21ST CENTURY CURES: EXAMINING BARRIERS TO ONGOING EVIDENCE DEVELOPMENT AND COMMUNICATION

HEARING

BEFORE THE

SUBCOMMITTEE ON HEALTH

OF THE

COMMITTEE ON ENERGY AND COMMERCE
HOUSE OF REPRESENTATIVES

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[1] The attachments to Ms. Grealy's testimony are available at *http://docs.house.gov/meetings/IF/IF14/20140722/102524/HHRG-113-IF14-Wstate-GrealyM-20140722.pdf.*

21ST CENTURY CURES: EXAMINING BARRIERS TO ONGOING EVIDENCE DEVELOPMENT AND COMMUNICATION

TUESDAY, JULY 22, 2014

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

The subcommittee met, pursuant to call at 3:00 p.m., in room 2322, Rayburn House Office Building. Hon. Joseph R. Pitts (chairman of the subcommittee) presiding.

Present: Representatives Pitts, Burgess, Shimkus, Blackburn, Lance, Bilirakis, Ellmers, Pallone, Green, Barrow, DeGette, and Waxman (ex officio).

Staff: Leighton Brown, Press Assistant; Noelle Clemente, Press Secretary; Sydne Harwick, Legislative Clerk; Robert Horne, Professional Staff Member, Health; Carly McWilliams, Professional Staff Member, Health; Chris Sarley, Policy Coordinator, Environment & Economy; Heidi Stirrup, Health Policy Coordinator; Jessica Wilkerson, Legislative Clerk; Ziky Ababiya, Staff Assistant; Eric Flamm, FDA Detailee; Eddie Garcia, Professional Staff Member; Karen Nelson, Deputy Committee Staff Director for Health.

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

Mr. PITTS. The subcommittee will come to order. The chair will recognize himself for an opening statement.

In this, the sixth hearing of our 21st Century Cures Initiative, we are examining continued evidence development and communication of information regarding treatments and cures in the real world setting. Discovery of the risks and benefits of drug or treatment does not end with FDA approval or clearance. It is often just the beginning of learning about different uses for drugs and devices, for different indications, conditions, and populations. Treatment in the real world also brings out additional information on safety and efficacy, and ensuring that this knowledge is shared widely among providers, patients, and researchers is critical.

As a result, the ability of patients, physicians, and developers to communicate effectively is so important for the future of cures in this country. Unfortunately, many of the witnesses and participants we have had before us since the Cures Initiative began have raised concerns regarding barriers to communication and evidence

development. This hearing is a direct result of the feedback we have received from patient groups and other interested parties.

As today's witnesses will discuss, efforts to limit off label use among the provider community, limitations on communication found under HIPAA, and the Physician's Sunshine Act are just a few of the barriers to 21st century cures that have been raised with us over the past few months. It is my hope that this hearing allows the members an opportunity to consider those potential barriers and the role they play in our healthcare system.

With that thought in mind, I would like to thank all of our witnesses for being here today, and I will yield the balance of my time to Dr. Burgess, vice chairman of the subcommittee.

[The prepared statement of Mr. Pitts follows:]

PREPARED STATEMENT OF HON. JOSEPH R. PITTS

The Subcommittee will come to order.

The Chair will recognize himself for an opening statement.

In this, the fourth hearing of our 21st Century Cures Initiative, we are examining continued evidence development and communication of information regarding treatments and cures in the real world setting.

Discovery of the risks and benefits of a drug or treatment does not end with FDA approval or clearance. It is often just the beginning of learning about different uses for drugs and devices, for different indications, conditions, and populations.

Treatment in the real world also brings out additional information on safety and efficacy, and ensuring that this knowledge is shared widely among providers, patients, and researchers is critical.

In such ways, the ability of patients, physicians, and developers to communicate effectively is so important for the future of cures in this country. Unfortunately, many of the witnesses and participants we have had before us since the cures initiative began have raised concern regarding barriers to communication and evidence development.

As our witnesses will discuss, efforts to limit off-label use among the provider community, limitations on communication found under HIPAA, and the Physician Sunshine Act are just a few of the barriers to 21st Century Cures that have been raised with us over the past few months. It is my hope that this hearing allows the members an opportunity to consider these potential barriers and the role they play in our health care system.

The importance of today's hearing and the reason for our calling it is really a direct result of the feedback we have received from patient groups and other interested parties.

With that though in mind, I would like to thank all of our witnesses for being here today, and I yield the balance of my time to Rep. _____.

Mr. BURGESS. Thank you, Mr. Chairman. I too want to welcome our witnesses. I certainly look forward to hearing from them today.

I appreciate the continued series of hearings on the 21st Century Cures Initiatives. Certainly looking forward today to exploring the role that healthcare providers, physicians, can have in increasing communications between patients, researchers, and those who innovate. Different uses for therapies are constantly being discovered through information highways, including social networks, patient advocacy groups, and physicians sharing information.

There is no doubt that technology and the ability to communicate easily with people all around the world will change how we conduct research, how clinical trials are managed, and how the post-market surveillance of technologies is handled.

We must recognize this fact and be open to rethinking the traditional means of how we have engaged with our patients. We must

also rethink our expectations of the ease with which patients may engage with each other. The fact of the matter is if I get on a plane with my iPad, I have got the New England Journal, I have got the Journal of the American Medical Association, and I have got the most current Journal of Obstetrics and Gynecology with me. And it is simply a matter of opening it and reading while on the plane. The ability to keep up with rapidly-changing and evolving fields is unlike anything anyone has ever had in the past.

So this is the world in which we live today, and we need to be open to realizing the benefits that can be drawn from this fact. And also recognize that while we are exchanging information, patient advocacy groups are likewise engaged.

So we certainly look forward to a lively discussion with the panel today. Mr. Chairman, I will yield back the time.

Mr. PITTS. Anyone on our side seek time? Vice chair, Ms. Blackburn?

Mrs. BLACKBURN. Thank you so much, Mr. Chairman. And to our panel, I want to welcome each of you. There is so much that is going on in the field of healthcare informatics, and Dr. Burgess just touched a little bit on that, and also medical devices. We are going to hear from Edwards Life Sciences about a heart valve which was approved in 41 countries before it was approved here in the U.S.

And this is something that is unacceptable when you look at the length of time that it takes to get these medical devices through the FDA's process. In Memphis, Tennessee, my home State, one in four jobs is dependent on medical devices. And when you look at what is happening in the Nashville area with healthcare informatics, you realize the importance and the increasing importance of that as an economic development sector to our State.

I think it is imperative that we provide a 21st century regulatory framework for 21st century technology and a framework that is going to encourage innovation while providing safe, effective, and new therapies. And with that, I yield back my time.

Mr. PITTS. The chair thanks the gentlelady. Now filling in for the ranking member, Mr. Pallone, Mr. Green of Texas, 5 minutes for opening statement.

Mr. GREEN. Thank you, Mr. Chairman. And thank you and the ranking member, who will be here shortly, on this continuing series of hearings on the 21st Century Cures. This is really what our Health Subcommittee should be about, how we can help. And following my colleague from Tennessee, although I did not know that many jobs in Memphis were for medical. I thought it was just barbecue or Graceland.

Mrs. BLACKBURN. If the gentleman will yield——

Mr. GREEN. Briefly.

Mrs. BLACKBURN [continuing]. It is because of the barbecue that we need the medical——

Mr. GREEN. Well, as you know, there is a difference between Tennessee and Texas barbecue. We like——

Mrs. BLACKBURN. I would ask the gentleman to yield again on that. There would not be a Texas if there were not Tennessee——

Mr. GREEN. Well, and I cannot disagree with that because, frankly, we got all the rebels from Tennessee and helped us win inde-

pendence in Texas. But with that, I am going to yield the balance of my time to my colleague, Congressman DeGette from Colorado.

Ms. DEGETTE. Thank goodness. Mr. Chairman, I really want to thank you for holding this next hearing in this series on the 21st Century Cures. I have got to say I was around my district all weekend, and everybody I talked to from the Jefferson County Economic Development Team to the telephone town hall meeting I had last night, to the OFA people. Everybody was excited to hear about this bipartisan effort that we are having, and I am excited, too.

Throughout all of the previous hearings and roundtables that we have had on all of these topics, we have already learned a tremendous amount about what role Congress should play in helping to further advance and accelerate treatment and cures.

Today the witnesses will talk about examining barriers to ongoing evidence development and communication. The potential areas for discussion are far ranging, to say the least, but I am looking forward to hearing some specifics from the witnesses on the potential benefits of enhanced data collection and improved maintenance and secured sharing of data and information.

These types of evidence development and communication can and do play essential roles in the drug and device development and approval processes, as well as in reimbursement determinations. For example, how can we take advantage of data and information to more effectively identify patients for clinical trials that are relevant to their individual disease or condition? How can we harness the data and information collected during clinical trials? What about information after the drug or device is introduced into the market? And how do we effectively utilize this information while maintaining a high standard of privacy protections?

On the reimbursement side, how is Medicare's coverage with the evidence development process currently being used? And how can we improve these processes to be clear?

Just to talk for a minute about some of the things that are going on in terms of evidence sharing and data, Mr. Burgess talked about taking his iPad on the airplane. And I just literally got off the airplane from Denver where I was reading this article from The New Yorker this week. Maybe some of you have seen it. It is about a family who has a child with a very, very, very, very rare genetic disorder: NGLY1. And they finally got it diagnosed, but they did not think anybody else had it until the dad, who is a computer professor at the University of Utah, wrote a blog which went viral, and everybody read about it.

And the upshot is that they have now identified patients with this genetic disorder around the world. They have all met. They have put together a research consortium. They have people doing research and writing a paper to be published in a scientific journal. And they are on their way to try to figure out what they can do about this very, very rare defect.

These patients did this on their own because they were sophisticated parents. So what I would like to know is what can we do to harness this in a much more systemic way so that these types of communications can occur effortlessly both within the United States and with our colleagues around the world. So all of these are important questions.

I really look forward to hearing the testimony today and to learning about these topics. Thank you very much, and I yield back.

