patient advocates and urge you to permanently authorize the Rare Pediatric Disease Priority Review Voucher (PRV) Program to support greater development of treatments for children suffering from rare pediatric diseases.

Despite preexisting legislation intended to accelerate rare disease therapies to market, drug development in pediatric rare diseases has been neglected. Recognizing this neglect, as part of the Food and Drug Administration Safety and Innovation Act of 2012, Congress supported the Rare Pediatric Disease PRV Program to foster drug development for rare and neglected diseases in children.

Since the passage of the Rare Pediatric Disease PRV Program, the Food and Drug Administration (FDA) has awarded three vouchers. The first voucher was awarded to a biological product approved for patients with Morquio A syndrome, a rare, severely debilitating and progressive disease that previously had no standard accepted treatment other than supportive care. The second voucher was awarded to a biological product for patients with neuroblastoma, a pediatric cancer. Notably, this product is only the third drug that has received initial FDA approval for a pediatric cancer in over 20 years. The third voucher was recently issued to the first FDA approved treatment for certain bile acid disorders that stunt growth and can result in life-threatening liver damage.

The PRV program ends in early 2016. With its termination, companies are left without a crucial incentive to invest in risky and challenging research and development efforts. A program that encourages more treatments for rare pediatric diseases is lost.

Rare disease biopharmaceutical innovators are utilizing the PRV program as intended, as evidenced by the three granted vouchers. The patient community clearly needs a program that drives biopharmaceutical companies to invest in research and development for rare pediatric diseases and provides hope to children who are suffering from these rare conditions.

We urge you to support legislation that permanently reauthorizes this critical program.

Thank you for your consideration of our views. We look forward to working with you.

Sincerely,

Adaptive Biotechnologies
Alexion Pharmaceuticals
Amicus Therapeutics
BayBio
Bayer Corporation
BioMarin
Biotechnology Industry Organization
California Healthcare Institute
CSL Behring
Genzyme
HealthCare Institute of New Jersey
Horizon Pharma
Insmed, Inc.
Lumos Pharma
Marathon Pharmaceuticals
Massachusetts Biotechnology Council
Pennsylvania Bio
Pfenex Inc.
Recordati Rare Diseases
Retrophin
Sarepta Therapeutics
Shire
Synageva BioPharma Corp.
Texas Healthcare and Bioscience Institute
Ultragenyx Pharmaceutical
Vertex Pharmaceuticals
XOMA Corporation

April 13, 2015

The Honorable Fred Upton, Chairman
House Committee on Energy & Commerce
2125 Rayburn House Office Building
Washington, D.C. 20515

The Honorable Frank Pallone, Ranking Member
House Committee on Energy & Commerce
2322A Rayburn House Office Building
Washington, D.C. 20515

Dear Chairman Upton and Ranking Member Pallone:

On behalf of the 30 million men, women and children in the U.S. living with a rare disease, the undersigned organizations urge you to permanently authorize the *Rare Pediatric Disease Priority Review Voucher (PRV) Program* to drive greater development of novel treatments for children with a rare pediatric disease.

There are an estimated 7,000 rare diseases, which are defined as a disease affecting 200,000 or fewer people. Of the nearly one in ten Americans with a rare disease, approximately two-thirds are children. Of the 350 most "common" rare diseases, 27 percent result in death before the child's first birthday.

Despite significant unmet medical need (the approximately 450 approved orphan products treat only about 350 rare diseases), manufacturers face significant obstacles that can hinder the pursuit of rare disease therapies for children, including difficulties associated with conducting clinical trials. To tackle these hurdles, Congress established the *Rare Pediatric Disease PRV Program*.

Currently, upon FDA approval of a novel rare pediatric disease treatment, the *Rare Pediatric Disease PRV Program* provides a biopharmaceutical manufacturer the opportunity to receive a voucher guaranteeing a six month priority review of a New Drug Application (NDA) or Biologic License Application (BLA) for another product, rare disease or not. The voucher can be sold to another company, and there is no limit on how often it may be transferred.

Unfortunately, this program expires March 2016, ending a clear pathway that encourages innovators to pursue treatments in a difficult disease space. To date, three vouchers have been awarded and the program has shown clear evidence that it is a valuable incentive to develop drugs and biologics in this underserved area.

Congress established the *Rare Pediatric Disease PRV Program* because it recognized the necessity of an incentive to enhance innovation in this key area of unmet patient need, a market segment previously overlooked.

We urge Congress to permanently authorize the *Rare Pediatric Disease PRV Program*, which has proven its initial effectiveness in providing hope to children who are suffering from these rare conditions, and drawing manufacturers to invest in the development of novel treatments for rare pediatric diseases.

Sincerely,

Adult Polyglucosan Body Disease Research Foundation
ALD Connect
Alstrom Angels
Alstrom Syndrome International
American Association of the Deaf-Blind
American Autoimmune Related Diseases Association
American Partnership For Eosinophilic Disorders
American Thoracic Society
Amyloidosis Support Groups Inc
Association for the Bladder Exstrophy Community
Association for Creatine Deficiencies
Association for Glycogen Storage Disease
Autoinflammatory Alliance
Avery's Angels Gastroschisis Foundation
Batten Disease Support and Research Association
Barth Syndrome Foundation
Bridge the Gap
CADASIL Together We Have Hope Non-Profit Organization
Canavan Foundation
CARES Foundation
CCHS Family Network
CFC International
Charcot-Marie-Tooth Association
Children's Brittle Bone Foundation
Children's Cardiomyopathy Foundation
Children's PKU Network
Chronic Granulomatous Disease Association
Circadian Sleep Disorders Network
Coalition for Pulmonary Fibrosis
Congenital Hyperinsulinism International (CHI)
Cooley's Anemia Foundation
Council for Bile Acid Deficiency Diseases
CureCADASIL Association
Cure AHC
Cure HHT
Cure JM Foundation
Cure SMA
debra of America
The Dent Diseases Foundation
Dravet Foundation
Dupuytren Foundation
EB Research Partnership
Encephalitis Global
Everylife Foundation for Rare Diseases
Fabry Support & Information Group
Fight ALD-Fighting Illness Through Education

FOD (Fatty Oxidation Disorders) Family Support Group
Foundation Fighting Blindness
Foundation for Angelman Syndrome Therapeutics
Foundation for Ichthyosis & Related Skin Types
FPIES Foundation
Friedreich's Ataxia Research Alliance
Galactosemia Foundation
GBS/CIDP Foundation International
Global Genes
Gwendolyn Strong Foundation
Hereditary Neuropathy Foundation
Histiocytosis Association
Hope for Hypothalamic Hamartomas
International FOP Association
International Pemphigus and Pemphigoid Foundation (IPPF)
Jeffrey Modell Foundation
LGS Foundation
Lipodystrophy United
Little Miss Hannah Foundation
Lymphangiomatosis & Gorham's Disease Alliance
Lymphedema Advocacy Group
The Marfan Foundation
Moebius Syndrome Foundation
Myotonic Dystrophy Foundation
National Adrenal Diseases Foundation
National Alopecia Areata Foundation
National Ataxia Foundation
National Brain Tumor Society
National Eosinophilia Myalgia Syndrome Network
National MPS Society
National Organization for Rare Disorders
National Stem Cell Foundation
National Tay-Sachs & Allied Diseases Association
NBIA Disorders Association
NGLY1.org
Noah's Hope Fund
NTM Info & Research
Organic Acidemia Association
Oxalosis and Hyperoxaluria Foundation
Parents and Researchers Interested in Smith-Magenis Syndrome
PCDH19 Alliances
PF Strategies
Phelan-McDermid Syndrome Foundation
Pituitary Network Association
Potocki-Lupski Syndrome Outreach Foundation
Project DOCC - Delivery of Chronic Care

Pulmonary Hypertension Association
Rare and Undiagnosed Network (RUN)
Rare Disease United Foundation
Rare Genomics Institute
The Reflex Sympathetic Dystrophy Syndrome Association (RSDSA)
Rettsyndrome.org
Sanfilippo Foundation for Children
Scleroderma Foundation
Simons VIP Connect
Stickler Involved People
Sturge-Weber Foundation
Tarlov Cyst Disease Foundation
The Transverse Myelitis Association
The United Leukodystrophy Foundation
United Mitochondrial Disease Foundation
U.S. Hereditary Angioedema Association
Usher Syndrome Coalition
Vascular Birthmarks Foundation
VHL Alliance
We are RARE Inc.
XLH Network
The XLP Research Trust
5p-Society

For additional information, contact Paul Melmeyer, Assistant Director of Public Policy, National Organization for Rare Disorders (NORD), pmelmeyer@rarediseases.org, (202) 588-5700 ext. 104.