Mr. PITTS. The chair thanks the gentlelady, and now recognizes the ranking member of the full committee, Mr. Waxman, 5 minutes for opening statement.

OPENING STATEMENT OF HON. HENRY A. WAXMAN, A REPRESENTATIVE IN CONGRESS FROM THE STATE OF CALIFORNIA

Mr. WAXMAN. Thank you very much, Mr. Chairman. Today we have an opportunity to learn more about several issues that were raised at our previous meetings on the 21st Century Cures Initiative. From the first roundtable discussion that kicked off the initiative, we heard that FDA and NIH are leaders in driving and using advances in molecular medicine and digital technology to help get new cures to patients more quickly. They have also made great strides in improving and streamlining procedures for conducting clinical trials and in reviewing innovative new drugs and medical devices.

However, we also heard about impediments that stand in the way of researchers and companies making full use of these advances. While patient registries can facilitate enrollment in clinical trials and help researchers find new research avenues to pursue, many believe more could be done to encourage their development and use.

Electronic health care records can help physicians and sponsors identify patients for clinical trials and evaluate the effects of drugs already on the market, but privacy concerns are limiting their use. And although FDA has shown an increasing willingness to accept data from smaller clinical trials, the more limited data generated to support FDA approval may not be adequate for coverage decisions by Medicare or private insurers. I look forward to hearing more about these barriers and what can be done to address them.

We should remember, though, that we have a review and approval system that is already working quite well. It has led to enormous breakthroughs and coverage of cutting-edge drugs and devices. FDA reviews and approves drugs faster than any other regulatory agency in the world. NIH and FDA are world leaders in clinical trial design and in integrating the newest science into their policies and approaches while protecting the health of the patients. And Medicare has demonstrated flexibility in its national coverage determinations so that beneficiaries can access these new cures.

I have a great interest in fostering greater access to innovative drugs, devices, and health services. But I also know that access to new, innovative medicine alone will not increase the quality and outcomes patients experience in our healthcare system. Incentives must be in place for providers to furnish high quality care to the right patient at the right time in the right setting of care.

The Affordable Care Act was a major advancement in meeting these challenges, but we still have work to do. In particular, we should enact the delivery reforms contained in our bipartisan SGR legislation. We can make another great stride forward if we can send this legislation to the President's desk before the end of this year.

I have a little time left, and I would be pleased—anybody on our side want it?

If not, I yield back the time, and let us hear from the witnesses.

Mr. PITTS. The chair thanks the gentleman. As always, members' opening written statements will be made a part of the record.

We have one panel today with five witnesses. I will introduce them in the order of them making their presentations. First, Dr. Josh Rising, Director of Medical Devices, the Pew Charitable Trust; Dr. Louis Jacques, Senior Vice President, Chief Clinical Officer of ADVI; Mr. Michael Mussallem, Chairman and Chief Executive Officer of Edwards Life Sciences Corporation; Dr. Gregory Schimizzi, Co-founder, Carolina Arthritis Associates, P.A.; and Ms. Mary Grealy, President, Healthcare Leadership Council.

Thank you each for coming. Your written testimony will be placed in the record. You will each be given 5 minutes to summarize your testimony. And at this time we will recognize Dr. Rising 5 minutes for his opening statement.

STATEMENTS OF JOSH RISING, DIRECTOR, MEDICAL DEVICES, THE PEW CHARITABLE TRUSTS; LOUIS JACQUES, SENIOR VICE PRESIDENT AND CHIEF CLINICAL OFICER, ADVI; MICHAEL A. MUSSALLEM, CHAIRMAN AND CEO, EDWARDS LIFESCIENCES; GREGORY SCHIMIZZI, CO-FOUNDER, CAROLINA ARTHRITIS ASSOCIATES; MARY GREALY, PRESIDENT, HEALTHCARE LEADERSHIP COUNCIL

STATEMENT OF JOSH RISING

Dr. RISING. Chairman Pitts, Ranking Member Pallone, members of the committee, I thank you for the opportunity to provide testimony. My name is Josh Rising. I am Physician Director of Medical Devices at the Pew Charitable Trusts.

We have an exciting opportunity today to talk about the future of healthcare, a future where we can harness electronic data to improve patient care. Advances in technology offer potential for new approaches to develop medical evidence through a continuous cycle that begins before a product is approved and continues as the product is used by patients.

As we move toward this total life cycle approach, we must consider two important issues. First, we know that clinical trials are the largest contributor to the cost and length of product development. We need to use new approaches to decrease their length and cost without doing away with these trials and the critical data they provide. Second, we must have the tools necessary to quickly and efficiently identify problems with approved drugs and medical devices, and to assess their performance in real world settings that can be different from clinical trials.

We are at a key turning point. Electronic health records today collect more data on patient outcomes than we have ever had, but we are failing to realize that potential. One important innovation to harness data from electronic health records is the registry, large databases that collect information on groups of patients treated for a particular medical condition.

Now, imagine if we could conduct clinical trials for a tenth of the current cost. This is precisely what physicians in Sweden recently

did using an existing registry. They studied heart attack prevention in more than 7,000 patients, comparing two different procedures. The data were drawn from electronic health records, and the trial cost only $300,000, or roughly $50 per patient. Conducting such a study outside of a registry in the United States would cost hundreds of millions of dollars, if not more. We can do this in the United States, too, but only if we fix the lack of interoperability among electronic health records and streamline certain electronic administrative processes.

Second, just as important as ensuring prompt access to new cures is the ability to detect problems with drugs and medical devices on the market and assess their performance in real world conditions. Here, too, registries can help. For example, an Australian registry of artificial joints found that one type of Metal-on-Metal Hip failed at a rate more than two times higher than conventional hips, ultimately leading to a worldwide recall of the device. Detecting such problems earlier is vital for patient safety and could save our healthcare system vast sums.

Pew will soon release a report on registries produced in partnership with the Blue Cross and Blue Shield Association and the Medical Device Safety Group and the EPINet. In this report, we recommend steps to deliver timely, actionable information from registries to all stakeholders, including the public.

Now, there are other ways that electronic data can also improve patient care. One is better use of the brand new Unique Device Identifier, or UDI, System, which was created by FDA at the direction of Congress and will result in a unique number assigned to nearly all medical devices. If we now incorporate this number into insurance clams, we can use FDA's Sentinel System to assess device safety problems the same way we do for drugs. Incorporating UDI into claims will also provide payers, such as CMS, with the necessary data unavailable elsewhere, to evaluate outcomes for patients with implanted medical devices.

Adding a UDI field to claims has generated support across healthcare, including from hospitals such as Geisinger and Mercy, health plans like Aetna, physician societies including the American College of Cardiology and the Society of Thoracic Surgeons, as well as patient and consumer organizations. Additionally, HHS Secretary Burwell articulated the benefits of adding UDI to claims du ring her Senate confirmation process.

New mechanisms to collect data both prior to and after FDA approval can help facilitate faster clinical trials and ensure that any problems are promptly detected. Congress should work with the Administration to maximize the potential of these new data sources to ensure patient access to safe and effective medical devices.

Thank you again for the opportunity to testify, and I welcome your questions.

[The prepared statement of Dr. Rising follows:]

Testimony before the Committee on Energy & Commerce, Subcommittee on Health
United States House of Representatives

July 22, 2014

Dr. Joshua P. Rising, director of medical devices
The Pew Charitable Trusts

Chairman Pitts, Ranking Member Pallone, and members of the Committee, thank you for the opportunity to provide testimony. My name is Josh Rising. I am a pediatrician, and I direct medical device work at The Pew Charitable Trusts, an independent, nonpartisan research and public policy organization dedicated to serving the public.

We have an exciting opportunity today to talk about the future of health care—a future where we can harness electronic data to improve patient care through a better understanding of how medical products impact health outcomes and more rapid cycles of product development. Technological advances allow us to consider evidence development as a continuous cycle that begins before a product is approved and continues as the product is used by patients.

This process begins during the product development and approval phase. The Food and Drug Administration (FDA) reviews data on drugs and medical devices to ensure that the benefits of new products outweigh risks. But the collection of data hardly stops when FDA approves a new medicine, implant or other technology used to treat, cure or prevent disease. Manufacturers, health plans, FDA and researchers all need information after approval to better understand the performance of new products.

This total life-cycle approach supports the development of the next generation of products while ensuring that sufficient data is collected both before and after approval. New electronic tools have the potential to improve the quality of the data and the efficiency of information collection throughout products' life cycles.

In particular, the expansion of health information technology and increased adoption of electronic health records (EHRs) have the potential to dramatically decrease the costs and time it takes to bring products to market.

Clinical trials are the gold standard of medical evidence. They are also the single largest contributor to the cost and length of product development. The key to facilitating innovation of new drugs and devices is to collect the information faster and cheaper, and ensure patients, providers, regulators and payers have the data they need. Registries, large databases that collect information over time on a group of patients treated for a particular medical condition, are one way to accomplish this.

We should seek to conduct clinical trials of the sort done by researchers in Europe studying heart attacks. They conducted a "registry-based randomized clinical trial" involving more than 7,000 patients, and—in unprecedented fashion—were able to keep track of every patient throughout

the course of the research. This study (the TASTE trial) only cost $300,000, roughly $50 per patient. Conducting such a study outside of a registry in the United States would cost hundreds of millions of dollars, if not more.[1]

Similarly, registries are used to identify problems with approved products. Registries can assess the real-world performance and long-term outcomes of medical devices that may not be detected in the clinical trial settings. Hip implants, for example, are expected to last 15-20 years[2] but typically require only two years of clinical data for FDA approval.[3] Demonstrating the ability of registries to detect problems, the Australian Orthopaedic Association National Joint Replacement Registry showed in 2007 that metal-on-metal hips—introduced in 2003 for younger patients needing hip replacements—failed at a rate more than two times higher than conventional hips,[4] leading to a worldwide recall. Registries are a central pillar in FDA's national medical device postmarket surveillance plan.[5]

Registry barriers must be overcome

Within the next few weeks, Pew will release the findings of a series of meetings that brought together medical device stakeholders to better define the role of device registries in our healthcare system. These meetings—hosted jointly by Pew, the Blue Cross and Blue Shield Association and the Medical Device Epidemiology Network Infrastructure Center at Weill Cornell Medical College—included representatives from device companies, FDA, clinical societies, payers and patients groups.

We concluded that registries should be established to collect evidence for those devices for which we do not have good data on their long-term performance, those where physicians and patients have a variety of choices, and those where the outcome may be dependent on surgical technique.

We also developed recommendations on necessary conditions to ensure that registries deliver timely, actionable information to all stakeholders, including the public. We recommend that registry findings and reports should be publicly released on a regular basis, and that the governance, operations, and financing should be made publicly available. FDA, the Centers for Medicare & Medicaid Services (CMS) and other stakeholders should encourage the use of registries that meet these criteria.

There are a number of challenges that must be overcome to enhance the use of registries in the United States today.

First, despite the dramatic uptake of electronic health information sources, these systems cannot easily transmit data among one another. This lack of interoperability, for example, hinders the ability for registries to extract clinical and outcomes data from EHRs. Instead, registries must develop the ability to extract information from the EHR systems at each facility, or require manual entry from providers. We urge the Committee to lend its full support to interoperability efforts by the Office of the National Coordinator for Health Information Technology and elsewhere.[6]

Additionally, many registries have sought clarity on when their studies are considered research or quality improvement efforts.[7] This confusion has slowed their use by hospitals and their ability to make a meaningful contribution.

Other tools can provide key data

In addition to registries, several other new data collection tools can provide critical information on the performance of new drugs and medical devices.

One such tool is the Sentinel Initiative, which can be used to evaluate the safety of drugs and biologics used in patient care. Congress instructed FDA to create this Sentinel program in 2007, and it has since been used both to identify safety concerns with products and to disprove suspected problem. For example, FDA utilized the Sentinel program to identify a correlation between a blood pressure medicine and intestinal problems.[8]

Given Sentinel's successes, Congress instructed FDA in 2012 to expand this system to medical devices. However, Sentinel relies primarily on data derived from health insurance claims. These claims currently lack any information on the specific devices used in care.

To resolve this problem, claims should include information about the specific devices implanted in patients. A new unique device identifier (UDI) system established by the FDA at the direction of Congress was designed with this purpose in mind. In 2007, Congress ordered FDA to create this UDI system to provide each medical device with a unique code corresponding to its make and model.[9] Medical device makers are now adding this code to their products. However, to be effective, it is important that health insurance claims include this code.

Documenting UDI in claims can also bolster other efforts to utilize data to better understand device performance. For example, incorporating UDI in claims will also provide payers—including CMS—with the necessary data unavailable elsewhere to evaluate outcomes for patients with devices.[10] As Medicare and Medicaid pay billions annually for health services involving devices, they should know what products they are purchasing and have the information necessary to make better coverage and reimbursement decisions.

Adding a UDI field to claims has garnered support across the health system—including from hospitals, health plans, physicians, patients, and consumers. Aetna, Mercy, Geisinger Health System, the American College of Cardiology, the Society of Thoracic Surgeons, Premier, Trust for America's Health, AARP, and many other organizations have expressed their support for documenting UDI in claims.[11] Secretary of Health and Human Services Sylvia Burwell also articulated some of these benefits during the Senate confirmation process [12]

New initiatives can leverage these tools

Through the development of these new tools, FDA, patients and clinicians can have confidence that problems with new medical products will be quickly identified. As previously stated, this confidence can enable FDA to expedite patient access to new products, such as by shifting some of the data collected premarket to after approval for technologies that fill serious, unmet medical

needs. These principles are at the heart of recent FDA proposals intended to expedite patient access to new medical devices.[13,14]

The success of expediting access by shifting data relies on the prompt collection of postmarket information. Often, despite current FDA requirements for manufacturers to conduct postmarket trials, commencement of those studies is delayed. For example, in May 2011 FDA responded to concerns of high failure rates with metal-on-metal hip implants by ordering manufacturers to conduct postmarket studies assessing adverse events associated with the products. Despite that order, by June 2012 postmarket study plans for less than one-quarter of metal-on-metal hip products were in place.[15] These types of delays will undermine efforts to shift premarket data to the postmarket setting.

Additionally, FDA must have the ability to quickly withdraw approval for a device if the necessary postmarket data are either not collected or demonstrate that the product does not meet the agency's approval standards. While FDA has the ability to take administrative actions to withdraw approval, removing products from the market can still take several months—if not longer. In the interim, patients may continue to be exposed to products whose risks outweigh their benefits.

FDA—and Congress—should evaluate whether FDA has sufficient authorities to promptly withdraw product approvals if the necessary data are not promptly collected or suggest that the product benefits do not outweigh risks. Congress should also ensure that FDA can fully implement its medical device postmarket surveillance plan, including through the adoption of UDI across the health care system.

Should FDA lack any of these authorities, Congress should provide the agency with enhanced abilities to protect the public through robust postmarket surveillance.

Conclusion

Expediting patient access to new cures requires a holistic view of the product life cycle. New mechanisms to collect data both prior to and after FDA approval can help facilitate faster clinical trials and ensure that any problems are promptly identified.

Given the proven value of electronic health information and registries, Congress should work with the Administration to maximize the potential of these data sources to expedite patient access to safe and effective medical products.

Thank you again for the opportunity to testify, and I welcome your questions.

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[1] M.S. Lauer and R.B. D'Agostino, "The Randomized Registry Trial—The Next Disruptive Technology in Clinical Research?" *New England Journal of Medicine* 369, no. 17 (2013): 1579, doi:10.1056/NEJMp1310102.

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Mr. PITTS. The chair thanks the gentleman, and now recognizes Dr. Jacques 5 minutes for an opening statement.

STATEMENT OF LOUIS JACQUES

Mr. JACQUES. Chairman Pitts, Ranking Member Pallone, and members of the subcommittee, my name is Louis Jacques. From October 2009 through February 2014 I was the Director of the Coverage and Analysis Group at the Centers for Medicare and Medicaid Services. I was the Division Director in that group from June 2004 until 2009.

We implemented coverage with evidence development and the FDA/CMS Parallel Review Pilot Initiative. We also revised CMS regulations pertaining to Medicare coverage and FDA-approved investigational device exemption clinical trials, and executed a memorandum of understanding between FDA and CMA.

CMS experience over the past decade is illustrative of the challenges to the wide adoption of certain innovative technologies. One, there are innovative products and services that do not clearly fall within the statutory scope of the Medicaid program benefit. Two, the available evidence at the time of initial marketing may not clearly establish the clinical value of a new technology in the relevant beneficiary population. Three, historic coding paradigms can be uninformative to the extent that the insurer cannot identify the specific item or service for which it is paying. This blind buying creates reluctance among insurers and hampers the establishment of brand value for high performing technologies.

I believe there are opportunities. External stakeholders have requested more opportunities for coverage with evidence development and FDA/CMS parallel review. While these programs were articulated in the early 2000s by a prior Administration, both are included in the 2012 White House National Bio Economy Blueprint.

Since 2009, CED has essentially replaced non-coverage in final national coverage determinations, thereby furnishing Medicaid coverage when it would otherwise have not been available. By contrast, in the 5 years before 2009, almost half of all national coverage determinations ended with non-coverage.

Unfortunately, CMS' ability to furnish CED is limited. CMS initiates CED under ARC's statutory authority. CMS implements CED through the formal national coverage determination process. Due largely to staffing cuts the annual number of NCDs published has dropped from approximately 12 to 13 in fiscal years 2007 and 2008 to only five in 2012 and six in 2013. Current staffing is approximately half of 2007 levels.

Under parallel review, both FDA and CMS maintain their separate standards. I have no reason to believe that either agency has toughened its process as a process of parallel review. While the structure of the pilot contemplates the possibility of a national coverage determination, parallel review does not inherently require that CMS undertake the NCD process. The content of the parallel review engagement depends on the product's development stage. Ideally, early discussions with CMS could result in more persuasive pivotal trial, evidence that leads local Medicare contractors to uniform coverage.

Results to date are encouraging. One product received unanimous yes votes and positive comments at its recent FDA panel meeting, which the company credited to the discussions with both agencies that inform the design of the pivotal trial. CMS does not have sufficient staff to match FDA's bandwidth on potential parallel review candidates. Despite interest from device manufacturers, the parallel review pilot has been limited to only two products.

In conclusion, CMS review of clinical trials serves three goals. First, it provides important financial support for innovation. Second, the sponsor can obtain CMS feedback on whether the initial trial design could persuasively inform a coverage decision. Third, CMS can inform the sponsor of existing coding or payment paradigms that may apply to the product.

The current vehicles for coverage in clinical trials are unnecessarily siloed, preventing the publication of an integrated, comprehensive policy. I believe this could be fixed with small changes in statute. The definition of a local coverage determination could be revised to align LCD authority with the actual scope of local contractor claims processing responsibility, thereby enhancing transparency and predictability. As an alternative to non-coverage, some stakeholders have expressed interest in new payment paradigms for early stage devices with immature evidence bases.

Acknowledging the challenges of the Federal administration budget, stable funding sources should be considered for these initiatives that are expected to produce downstream benefits. Their investment requires funding that is more predictable potentially from the Medicare Trust Fund itself.

Thank you for the opportunity to share my thoughts, and I would be happy to answer any questions.

[The prepared statement of Mr. Jacques follows:]

Testimony of Louis Jacques, MD
Senior VP and Chief Clinical Officer, ADVI
Before the U.S. House of Representatives
Energy and Commerce Subcommittee on Health Hearing

"21st Century Cures: Barriers to Ongoing Communication and Evidence Development."