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ONE HUNDRED FOURTEENTH CONGRESS

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June 4, 2015

Dr. Kathy Hudson
Deputy Director for Science, Outreach, and Policy
National Institutes of Health
1 Center Drive
Bethesda, MD 20892

Dear Dr. Hudson:

Thank you for appearing before the Subcommittee on Health on Thursday, April 30, 2015, to testify at the hearing entitled "Legislative Hearing on 21st Century Cures."

Pursuant to the Rules of the Committee on Energy and Commerce, the hearing record remains open for ten business days to permit Members to submit additional questions for the record, which are attached. The format of your responses to these questions should be as follows: (1) the name of the Member whose question you are addressing, (2) the complete text of the question you are addressing in bold, and (3) your answer to that question in plain text.

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Thank you again for your time and effort preparing and delivering testimony before the Subcommittee.

Sincerely,

Joseph R. Pitts
Chairman
Subcommittee on Health

cc: The Honorable Gene Green, Ranking Member, Subcommittee on Health

Attachment

Hearing of the U.S. House Energy and Commerce Committee, Subcommittee on Health
"Legislative Hearing on 21st Century Cures"
April 30, 2015

Questions for the Record – Dr. Kathy Hudson

The Honorable Joseph R. Pitts

1. Should there be a link between the unfunded burden of illness relative to typical NIH dollars spent for a similar burden, and program announcements (PAs), requests for applications (RFAs), and requests for proposals (RFPs)?

NIH currently uses Funding Opportunity Announcements (FOAs) in the form of program announcements (PAs), requests for applications (RFAs), and requests for proposals (RFPs), as a way to call for projects from the extramural community that address promising opportunities and unmet needs, both for health and for science.

NIH takes public health needs into account when setting priorities for resource allocations, while also considering other factors, including scientific opportunity, scientific merit, and portfolio balance. Scientific opportunity is particularly relevant because two diseases that impose similar burdens may not be equally ripe for scientific discovery.

Deciding whether a particular research area is under or overfunded is not straightforward. Much of the NIH portfolio involves basic research, which seeks to understand the basic biological processes involved in both health and disease. The Human Genome Project and BRAIN Initiative are good examples of basic research initiatives. Because knowledge generated by basic research may be applicable to numerous diseases and conditions, this research does not neatly map onto a single disease or condition. NIH believes that a priority-setting process that includes measurements of public health needs but is also informed by these and other factors allows us to fund the best science.

2. What other mechanisms exist to encourage funding for disorders that are currently underfunded relative to disease burden? How are they currently being applied toward underfunded diseases?

As noted, NIH weighs indicators of public health need and scientific opportunity when setting priorities. RFAs and RFPs are regularly used during this process to solicit extramural research in targeted disease areas. Moreover, NIH invests significantly in developing research infrastructure, training, intramural activities and partnership with other entities to address targeted disease areas as well.

In recognition of public health challenges either chronic or newly emerging, NIH supports infrastructure often in the form of research centers, networks, and core facilities to enhance research capacity focused on specific diseases or conditions. As just one example, clinical research in stroke is a high priority at the NIH, and new infrastructure through the Stroke Trials Network promises to enhance the capacity of the community to address the most important clinical questions in stroke care.

NIH-supported training grants, alone or linked to research initiatives, provide young investigators the opportunity to gain expertise in under-developed research areas. For example, NIH is supporting research to develop new artificial pancreas technologies, and a recently released RFA will pave the way for pivotal trials to collect data needed for FDA approval of artificial pancreas technologies. In tandem, NIH is also

supporting research training of engineers and behavioral scientists—fields that are critical for propelling progress in this area.