July 22, 2014

Chairman Pitts, Ranking Member Pallone, and Members of the Subcommittee, my name is Louis Jacques, and I am testifying as an individual with experience on the topic of this hearing. From October 2009 through February 2014, I was the Director of the Coverage and Analysis Group at the Centers for Medicare & Medicaid Services. I was a division director in that group from June 2004 until my appointment as the group director. During my tenure there we implemented Coverage with Evidence Development (CED) and the FDA – CMS Parallel Review pilot initiative. We also revised CMS regulations pertaining to Medicare coverage in FDA approved Investigational Device Exemption (IDE) clinical trials, and executed a memorandum of understanding between FDA and CMS. I am currently a Senior Vice President at ADVI, where I am also Chief Clinical Officer and a partner. ADVI has offices in Austin, San Francisco and Washington DC. ADVI's mission is to help healthcare companies and organizations develop and articulate evidence that is informative and persuasive for patients, practitioners and public and private healthcare payers.

Background

While CMS has consistently expressed a desire to support evidence based medical technology innovation, this goal would be better accomplished if CMS had clearer authority and greater administrative agility in Medicare coverage and payment for innovative technologies that are in the adolescent phase of their overall product life cycle. This would allow CMS to establish and implement a clearer and more predictable paradigm for coverage of certain technologies that may receive FDA approval or clearance despite the lack of sufficient evidence relevant to the Medicare beneficiary population, in particular the elderly who have multiple comorbid medical conditions.

The historic practices of many medical technology developers reflect insufficient knowledge and attentiveness to clinical questions that are relevant to patient care and health insurance. While this issue is not unique to a particular product category, it can be a particularly vexing challenge in the medical device sphere where the "garage based inventor with a good idea" ethos coexists in the same space with large comparatively more sophisticated multinational firms. This leads to interactions with insurers that can be mutually frustrating, more so if the manufacturer claims enhanced clinical or economic value for a device cleared under 510(k) as substantially equivalent

to a predicate device. The ultimate accuracy of these claims could be addressed with a clinical study but a small company may have limited funds to support additional development or research by the end of its FDA review. I believe that the opportunity for earlier engagement with representative public and private insurers would help these companies make better informed choices at earlier stages in product development, before they commit more resources to a strategy that would predictably fall short.

Medicare is not the only relevant insurer, but the difference between FDA's regulatory standard (safe and effective) and Medicare's overarching standard (reasonable and necessary) is a frequent discussion topic. While this difference is appropriate since the agencies have distinctly different mandates, both agencies share broader national goals to improve public health and protect beneficiary access to those products and services that demonstrate genuine benefit. As a practical matter, FDA approval for drugs and biologics, devices and diagnostic tests puts the product on the store shelf, but prudent purchasers should not be expected to reflexively buy every stocked item without regard to their own needs and priorities.

CMS' experience over the past decade is illustrative of the factors that may constrain the wide adoption of certain innovative technologies. Several of these factors relevant to Medicare coverage and payment are illustrated below. While there is significant alignment among payers on the need for pertinent clinical evidence, commercial or other governmental health insurers may have different flexibility on other factors.

1. There are innovative products and services that do not clearly fall within the statutory scope of the Medicare program. Early engagement with CMS could help companies anticipate this issue and develop better strategies.

The Social Security Act (the Act) establishes the scope of the Medicare benefits under parts A and B. These 50-some "benefit categories" include items and services such as inpatient hospitalization, drugs administered incident to a physician service, durable medical equipment (DME), physician care, etc.

Medicare pays for external drug pumps under the DME benefit. An innovative external drug pump may have characteristics that place it outside the statutory definition of DME.

Medicare does not cover "vaccines" except in certain circumstances such as influenza and pneumococcal immunizations. Thus, certain cancer immunotherapies may unnecessarily pose questions of their inclusion in the Medicare benefit, particularly if they are described as "vaccines" in the press.

A smartphone based technology could be excluded because smartphones are not medical devices. This limits Medicare's ability to consider coverage and payment for applications (apps) that could potentially take the place of certain physician or provider services that currently entail physician supervision.

2. The available evidence at the time of initial marketing does not clearly establish the clinical value of a new technology in the insurer's population of interest. CMS has tried to address this

issue with its Coverage with Evidence Development (CED) initiative, but there have been impediments to the more agile and efficient implementation of CED.

Medical device trials are generally much smaller than drug trials, and often exclude populations of interest from enrollment. Commonly, older patients with multiple comorbid conditions, i.e. typical Medicare beneficiaries, are not well represented in clinical trials done for FDA approval. Under the 510(k) paradigm some devices may be cleared for marketing with no relevant clinical trial evidence at all.

Lumbar artificial disc technology is a good example. The pivotal clinical trials excluded subjects over age 60, and persons with osteoporosis. Given the advanced aged and predominance of women among Medicare beneficiaries, the evidence base could not be reasonably applied to the core Medicare population.

Clinical trials often employ outcomes that poorly identify the ultimate impact on the patient. These may be only short term outcomes for devices that are intended to last for years, nonclinical performance targets or potentially misleading surrogate laboratory outcomes that poorly reflect the patient experience of illness. Other significant limitations include small sample sizes, absence of randomization or adequate controls, and additional sources of bias that limit the persuasiveness of the reported results.

3. Historic coding paradigms can be uninformative to the extent that the insurer cannot identify the specific item or service for which it is paying. This "blind buying" creates reluctance among insurers, and prevents the establishment of brand value for higher performing technologies.

Molecular diagnostic tests are the clearest example of this practice, in which claims for payment historically comprised "stacks" of nonspecific technical procedures performed in the processing of the test sample. The recent Protecting Access to Medicare Act of 2014 (PAMA) legislation addresses this issue with a requirement for granular, product specific coding and a new payment calculation for "advanced diagnostic tests" that meet certain criteria. PAMA creates an incentive to invest in higher performing technologies that can be favorably covered and paid based on evidence of enhanced value.

Despite the newness of these provisions, I am aware of some interest in the venture capital community that a similar paradigm could be applied to other innovative technologies that meet consistent and transparent prespecified requirements. This could address a common complaint that new technologies are billed with nonspecific or temporary codes that some stakeholders believe dissuade adoption by physicians and hospitals.

Opportunities

A. There is significant stakeholder interest in expanding the CMS initiatives that support medical technology innovation but CMS has limited capacity to respond.

External stakeholders have told me they want more opportunities for Coverage with Evidence Development (CED) and FDA-CMS Parallel Review, as well as interaction with private payers under a neutral umbrella.

A recent example of CED is the 2012 decision to cover transcatheter aortic valve replacement (TAVR) in the context of national registries and clinical trials. CMS, with a joint formal request from the American College of Cardiology and the Society of Thoracic Surgeons, established predictable Medicare coverage for current and future FDA approved indications of TAVR; as well as coverage in future clinical trials for unlabeled indications. The resulting data after one year prompted FDA to expand the label for TAVR without the need for an additional clinical trial.

Unfortunately, CMS' ability to engage is limited by historic interpretations of its authorities, and by severely reduced resources in the Coverage & Analysis Group (CAG) that oversees these initiatives. Under current statute, CMS only initiates CED under AHRQ's authority: 1862(a)(1)(E) of the Act, which references AHRQ's authority to conduct Medicare research under section 1142. CMS only implements CED through the National Coverage Determination (NCD) process. Due largely to staffing cuts, the annual number of NCDs published has dropped from approximately 12-13 (FY 2007 and 2008) to 5 (FY 2012) and 6 (FY2013). Competing agency priorities, e.g. expanded coverage of prevention, further limit the application of NCD assets to innovative technologies and CED.

Similarly, with limited resources CMS cannot match FDA's bandwidth on potential parallel review candidates. Despite expressed interest from device manufacturers, the parallel review pilot has been limited to only two participants. CMS also does not have a counterpart to FDA's Entrepreneurs in Residence (EIR) program to bring in-house experience from private payers, outside innovative thinkers, etc.

B. *Responding to stakeholder input, CMS recently revised its regulations regarding coverage of items and services in FDA approved Category B IDE (investigational device exemption) clinical trials.*

While CMS approval is not required to conduct IDE trials, those manufacturers who choose to bill Medicare for trial costs must request coverage. Manufacturers had noted inconsistencies and inefficiencies in the historic paradigm that required separate coverage requests and approvals from each local Medicare contractor. CMS in the CY2014 Physician Fee Schedule regulation established basic criteria and a centralized application and review process.

This new process serves three complementary goals. First, it provides important financial support for approved research studies. Second, the sponsor can obtain CMS feedback on the design of the trial, especially on the inclusion of subjects who are representative of the targeted Medicare beneficiary population and the relevance of the proposed outcomes to meaningful changes in patients' experience of illness. Third, CMS can clarify any assumptions that the manufacturer may have about benefit category, coding, payment bundles etc. that may impact the financial projections that inform investors.

The successful implementation of this initiative (effective date January 1, 2015) depends on CAG having adequate resources (staff and budget) to quickly review IDE protocols and publish a real time list of approved trials.

C. *The innovative CMS MolDX pilot established granular coding, coverage, and payment determination for molecular diagnostic (genomic or proteomic) tests. Recent PAMA legislation has codified in statute the core principles of the MolDX pilot, while also requiring the use of the Local Coverage Determination (LCD).*

There are well over 1000 MolDX tests purported for clinical use. For many tests there are multiple versions developed by different laboratories and based on different underlying technologies (platforms.) The published medical literature and public testimony inform us that the performance of these tests, even those marketed for the same purpose, varies in meaningful ways. It is reasonable to expect that insurers would and should recognize the higher value tests with more favorable coverage and payment. We are aware of estimates that over 500 new MolDX tests are developed every year.

We also recognize that many of these tests (home brews – laboratory developed tests) have been marketed without review by FDA. In general, the available evidence of clinical utility (actual impact on the patient if treatment decisions are based on the test result) is uneven, especially for tests that claim to predict distant outcomes. The ultimate clinical value of these tests will be determined with prospective evidence from real world use. Some of these important questions could be answered with Coverage with Evidence Development (CED), but there is no clear pathway for local CED via the LCD process. Thus a Medicare contractor acting appropriately on currently available evidence might noncover a MolDX test that could, with a more mature evidence base developed over time, have proven to be ultimately beneficial.

The statutory definition of the LCD in 1869(f)(2)(B) of the Act describes it as a coverage determination under 1862(a)(1)(A) of the Act. Thus the LCD vehicle is not currently available for CED, which is currently articulated under 1862(a)(1)(E) of the Act. Interestingly the definition of a National Coverage Determination in 1862(l)(6)(A) is broader; "a determination by the Secretary with respect to whether or not a particular item or service is covered nationally under this title."

In light of the PAMA provisions requiring the use of the LCD for MolDX test coverage, a clear path to local CED could streamline the process for diagnostic test coverage with significant benefits to innovators and CMS alike.

Conclusions and Recommendations

I. CMS needs unambiguous authority to review clinical trials when claims related to these trials will be submitted for Medicare payment. The current vehicles for coverage in clinical trials are unnecessarily siloed, preventing the publication of an integrated comprehensive policy to deal with 1) costs for routine clinical care in trials (currently under a White House Executive Order from the end of the Clinton administration); and 2) costs of the investigational care itself, including related clinical care (currently under CED or the IDE regulation.) The status quo does not clearly establish a prospective route for coverage and payment of investigational care in other settings, i.e. clinical trials beyond CED and FDA Category B IDEs.

There are many potential approaches to this issue. Some are noted below, but this is not an exhaustive list.

- The research authority in 1862(a)(1)(E) could be extended to CMS with or without preserving AHRQ authorities in parallel.
- 1862(a)(1)(A) could be amended to explicitly include items and services furnished in CMS approved clinical trials.
- A distinct new section could establish a singular broad CMS authority related to Medicare coverage and payment in approved clinical trials.

II. The definition of a Local Coverage Determination could be revised to permit LCDs to be used as determined by the Secretary within the scope of Title XVIII. This would align LCD authority with the actual scope of local contractor claims processing responsibility. With more flexible LCD authority, contractors could write LCDs to establish CED as an alternative to noncoverage for various technologies.

III. Some stakeholders have expressed interest in new payment paradigms for early stage devices with immature evidence bases. As an alternative to noncoverage, such devices could be covered but paid at a lower rate initially, for a predetermined period of time, while evidence is being collected. Payment rate increases and possibly "premium" payment levels could be attainable if the additional evidence demonstrates prespecified enhanced patient-centered value. This would align the interests of the developer, the insurers, patients and healthcare professionals to provide earlier access to new technologies while also answering important clinical questions quickly and efficiently. Such a "rapid learning" paradigm would identify both truly beneficial technologies as well as those that ultimately prove to be disappointing in subsequent use.

After a predetermined period of time the payment amount could gradually fall to a prespecified percentage of the premium price. This recognizes that a technology does not remain innovative forever, and returns resources to the payment system to support subsequent innovative technologies.

IV. The implementation of these initiatives requires stable funding and reasonable alignment of resources with the workload. The Coverage and Analysis Group has been decimated by successive cuts in staff and budget. Current staffing is approximately half of 2007 levels. Approximately one-quarter of the staff was lost to retirement, reassignment and resignations during the sequester and could not be replaced. The frequent inability to recruit external candidates has stymied a more strategic needs-based approach to staffing. Alternative funding could be considered, possibly from the Medicare Trust Fund or other sources.

Thank you for the opportunity to share my thoughts and I would be happy to answer any questions.

Mr. PITTS. The chair thanks the gentleman.
Mr. Mussallem, you are recognized for 5 minutes.

STATEMENT OF MICHAEL A. MUSSALLEM

Mr. MUSSALLEM. Yes. Thank you very much, Mr. Chairman Pitts, Ranking Member Pallone, Congresswoman DeGette, and members of the subcommittee. My name is Mike Mussallem. I am the chairman and CEO of Edwards Life Sciences. I am truly honored to join the other panelists today to discuss the path to revitalizing medical device innovation in the United States.

I and the other employees of Edwards Life Sciences, from our engineers to our valve assemblers, share a passion for helping patients. I am privileged to lead a company that is the world leader, and has been for 50 years, in heart valve replacements.

The reason I am here is that I am worried about innovation in the U.S. and that it is suffering from increasingly costly, cumbersome, and risk averse culture in our regulatory and payment systems. Our recent experience with a transformative therapy to heart valve replacement patients gives us a unique perspective on the current climate.

In short, Edwards Technology allows a heart team to deliver a collapsible prosthetic valve through a catheter into the body to avoid cracking the chest, stopping the heart, and avoid the long and painful recovery that goes along with that open heart surgery.

This has become the most extensively studied heart valve ever, including an unprecedented four New England Journal of Medicine articles that demonstrated a triple win, which is a substantial and sustainable clinical effect, cost effectiveness, and extraordinary quality of life improvement.

We appreciated a productive relationship with Dr. Jeff Shuren in FDA, as well as Dr. Patrick Conway and his colleagues at CMS, whose approach ensured that there was a balanced and reasonable process for this transformative therapy.

Also in a remarkable effort of groundbreaking collaboration between medical societies, regulators, and other stakeholders, we built a comprehensive clinical evidence and quality measurement tool for this therapy called the TBT registry.

But there is room for improvement. We all know the path to approval and reimbursement is not easy, and it should not be. Yet the U.S. approval of this American technology trailed Europe by 4 years. We are pleased that the FDA leadership viewed this delay as a catalyst to improve, and we see several opportunities to remove barriers. I am going to focus on three.

Number one, evidence development mechanisms can be improved to reduce cost and delay. FDA had recently proposed a number of improvements to the pre-market clinical trial process and the post-market surveillance process that hold the promise. And these have been discussed at this committee. In my view, when registries are done right, they can yield extremely useful information about patients' outcome and device benefits.

However, the clinical and scientific benefits of registries must be balanced with a potentially significant cost burden, complexity, and potential misuse of that data. In our case, many physicians told us

it takes longer to fill out the 300 fields in the TBT registry than it does to perform the procedure itself.

Number two, reimbursement incentives need to be aligned with promoting innovation. Efforts to curb healthcare spending could have the unintentional consequence of slowing down innovation in our cost-cutting frenzy. It is imperative to recognize that medical device innovations become more effective and more efficient with time and with experience. We need a system that does not shut the door to reimbursement on day one.

In select cases, coverage with evidence development can be a tool that allows promising technologies to reach patients sooner while developing evidence to support lasting reimbursement. And finally, FDA's vision to improve the regulatory process must be accelerated. Dr. Shuren and his team have outlined strategic priorities that strike the right balance between pre-market and post-market data collection and improving customer service.

We know FDA is a complex bureaucracy to manage, and our leaders need a mandate to change more quickly. Congress could encourage FDA by providing additional support to expedite these changes and give them room to innovate.

And finally, no discussion about medical technology is complete without understanding the true impact that they have on patients. And we meet a lot of patients. To mention one, Lester Tenney, a true American hero, part of our Greatest Generation, survivor of the Bataan Death March, and a Japan POW, had long sought an apology from the Japanese government on behalf of Federal soldiers. Unfortunately, just as this apology was agreed upon, he was diagnosed with disabling and inoperable aortic stenosis. He would not survive long, let alone long enough to make this trip.

The good news is that Lester received an Edwards trans-catheter heart valve, was able to travel to Japan, get the apology. This would not have been possible even 5 years earlier. And he remains vital to this day and dedicated to helping veterans. Lester and tens of thousands of other patients we have had the opportunity remind us every day that our work is personal. It impacts people individually.

Thank you for the opportunity to testify today.

[The prepared statement of Mr. Mussallem follows:]

Edwards

Testimony of Michael A. Mussallem
Chairman and CEO, Edwards Lifesciences
before the
U.S. House Committee on Energy and Commerce, Subcommittee on Health
Hearing on
"21st Century Cures: Examining Barriers to
Ongoing Evidence Development and Communication"
July 22, 2014

Introduction

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

I am Mike Mussallem, chairman and CEO of Edwards Lifesciences, based in Irvine, California. I am truly honored to join my fellow panelists today to discuss a path to revitalizing medical device innovation in the United States.

I am here because I am passionate about helping patients. That's why I and hundreds of thousands of U.S. medical device company employees like me come to work each day. We love what we do because it can have such an amazing impact on Americans' quality of life.

Based on Edwards' experience in developing and delivering new therapies to American patients over the last several decades, I am very concerned that we are seeing an alarming and documented decline in U.S. medical innovation[1], as this Subcommittee has heard previously. The balanced ecosystem that has supported innovation in the U.S. is being eroded by an increasingly costly and cumbersome risk-averse culture in our regulatory and payment systems.

Our recent experience with the development of an innovative heart valve replacement therapy, which enables a team of physicians to replace a patient's aortic heart valve without open-heart surgery, has provided us a unique perspective on the current regulatory process. During the last decade, as we have navigated the regulatory channels to bring this therapy to U.S. patients, we have

[1] National Venture Capital Association. (2014). *NVCA 2014 Yearbook.* Arlington, Virginia: Thomson Reuters.

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taken note of not only the challenges, but also the forward-looking vision of the leaders of FDA and CMS to develop opportunities for better collaboration with the agencies. We believe there are several opportunities to remove barriers in regulatory approval and reimbursement that will help promote America's continued worldwide leadership in the area of medical device development. While we have a number of recommendations for improvements that could be made, today I will focus on three primary areas:

- Evidence development mechanisms can be improved to reduce costs and delays.
- Economic incentives need to be aligned with promoting innovation.
- FDA's vision to improve the regulatory process must be accelerated.

Our Unique Perspective

Over the 35 years I have spent working in medical devices, I have had the opportunity to be involved with the development of dozens of innovative therapies for the treatment of heart valve disease and the critically ill. I am privileged to lead the more than 8,700 employees of Edwards Lifesciences, who dedicate their lives in a very personal way to helping patients around the world. We have been the leaders in heart valve innovation for more than 50 years, starting when an engineer, Miles Lowell Edwards of Santa Ana, California, partnered with a cardiac surgeon, Dr. Albert Starr of Portland, Oregon, to develop the first commercially available artificial heart valve. I have also had the honor of representing our industry in a number of leadership roles, noteworthy among them my term as chairman of the Advanced Medical Technology Association (AdvaMed).