NIH often partners with other entities in the biomedical research enterprise to address areas of high need. For example, the Accelerating Medicines Partnership (AMP) is a public-private partnership between NIH, the Food and Drug Administration, and a group of pharmaceutical and nonprofit organizations. AMP is aimed at identifying new diagnostics, disease biomarkers, and potential therapeutic targets with an integrative structure that allows stakeholder needs and input to inform its governance and shares data among its constituent groups. The initial three disease areas—Alzheimer's, type 2 diabetes, and lupus—are all diseases for which a substantial public health need is present, and for which gaps in our knowledge pose significant risks and barriers for developing potential therapies. The public-private nature of the AMP allows it to address these unmet needs with a targeted approach aimed at reducing barriers to translation and the eventual development of therapies.

NIH considers disease burden not just as the number of people affected at a given time, but also the potential burden of an emergent threat to be contained. The recent Ebola epidemic response in West Africa illustrated how NIH can leverage various flexible funding mechanisms and established infrastructure to address an emergent threat with high mortality rate and a rapidly expanding disease burden. In 2014, for example, more than 30 different therapeutic candidates and more than 20 different vaccine formulations were evaluated using animal models, which were supported by NIH over many years. Researchers in NIH's intramural Vaccine Research Center, in collaboration with GlaxoSmithKline, quickly initiated testing of a new vaccine at the NIH Clinical Center. Furthermore, NIH, with other U.S. government and industry partners, launched a large clinical trial in 2015 to assess the safety and efficacy of two experimental Ebola vaccines in Liberia, one of the areas hardest hit by the disease. The speed by which NIH was able to move against this outbreak illustrates the flexibility by which long-standing research infrastructure can be tapped, along with the initiation of new funding mechanisms, to address public health needs.

3. What is the best metric for disease impact? The WHO recommends DALYs. Is there a better metric that incorporates both death and disability?

Because of the challenges inherent in choosing rigorous, comparable data sources and measurements, NIH believes that a careful consideration of appropriate burden measurements on a case-by-case basis for each disease is the best way to approach this question. The majority of rigorous public health research, including the majority of studies conducted by the CDC, uses measurements and data sources selected on an individual basis, based on the best fit for the disease or condition being studied. For example, estimating the number of Americans suffering from headaches (a condition in which medical care is not necessarily sought) will employ much different methodologies than attempts to measure the incidence of severe mental illness, in which the condition may be difficult to diagnose and the patient population often is difficult to reach (e.g., homeless patients). Considering the best approach for each disease and condition ensures that the most appropriate, objective measurements are included. However, this makes comparisons between conditions difficult to make.

NIH is aware of the utility of disability-adjusted life years (DALYs) as a tool for comparison between diseases and conditions. The World Health Organization, along with its academic and nonprofit collaborators, has pioneered the measurement of DALYs across a large range of diseases and countries using its Global Burden of Disease study. Plots of an exploratory analysis of the alignment between NIH funding and several measurements from the Global Burden study, including DALYs, are posted on the NIH web site at http://report.nih.gov/info_disease_burden.aspx. While DALYs are currently the best metric for comparing across diseases that can cause both death and disability, there are significant caveats to using DALYs as the sole means of capturing disease burden. DALYs are a measurement that attempts

to combine death and disability into a single measure in order to compare diseases that impose different types of burden. To calculate this metric, the severity of disability for a given condition is given a subjective weight before being combined with age-adjusted mortality data, and the underlying assumptions behind that weighting are not always clear or consistent between studies. When DALYs are used to compare vastly different diseases that impose a variety of types of burden (financial, disability, mortality, U.S. vs. global), they can provide an incomplete picture of the differences between diseases. Given these concerns, NIH believes that DALYs data should be taken into consideration as one of several measurements in order to form the most comprehensive picture of disease impact.

Hearing of the U.S. House Energy and Commerce Committee, Subcommittee on Health
"Legislative Hearing on 21st Century Cures"
April 30, 2015

Questions for the Record – Dr. Kathy Hudson

The Honorable Leonard Lance

Dr. Hudson, thank you for testifying before the Committee this morning and lending your expertise as we continue to move forward with this important initiative. One issue which has not been raised today, though it affects five million Americans each year, is what we can be doing to support the furtherance of research in critical care.