It is my experience that successful medical device innovators keep an unwavering focus on patients. We count it a privilege to serve these patients, creating and supplying devices and therapies that save, enhance and prolong lives. We are the toolmakers for clinicians, working closely with them to develop technologies to address unmet patient needs. Each new innovation is also a stepping stone that lays the path to something even better. Innovation is a powerful and iterative force, and

those who are involved in it are never satisfied with the status quo. It is our passion and mission to keep finding better solutions to improve human health.

Edwards' innovation story is similar to many companies that have made medical technology a uniquely American success story. In just the most recent decade, between 2000 and 2010, medical advancements helped add nearly two years to U.S. life expectancy[2]. Specifically, fatalities from heart disease were cut by a third; deaths from stroke were reduced by more than a third; and mortality from breast cancer was cut by almost a fifth[3]. Medical technology has been a strong contributor to the U.S. economy, responsible for about 1.9 million U.S. jobs, including both direct and indirect employment, and nearly $150 billion in direct economic output (sales)[4]. Clusters of innovation in states like California, Texas, Minnesota, Massachusetts, New York and North Carolina, are responsible for addressing the world's most serious health challenges, while at the same time serving as a robust economic engine, providing attractive U.S. jobs and economic growth far into the future.

The success of these companies, and the existence of these clusters, is not by happenstance. There are a few essential elements to fostering an ecosystem that incentivizes curiosity and rewards innovators who develop new therapies for patients:

- Patient/physician need
- Ready access to capital and supportive economic climate
- Functional/timely/predictable regulatory processes
- Reimbursement system that welcomes novel therapies as they undergo a continuous improvement process
- Strong intellectual property protection

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[2] National Center for Health Statistics. "Health, United States, 2012: With Special Feature on Emergency Care." Hyattsville, MD. 2013.
[3] Ibid.
[4] "The Economic Impact of the U.S. Advanced Medical Technology Industry," Battelle Technology Partnership Practice, March 2012.

Advancements in medical technology can also yield savings across the health care system by replacing more expensive procedures, reducing hospital stays and allowing people to return to work more quickly. Between 1980 and 2010, advanced medical technology helped cut the number of days people spent in hospitals by more than half[5].

Edwards Lifesciences has been at the forefront of an extraordinary opportunity to impact the lives of patients suffering from a deadly heart valve disease called aortic stenosis. The Edwards SAPIEN transcatheter aortic heart valves deliver a collapsible prosthetic valve into the body via a catheter-based delivery system. The valve is designed to replace a patient's diseased native aortic valve while the heart continues to beat – avoiding the need to saw open the patient's chest, connect them to a heart-lung machine, and stop the heart. Those of you who have a friend or relative who's had open-heart surgery know first-hand how difficult this procedure and its arduous recovery can be. Our new heart valve allows patients to avoid that pain and suffering.

Some patients who receive the SAPIEN transcatheter valves can leave the hospital and go to their own homes the next day. It's extremely gratifying to hear physicians and patients describe the immediate improvement in patients' health after TAVR. They can breathe and speak more easily, their skin transforms from gray to pink as their vital organs once again receive the oxygen-rich blood they need, and their vibrancy returns within hours. In reporting the results in 2010 of a quality-of-life sub-study with the SAPIEN valve, David J. Cohen, M.D., M.Sc., Director of Cardiovascular Research at St. Luke's Mid America Heart and Vascular Institute, said, "The degree and immediacy of the quality of life improvement was striking, with significant benefits seen as early as one month. By one year, patients experienced both cardiovascular and physical health benefits, with the physical improvements roughly

[5] National Center for Health Statistics. (2013, March 14). Table 103 – Discharges, days of care, and average length of stay in nonfederal short-stay hospitals, by selected characteristics: United States, selected years 1980 through 2009-2010. Retrieved March 15, 2013, from Centers for Disease Control and Prevention: http://www.cdc.gov/nchs/data/hus/2011/103.pdf.

comparable to a 10-year reduction in age. Quality of life is critically important, particularly for patients like those in this trial – and they are not just surviving, but also thriving."[6]

Patients receiving the Edwards SAPIEN valve go home with potential years of good health added on to their lifespan. Extensive study of this valve – including an unprecedented record of four *New England Journal of Medicine* papers – has demonstrated the "triple win": a substantial and sustainable clinical benefit, extraordinary quality-of-life improvement, and cost effectiveness in inoperable patients. In fact, the SAPIEN valves are the most studied heart valve in history, with more than 300 peer-reviewed, published articles on clinical outcomes associated with the valves. There are also more than 120 cost-effectiveness and quality of life articles related to transcatheter aortic valve replacement (TAVR).

While our experience with the SAPIEN valves and TAVR has ultimately been successful, it is important to reflect on its unique and challenging regulatory pathway, including some key milestones:

- In 1999, Edwards began an internal program exploring transcatheter valve replacement.
- In 2002, Professor Alain Cribier performed the first-in-human procedure of a transcatheter aortic valve replacement in France.
- In 2007, the Edwards SAPIEN valve, our first commercial transcatheter heart valve, received CE Mark for European commercial sale. The next-generation SAPIEN XT valve received CE Mark in 2010.
- Before SAPIEN was approved by FDA, CMS took the unusual step of initiating a National Coverage Determination (NCD) in October 2011.
- Four years after obtaining CE Mark in Europe, the SAPIEN valve was approved by FDA in November 2011 for the treatment of inoperable patients, making the U.S. the 42nd country in the world to approve the device.

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[6] Edwards Lifesciences. (2010). Edwards SAPIEN Transcatheter Heart Valve Demonstrates Substantial Improvement in Quality of Life in Inoperable Patients [Press release]. Retrieved from http://www.edwards.com/newsroom/Pages/NR20101115.aspx

- We achieved an additional regulatory approval in 2014 that means today, U.S. patients benefit from our second-generation device, during approximately the same time that our even more advanced third-generation device was launched in Europe.

As we have continued to innovate new generations of transcatheter heart valves, the U.S. has trailed Europe and other regions of the world in approving these more advanced valves. We believe FDA leadership has viewed this as an opportunity to identify improvements and seek helpful changes to the regulatory system that can improve, and shorten, the approval timeline for future generations of transcatheter heart valve devices.

We've appreciated the productive relationship with Dr. Jeff Shuren and the team at CDRH/FDA, along with Dr. Patrick Conway and colleagues at CMS, whose approach ensured that there was a balanced and reasonable review process for this transformative therapy. At FDA, Dr. Shuren's team worked to develop a post-approval study that allowed us to use registry data to satisfy our postmarket surveillance requirements. While Edwards did not request a formal parallel review process, CMS' early engagement was unique and demonstrated that the agency could move in an expedited fashion. Ultimately, Dr. Louis Jacques on Dr. Conway's team worked to develop a "flexible" NCD, which provides coverage for current *and* future approved TAVR indications and devices – although iterative therapies are best left to clinician judgment using existing payment pathways.

Lessons Learned

Evidence Development Mechanisms Need Improvement

To support regulatory decisions for approval and reimbursement of new medical technologies in the U.S., manufacturers are required to gather a great deal of clinical and economic evidence. Evidence development can be an extremely costly endeavor at each stage of the process. Often the cost to the system and inevitable delays that result are not a critical consideration for regulators. We've invested more than 10 years in just the pursuit of U.S. approval for the SAPIEN platform, dedicating time, resources and significant funding to product development, clinical trials and data collection and analyses. Focus should be put on reducing the delay and expense that data collection adds at every step in the process.

FDA has recently proposed a number of improvements to the premarket clinical trial process that hold promise, many of which have already been discussed by this Committee during previous 21st Century Cures hearings. Some of these improvements that we support include:

- Streamlining the investigational device exemption (IDE) approval process to reduce IDE approval timeframes.
- Reducing the legal complexity and inconsistency between each hospital Institutional Review Board (IRB) through the creation of a centralized or standardized review process.
- Incorporating patients' voices and tolerance for risk into the regulatory decision making process – from clinical trial design to PMA approval review.
- Addressing potentially duplicative clinical evidence through the consideration of surrogate endpoints and greater use of data developed outside of the U.S.

We also see opportunity for innovation on the postmarket side. Under the TAVR NCD, CMS requires that every U.S. patient be enrolled in a qualified prospective registry that tracks appropriate outcomes data to the patient level. In a remarkable effort of collaboration between the medical societies, regulators and other interested stakeholders, the American College of Cardiology (ACC) and

the Society of Thoracic Surgeons (STS) helped build what has become one of the most robust clinical evidence and quality measurement tools ever created: the STS/ACC TVT Registry. In my view, when registries are done right, they can yield extremely useful information about patient outcomes and device benefits. Access to more data more quickly can help patients and clinicians more accurately weigh risks and benefits of a procedure, and also helps inform new physician training and device design. For example, data from the STS/ACC TVT Registry for transcatheter aortic valve replacement procedures were used to follow patients, report on outcomes and ultimately help expand the indications for use of our SAPIEN technology, allowing access to a broader patient population.

However, the clinical and scientific benefits of registries must be balanced with their potentially significant cost, complexity and potential for misinterpretation and misuse of the data. We've seen through the vaccine debate what can happen when misused data, or data in the wrong hands, can keep therapies from helping patients. Too often, well-intended advocates have driven sensational headlines, citing cherry-picked data or anecdotal incidents that have received outsized attention. In clinical trials, sample sizes and statistically based clinical endpoints are created to ensure data cannot be manipulated later. Investigators are blinded to the outcomes until the predefined milestones are met. These basic scientific principles are the cornerstone of clinical research and prevent conclusions that are not statistically supported.

The burden and cost of complying with registry requirements is not insignificant. For example, the patient data registry form for the STS/ACC TVT Registry for transcatheter aortic valve replacement procedures is eight pages long and consists of more than 300 separate fields, requiring special staffing, and dedicated personnel, and hours of work to complete this exhaustive form. Many physicians have told us that it takes longer to fill out the TVT Registry form than it does to perform the procedure. In addition to the significant financial commitment manufacturers must make to support the development and ongoing operations of registries, hospitals are charged ongoing fees to participate.

In a time of extreme budget pressure, we need to ensure that this process is not so costly and burdensome that the long-term prospects of the registry diminish over time.

Because of the potential for registries to increase the costs and burdens of healthcare delivery, we support AdvaMed's position that a number of "threshold questions" should be answered before determining whether a registry is the appropriate mechanism for meeting the defined objective:

- Are there already reliable data collection instruments available to collect the data needed to achieve the objectives?
- Will the registry have a stable and diverse source of funding to promote long-term sustainability?
- Is using a registry the least-burdensome means to collect the necessary data to achieve the scientific objectives?
- Do the objectives warrant the level of investment required to develop and maintain a registry?

In addition, the AdvaMed principles outline several key elements that should guide the development of any medical device registry, including: establishment of a data governance committee to oversee issues on ownership, as well as access to and use of any data collected; prospective registry design, to establish clear objectives and data analysis plans; policies for sharing of data collected with qualified scientific or medical researchers; and policies for the use and publication of registry data.

Economic Incentives Must be Aligned to Incentivize Innovation

Our legal and regulatory framework has created an increasingly challenging environment for innovation over the past several years. Unfortunately, efforts to curb healthcare spending could have the unintentional consequence of sweeping up innovation in a cost-cutting frenzy. For example, accountable care organizations (ACOs) and bundling payment models, while interesting for traditional procedures to treat established diseases, have the potential to incentivize providers to restrict access

to new therapies that may address an unmet patient need. If implemented successfully, such reforms could help ensure that patients receive better-coordinated and higher quality care, while also restraining rising costs. If implemented poorly, hospital value-based purchasing strategies could tilt toward simply restricting access and creating new barriers for patients and physicians as they seek advanced, clinically appropriate care.

It is imperative to recognize that medical device innovations become more effective and more efficient with time, experience and device improvement. If we hold new innovations to the same unforgiving standard that we hold well-established technologies that have been honed to near perfection over decades, we will miss opportunities to help American patients with new and transformational technologies. As toolmakers, we gather a lot of feedback on our first generation technologies, find opportunities for improvement, iterate and make it better. There is a learning curve, and we need a system that takes this into account and does not shut the door to evaluation on day one, while always maintaining patient safety along the way.

One effort that intends to provide earlier access to promising new therapies is Medicare's use of Coverage with Evidence Development (CED). When utilized properly, CED can be a useful tool for our reimbursement system. CED is a mechanism to provide coverage for new technologies that CMS doesn't believe reaches the "Reasonable and Necessary" threshold. However, CMS should be careful that CED does not become more of a burden to patient access than a tool for data development, particularly in cases where sufficient clinical evidence has already been developed – if so, the evidence requirement simply adds unnecessary time and cost.

I've had colleagues at other companies, as well as clinicians, ask me if our rigorous PARTNER Trial did not demonstrate "Reasonable and Necessary," how can we expect other technologies to meet this threshold? While CED has provided CMS with the ability to mandate hospitals submit data to the TVT Registry, any CED mandate should be removed once careful evaluation of the ongoing data supports continued coverage.

FDA's Vision to Improve the Regulatory Process Must be Accelerated

FDA is already taking on a number of initiatives to improve the regulatory processes to help improve patient access to innovative therapies. Thanks to the Food and Drug Administration Safety and Innovation Act (FDASIA), FDA has agreed to improved review and approval performance metrics tied to dramatic increases in manufacturers' user fees, and we are just beginning to see positive performance. Beyond that, during the last few years, Dr. Shuren and his team at FDA have outlined strategic priorities to strengthen the clinical trial enterprise, striking the right balance between premarket and postmarket data collection and improving customer service. Over the past year, a number of guidance documents have been drafted to provide manufacturers and FDA reviewers more clarity, including:

- Priority review for premarket submissions
- IDE and IRB approvals
- Patient preferences and benefit-risk analysis for premarket devices
- IDEs for Early Feasibility clinical studies
- Balancing premarket and postmarket data collection
- Expedited access for certain premarket approval devices

In addition, FDA's expanded efforts to improve device quality and safety by shifting the focus from the old regulatory compliance approach to an upfront quality assurance effort through its "Case for Quality Initiative" is promising. Finally, FDA's efforts to improve its regulatory management processes and structure through the recommendations coming from its Program Alignment Group is an important step in the right direction.

The biggest issue here is that FDA needs the resources and support to move faster on these initiatives. Drs. Hamburg and Shuren have a complex bureaucracy to manage, and they need the mandate to make change quickly. Congress could lend support to FDA by providing additional resources to FDA to help expedite these changes and give them room to innovate.

The TAVR Patient Experience

No discussion about medical technology is complete without understanding the true impact medical advancements have on patients – and we meet a lot of patients. I'd like to share one story of an Edwards SAPIEN transcatheter valve patient I have had the honor of coming to know during the last several years:

Lester Tenney is a true American hero, a part of our Greatest Generation.

As a tank officer in the Pacific Theater in World War II, Lester fought the Japanese in the Philippines before being taken as a prisoner of war in 1942 and forced to participate, along with 78,000 other soldiers, in the 85-mile trek that has since become known as the Bataan Death March.

Lester chronicled his experience in an inspiring memoir, *My Hitch in Hell*.

Having written this book about his unlikely survival, Lester's primary cause has long been the Japanese government's recognition of, and apology for, the suffering experienced by their prisoners of war.

In 2009, after decades of pursuit by Lester, the Japanese government agreed to sponsor a group of former prisoners of war to travel to Japan and receive that apology. The only problem: Lester's heart was giving out. Lester was having chest pain and couldn't catch his breath. The aortic valve in his heart had started to narrow and harden from aortic stenosis. Lester was 90 and had already undergone triple bypass surgery 20 years prior, so his doctors didn't think he could survive another open-heart operation.

Like all other aspects of his life, Lester was tenacious and sought another answer. He refused to accept that nothing could be done to address his aortic stenosis. Through his own research, he found out about TAVR and pursued this treatment option.

Lester had a transcatheter valve replacement in the spring of 2010 as part of a clinical trial at the Scripps Clinic in San Diego, and was discharged less than a week later. As a result of this life-saving procedure, Lester traveled to Japan with a group of six veterans, who met with parliament,

dined with Ambassador Roos, and, in an incredibly important victory, received a formal apology from Japan's foreign minister.

Today, Lester is at work on a new book, *The Courage to Remember*, which has a message of healing – in his case, this means healing from the PTSD of his war-time experiences and also his recovery from TAVR.

Lester and the tens of thousands of other patients we've had an opportunity to help remind us in the U.S. medical device industry daily that our work is personal, and it impacts people individually. Each heart valve represents a patient and their family, who otherwise would miss out on both the extraordinary and precious ordinary experiences of their daily lives.

Our mission is focused and our way forward is clear. I thank Chairman Pitts, Ranking Member Pallone and Members of the Subcommittee for the opportunity to testify today, and to share Edwards' experience in delivering an important new therapy to U.S. patients in need. I applaud the work you are doing with the 21st Century Cures initiative to ensure that U.S. patients continue to benefit from the amazing innovations being developed close to home. We welcome your support to remove the barriers that may prevent patients like Lester from accessing therapy that, in the words of some wise physicians, puts more years in their life, and more life in their years.

Mr. PITTS. The chair thanks the gentleman.

Dr. Schimizzi, you are recognized for 5 minutes for an opening statement.

STATEMENT OF GREGORY A. SCHIMIZZI

Dr. SCHIMIZZI. Chairman Pitts, Ranking Member Pallone, members of the subcommittee, and honored guests, it is a distinct honor to be here today and testify before you. My name is Gregory Schimizzi, and I am testifying before you as a Member of the Board of Directors and past President of the Coalition of State Rheumatology Organizations, or CSRO. And I am a private practice Rheumatologist at the Carolina Arthritis Associates in Wilmington, North Carolina.

The CSRO appreciates the opportunity to share our views related to barriers to ongoing evidence development and communications transparency. Specifically, I will focus on situations in which valid communications pathways are being hampered by outdated practices of the Food and Drug Administration, or FDA, as well as touch upon some unintended consequence of the Sunshine Act, or open payments, as implemented by the Centers for Medicare and Medicaid Services, or CMS.

The FDA does not allow pharmaceutical companies to actively distribute key clinical information even if it is related to the on-label indicated, unless it is explicitly referenced in the package insert of that product. By limiting the sharing of information, physicians are hampered in their ability to gain all of the firm scientific rationale and medical evidence needed to treat patients.

So that clinicians may be better informed, the CSRO urges the FDA to develop standards for qualifying real world data through a public process, to expand the current process of review of materials beyond what is included in the package insert, to also cover other key data, such as sub-population, pharmaco-economic, or comparative cost data, and to ensure a timely review process for such information.

As part of the Affordable Care Act, Congress required the Administration to set up a process by which transfers of value from certain covered entities, primarily manufacturers of drugs and devices to physicians, would be reportable. Such reportable information would then be made publicly available. The overall goal of this transparency is to make particular potential financial conflicts of interest more transparent.

However, there are considerable problems with the current implementation of open payments, including the lack of guidance and clarity regarding the physician registration process, as well as the review of dispute process lacking necessary protection for physicians.

Finally, a recent CMS-proposed rule related to open payments would severely hamper the flow of information. Therefore, the CSRO respectfully requests that CMS provide additional provider-specific guidance for the registration process and adopt policies that allow for flexibility of enrollment requirements so that physicians struggling to enroll remain able to participate in a meaningful manner, ensure an impartial process for disputing the accuracy of financial information intended for public disclosure, take steps to

enhance the fairness and accuracy of the program by ensuring that healthcare providers have access to meaningful mechanism for limiting the distribution of disputed information, and reconsider its proposal to eliminate the continuing medical education exemption, and instead appropriately expand the list of certified CME accrediting or issuing agencies beyond the five currently cited in regulation.

As I hope I have outlined today, current practices at both the FDA and CMS may be inappropriately hampering the exchange of information, making it difficult for physicians to receive the information they need to make valuable treatment decisions.