As you are aware, critical care medicine is the care of patients whose illnesses or injuries present a significant danger to life, limb, or organ function and encompasses a wide array of diseases and health issues. This care is typically provided by highly-trained physicians using complex therapies in the intensive care unit (ICU). Unfortunately, despite the likelihood of a patient requiring care in the ICU throughout their lifetime, and the economic cost of providing this care – last estimated in 2005 to be $81.7 billion per year, representing 13.4% of hospital costs, 4.1% of national health expenditures, and 0.66% of gross domestic product – very few breakthroughs have been made in therapies and treatments for these patients. One reason for this may be that critical care research is complex and involves many departments, specialties, professional societies and research institutes/foundations. Lack of coordination and collaboration among these stakeholders has stymied progress, particularly at the National Institutes of Health (NIH) where critical care-related projects are ongoing throughout the 27 Institutes, leaving the field without a solid foundation from which to advance new treatments and therapies.

The NIH recently demonstrated the importance and efficiencies that come from increased coordination among stakeholders by establishing an Office of Emergency Care Research, which serves as hub for basic, clinical and translational emergency care research and training across the NIH.

1. Recognizing the distinct difference between emergency care and the unique care occurring in the ICU, Dr. Hudson, what is the rationale for not having a similar office at NIH to coordinate and streamline, as well as identify gaps in, our nation's critical care research?

A great deal of critical care research is supported at NIH. The Trans-NIH Office of Emergency Care Research (OECR) already advocates for and promotes critical care research where it interfaces with emergency care across the NIH. Creating a new office would result in significant overlap with the existing OECR, which is already focused on many aspects of acute critical care medicine. Below are a few examples of the many clinical studies of ICU patients supported across NIH in just the last two years.

The National Heart, Lung and Blood Institute (NHLBI) supports a large number of ICU-based studies:

- An intervention to reduce ventilator-associated pneumonia in the ICU (5R01HL105903-05).
- An investigation of the relationship between low-level secondhand smoke exposure and susceptibility to acute lung injury in the ICU (5R01HL110969-03).
- A study to improving decision making for patients with prolonged mechanical ventilation (5R01HL109823-03).

- A study of Acute Respiratory Distress Syndrome after isolated traumatic brain injury (1F32HL124911-01).
- A study of skeletal muscle dysfunction in ICU patients (5R01HL113494-02).
- A study of nutrition on patients in the ICU with respiratory distress syndrome (5R01HL093142-05).

Several studies are supported by the National Institute of Nursing Research (NINR):

- Sedation and pain the in the ICU (5K23NS090900-02).
- Early exhaled biomarkers of infection in ICU patients (5R00NR012016-05).
- Oral care in mechanically ventilated ICU patients (2R01NR007652-10A1).
- The effect of endotracheal tube movement on patient discomfort and agitation in the ICU (5F31NR011373-04).
- Pain and hypoxia in premature babies in the neonatal ICU (5R01NR011209-04).

In addition, two studies—one in the control of early sepsis in the ICU (5K23GM094465-05) and another of critically ill patients with sepsis (3P50GM076659)—have been supported by the National Institute of General Medical Sciences (NIGMS).

In the area of neurointensive care, the National Institute of Neurological Disorders and Stroke (NINDS) funds basic and applied research to enable brain protection and resuscitation in persons with critical illness. NINDS-funded investigators are pursuing new methods to monitor brain oxygen, blood flow, intracranial pressure, electrical activity, and neuroimaging to guide care in the critical care unit. Others work to develop neuroprotective drugs, hypothermia, and means to optimize brain metabolism to maintain brain and spinal cord health in persons with critical illness. Advances in neurointensive care have improved the outcomes of persons with a variety of tragic conditions such as subarachnoid hemorrhage, acute stroke, Guillain-Barre syndrome, intracerebral hemorrhage, cardiac arrest, and traumatic brain injury.

In pediatric critical care, The Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD) supports critical care research in a number of ways. Investigator-initiated funded projects target such important clinical issues as the role of monitoring of intracranial pressure in traumatic brain injury, improving the quality of in-hospital cardiopulmonary resuscitation, developing bioresorbable splints for airway weakening, and monitoring long-term outcomes from sepsis and other forms of critical illness. The Institute also supports research evaluating the decision-making process and parent-provider communication surrounding critical illness. In addition, the Institute supports a Network of seven, large tertiary care children's hospitals to conduct collaborative, multicenter research on critical illness. Finally, several training programs are funded by the Institute to support young critical care providers wishing to perform research in the field.

2. Do you believe the creation of a working group within the NIH to assess the particular needs of this field would fall within the scope of this committee's effort to promote policies to accelerate the discovery, development and delivery of therapies and cures?

NIH currently has an Emergency Care Research Working Group, a trans-NIH body charged with adding value and efficiency to both current and future research on the many conditions relevant to emergency care. As indicated in the answer to the preceding question, many of these conditions involve aspects that are directly relevant to—or overlap with—critical care. Given the scope of this group's charge as well the myriad of activities that are currently being supported by NIH in the area of critical care research, an additional working group does not seem needed at this time. NIH can and does, however, contemplate

what specific scientific questions are not being addressed by its current portfolio of critical care research. This is done through a careful and balanced portfolio and gap analysis. Although these analyses take considerable time and effort to perform, they are nonetheless performed at NIH so that appropriate responses can be determined and subsequently executed. Recent analyses conducted by NICHD on its critical care portfolio, for instance, elucidated the need to heighten attention to areas of research related to multiple organ dysfunction syndrome. Consequently, NICHD sponsored a conference on this topic in the spring of 2015 and received approval to issue a corresponding Program Announcement in Fiscal Year 2017. Analyses such as these have been used to both identify specific areas of need and to focus research efforts in an attempt to fill those needs.

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June 4, 2015

Dr. Janet Woodcock
Director
Center for Drug Evaluation and Research
U.S. Food and Drug Administration
10903 New Hampshire Avenue
Silver Spring, MD 20993

Dear Dr. Woodcock:

Thank you for appearing before the Subcommittee on Health on Thursday, April 30, 2015, to testify at the hearing entitled "Legislative Hearing on 21st Century Cures."

Pursuant to the Rules of the Committee on Energy and Commerce, the hearing record remains open for ten business days to permit Members to submit additional questions for the record, which are attached. The format of your responses to these questions should be as follows: (1) the name of the Member whose question you are addressing, (2) the complete text of the question you are addressing in bold, and (3) your answer to that question in plain text.

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Thank you again for your time and effort preparing and delivering testimony before the Subcommittee.

Sincerely,

Joseph R. Pitts

Joseph R. Pitts
Chairman
Subcommittee on Health

cc: The Honorable Gene Green, Ranking Member, Subcommittee on Health

Attachment

Attachment—Additional Questions for the Record

The Honorable Gus Bilirakis

Dr. Woodcock, I held a 21st Century Cures related roundtable which featured a constituent of mine living with Chronic Obstructive Pulmonary Disease (COPD). She talked about some of the work that the COPD Foundation is doing including the creation of the COPD Biomarker Qualification Consortium (CBQC). The CBQC is a unique public-private partnership driven by the need to address our nation's need for new therapies for the treatment of COPD, third leading cause of death. If the CBQC fails, it is possible that we may never see a unique partnership that engages the patient advocacy community, academia, industry and the NIH collaborate on amassing this amount of pre-clinical data, pre-competitive data again.

1. With this example in mind, if 21st Century Cures legislation codifies the biomarker process at the FDA without timelines, what do you predict the time frame will be for biomarker qualification?

2. Could you update my office on the present timeline for the CBQC fibrinogen application?

The Honorable Renee Ellmers

1. It seems evident that not all therapies "fit" into the FDA's pathway (section 351/361) for small molecules. For example, FDA has already developed a separate pathway for biologics. Has FDA given consideration to the notion that other therapies, like personal precision regenerative stem cell therapies may require a separate regulatory path, similar to the more flexible and workable pathway that currently exists/ is in the future direction for Europe, Korea and Japan?

2. Has the FDA examined how the European Medicines Agency (EMA) and European National Agencies handle advanced therapy regulation, e.g. regenerative cell products, and also the future global trends for regulation in these areas?

3. It is clear that other countries have developed processes on how to accelerate stem cell product approvals, while still ensuring patient safety. Is the FDA willing to consider a new regulatory framework for regenerative cell therapy that follows a hybrid of some of the current models, for example those involving "minimally manipulated" regenerative cell products?

4. Is the FDA considering any regulatory changes or guidance changes with regards to regenerative therapies, in light of the advancements and changes in other countries? And if not, why not?

5. In 2014, the FDA finalized licensure requirements for public cord blood banks to ensure the safety, purity and potency of cord blood units for transplantation. To date, five unrelated cord blood banks in the USA have obtained a Biologics License Application (BLA). The FDA licensure requirements are based on regulations that were created for pharmaceuticals rather than specifically for cells or cord blood units and, as such, impose unnecessary burdens on public cord blood banks.

   In addition, the FDA requirements stifle innovation in this emerging field, and they unnecessarily divert the limited funds available for public cord blood banking to processes that are detrimental to the growth of the national cord blood inventory. This, in turn, limits access to life-saving transplantation for patients with serious life-threatening diseases and conditions, especially among minority populations.

The licensure requirements have created challenges for public cord blood banks as follows:

- Barriers to making improvements. The current Good Manufacturing Processes in the FDA licensure requirements delay the ability of banks to make timely adjustments to their processes, which may be necessary to enhance safety and effectiveness or promote innovation.
- Duplicative validation. The FDA has required cord blood banks to validate a number of processes and products used in the manufacturing process, although many of the processes and products are already FDA approved for their intended use.
- Burdensome environmental monitoring requirements. The preparation of cord blood is not comparable to the manufacture of pharmaceuticals – in fact, cord blood is processed in an automated closed system. However, the FDA licensure requirements have not modified the environmental monitoring requirements to reflect this difference. This has resulted in the imposition of requirements that exceed the conditions under which manufacturing occurs.
- Required expiration date. Before there was FDA licensure, cord blood banks always tested units to ensure high quality before use for transplant. FDA now requires annual testing of licensed units which, in turn, leads to fewer units for actual patient use. There is no science indicating that cord blood loses potency over time in storage. Therefore, it is difficult to understand why expiration dates are required in the labeling of these units. Units that are identified for therapeutic use should, of course, be tested before release, but it makes little sense to annually sacrifice good units that might be the unique match for a patient in need.
- Required stability testing. The current Good Manufacturing Processes also require cord blood banks to sacrifice units for stability testing, although such testing has not been shown to improve potency, integrity or sterility. Again, this testing leads to fewer units being available for patient use.

With this background, would FDA be willing to work with the cord blood community to resolve these important issues?

The Honorable Doris O. Matsui

This draft includes a provision relating to studying and improving drug manufacturing practices. The draft does not address the proper manufacturing practices for cord blood units, which are currently treated as a drug by the FDA.

The FDA requires licensing of cord blood banks that provide cord blood units for non-family use. Currently, it uses the framework developed for pharmaceutical manufacturing for regulation of these banks. This framework does not reflect the unique nature of cord blood banking in many respects.

How will the FDA adapt its regulatory approach to achieve the goals of licensing without imposing undue burden?

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June 4, 2015

Dr. Jeffrey Shuren
Director
Center for Devices and Radiological Health
U.S. Food and Drug Administration
10903 New Hampshire Avenue
Silver Spring, MD 20993

Dear Dr. Shuren:

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Subcommittee on Health

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Attachment — Additional Questions for the Record

The Honorable Joseph R. Pitts

1. Recently, we have heard from individuals and companies about a growing concern with counterfeit medical devices being used by certain physicians and practices. In some cases, patients have been harmed. Awareness of this illegal activity surfaced via FDA's MDR system. Upon further examination, it was concluded that the legal products were never purchased by the identified physician/facility and that counterfeit products were used on patients.

2. Please explain if you are aware of these types of situations. If so, what steps are you taking to address? How long does it take for FDA to close out an investigation once it begins?

The Honorable Leonard Lance

1. Are FDA-approved tests safer or more effective than LDTs approved under the current process, and what data do you have that supports an answer either way?

2. Initiating an entirely new regulatory regime for LDTs will likely take significant time and resources. The FDA guidance requires nine years to fully implement. It has been suggested by the Diagnostic Test Working Group that FDA tackling this job will require creating a whole new division in FDA.

3. Do you have an estimate of how much this will cost and how it will be paid for?