For the FDA, I hope that Congress will examine ways to allow for more proactive changes among clinicians with appropriate safeguards to ensure that such information is truthful and not misleading. For CMS, I hope that Congress can urge strategic plan programmatic changes to make the transparency process accurate and appropriately descriptive of the financial relationships among the various entities.

Thank you once again for allowing me to speak today and to consider my comments today as well as the other information captured in my written statement. The Coalition of State Rheumatology Organizations looks forward to working with the committee to address these issues. I look forward to your questions. Thank you very much.

[The prepared statement of Dr. Schimizzi follows:]

Gregory F. Schimizzi, MD

Coalition of State Rheumatology Organizations

Testimony for the Record

Before the House Energy and Commerce Subcommittee on Health

Hearing Entitled

"21st Century Cures: Examining Barriers to Ongoing Evidence Development and Communication"

Tuesday, July 22, 2014

Chairman Pitts, Ranking Member Pallone, members of the Subcommittee, and honored guests, my name is Gregory Schimizzi and I am testifying in front of you today as a member of the Board of Directors and Past President of the Coalition of State Rheumatology Organizations (CSRO) and as a private practice rheumatologist at the Carolina Arthritis Associates in Wilmington, North Carolina. The CSRO would like to thank the House Energy and Commerce Subcommittee on Health for taking an in-depth look at innovations in the practice and delivery of medicine and considering how the legislative and regulatory framework should adapt to support improved communication and collaboration. The CSRO appreciates the opportunity to share our views related to barriers to ongoing evidence development, communication, and transparency. Specifically, I will focus on situations in which valid communication pathways are being hampered by outdated practices of the Food and Drug Administration (FDA), as well as

touch upon some unintended consequences of the Sunshine Act, or 'Open Payments,' as implemented by the Centers for Medicare and Medicaid Services (CMS).

For your reference, the CSRO is a group of state or regional professional rheumatology societies formed to advocate for excellence in rheumatologic disease care and to ensure access to the highest quality care for the management of rheumatologic and musculoskeletal diseases. We represent 28 state and regional rheumatology societies in the country. The CSRO's mission is to promote access to the highest quality care for patients with autoimmune inflammatory and musculoskeletal diseases. The CSRO is also a member of the Alliance of Specialty Medicine which represents more than 100,000 practicing specialist physicians in the United States. In addition, I am one of the founding members of Carolina Arthritis Associates in Wilmington, North Carolina, which is a private rheumatology practice with 23 years of service to patients with disabling, disfiguring, inflammatory and destructive autoimmune diseases. I am also a member of the North Carolina Arthritis Association and the American College of Rheumatology.

BACKGROUND

It is the mission of physicians in all specialties to use the safest and most effective means to assist patients in health maintenance, disease prevention, effective disease management and accessing curative therapies. Most of these endeavors are accomplished with the use of treatment modalities that are not only the standard of care but also FDA approved. However, in instances and circumstances where no definitive FDA-approved indication is available, the use of medically accepted alternative uses of approved medicines is often necessary.

Non-approved use of medical products has actually become the standard of care in the treatment of many orphan diseases and also frequently used when standard, accepted treatments fail in common diseases. In my specialty of rheumatology, there are many diseases where little or no scientific or clinical information is present regarding the treatment of certain autoimmune diseases, including Sjögren's syndrome, Behcet's disease, many forms of vasculitis, inflammatory muscle diseases, scleroderma, calcium pyrophosphate deposition disease and other conditions. Given the small patient population, manufacturers may not consider pursuing new indications for a pharmaceutical agent economically feasible since the costs of such endeavors are daunting. Despite the lack of FDA approved indications, those patients still require treatment and, as their physicians, we endeavor to use whatever information is available to help with informed decision-making. For instance, many non-approved indications can be found in standard textbooks of medicine and surgery in all specialties and subspecialties for patients of all ages and are the generally accepted standard of medical care.

The use of medical products, devices and medications is always undertaken using the best available clinical evidence, judgment and consideration with the utmost care, thoughtfulness and regard for patient safety. These decisions take into consideration the patient's comorbid conditions and concomitant medical therapies. In some patients with orphan diseases or illnesses that are poorly understood, non-approved therapies are the only treatments available. Management modalities for these are frequently publicized in scientific meetings, peer-reviewed literature and other compendia. Publicizing these treatments is an important method of communicating effective treatments in the medical community and a source of investigational stimulation to academicians and clinicians into new areas of research and development.

The goals of medicine and medical research in these areas must continue to be the improvement of health of our population, prevention of disease, and the safest and most effective treatment for individuals afflicted with any illness or condition. It is my belief and that of my colleagues that open discussions and distribution of truthful scientific information is a cornerstone to achieve those goals where sound research and data have been completed but it must be shared and distributed.

FOOD AND DRUG ADMINISTRATION

As a member of the Alliance of Specialty Medicine, the CSRO supports the Alliance's recently developed position statement focused on Physician-Directed Applications (also known as "off-label use"), which is included in the appendix of my testimony. One key component of that position statement is that "[i]f specialty physicians use a product for an indication not in the approved or cleared labeling, they have the responsibility: (1) to be well informed about the product; (2) to base its use on a firm scientific rationale and sound medical evidence; and (3) to maintain awareness of the product's use and effects." I agree wholeheartedly with this requirement and would like to highlight some potential problems with recent Food and Drug Administration (FDA) requirements which may hamper my ability as a physician to be well informed about a product and to base its use on firm scientific rationale and sound medical evidence.

My understanding is that the FDA does not allow pharmaceutical companies to actively distribute any key information, even if it is related to the on-label indication, unless it is explicitly referenced in the package insert. Therefore, observational data, subpopulation information, comparative data derived from clinical trials other than randomized controlled trials, and pharmacoeconomic or comparative cost data cannot be proactively shared with clinicians unless such data is directly referenced in the package insert. Further, for medically acceptable alternative uses, such as those which may be referenced in various compendia or practice guidelines as an appropriate treatment, that data can only be shared if a clinician directly and specifically requests such information. By limiting the sharing of information, physicians are hampered in their ability to access all available sound medical evidence and firm scientific rationale necessary to treat patients with difficult problems.

For example, one of our distinguished colleagues attempted to proactively request information to aid in the treatment of a patient with sclerits, which is an inflammatory disease of the eye that can occur in diseases such as rheumatoid arthritis. Left untreated this condition has potentially devastating consequences including complete loss of vision or even perforation of the eye itself. Due to current regulations and limitations that require a physician to explicitly request information, effective treatment of this patient's condition was delayed. This particular patient did not immediately respond to traditional therapy options, but our colleague remembered a presentation suggesting that rituximab may be a suitable physician-directed application. After several failed attempts to contact the speaker, he contacted the pharmaceutical company directly and requested any specific data that the manufacturer possessed relating to this specific potential use. He received the required information, and the product helped his patient. However, the 2-3

weeks required to obtain all of the necessary information, patient consent, and then insurance authorization, caused unnecessary delays in treating his patient and impacted the outcome by delaying access to safe effective care.

It would be preferable to allow the pharmaceutical company with its wealth of information to share key data in order to inform and assist in decision–making. That is not to say that I would recommend a change in all of the current requirements for the FDA to review such information to ensure that it is truthful and not misleading. **The CSRO urges the FDA to expand the current process of review of materials beyond what is included in the package insert to also cover other key data.** The FDA review process should be in real-time and not potentially delayed for a year or more. In addition, **The CSRO urges the FDA, through a public process, to develop standards for qualifying real world data, so that clinicians can be better informed.** With additional comparative effectiveness research, the focus on quality outcomes, and other health care reforms, Congress and the FDA should be encouraging the exchange of scientific information, not hampering it. Blocking access to data on medically acceptable alternative uses seem to countermand these new requirements and complicate my ethical responsibility to provide patients with information on risks, benefits and alternatives to medical treatments as part of the informed consent process. As we move closer to newer, alternative payment models (APMs), where shared decision-making tools will likely be a key component, I am concerned about how this lack of information will impact my ability to truly educate my patients on their options and give them a fair opportunity to engage in the establishment of their care plan.

CENTERS FOR MEDICARE AND MEDICAID SERVICES

Next, I would like to discuss the Physician Sunshine Act, or 'Open Payments,' administered by the CMS. In April, my mother-in-law was pleased to share with me an article on the front page of our local paper vilifying me for inappropriate Medicare payments. The article highlighted that I had received $1.49 M in Medicare reimbursements for 2012. What it failed to disclose and characterize was that those reimbursements not only covered payments for my services but, more importantly, covered the Medicare reimbursements for very expensive medications which my patients received. Thus, despite my mother-in-law's hopes, my salary from Medicare was not $1.49 M in 2012. While I and other physicians similarly mentioned in newspaper articles across the entire country received apologies from CMS regarding the inappropriate use of this information, I am not sure if Congressional members realize all of the unintended consequences and mischaracterizations that may result from the release of such information or how easily this information can be misused. I realize that my example is not directly related to the Open Payments program, but I wanted to highlight this situation to Congress before the public release of the Open Payments on September 30, 2014. I fear that similar situations will be common once the Open Payments information is publicly released.

As part of the Affordable Care Act, Congress required the Administration to set up a process by which transfers of value from certain covered entities (primarily manufacturers of drugs and devices) to physicians would be reportable. Such reportable information would then be made publicly available. The overall goal of this transparency is to make particular potential financial conflicts of interest more transparent. However, there are still considerable problems

with the current implementation of Open Payments, including the lack of guidance and clarity regarding the physician registration process, as well as the review and dispute process lacking necessary protections for physicians. Finally, a recent CMS proposed rule related to Open Payments would severely hamper the flow of information.

Registration Process Needs Sufficient Guidance, Clarity

CMS is encouraging physicians to register in CMS' Enterprise Portal (Enterprise Identification Management system or EIDM) and the Open Payments system in order to view the data reported by industry that will be made public on September 30, 2014. However, the CSRO is concerned that the lack of adequate notice before the beginning of registration periods has handicapped providers who hope to participate in the program in a meaningful manner. Given the importance of sufficient participation levels and the role of physicians in ensuring data integrity, the CSRO is concerned that the failure to provide sufficient notice could be a detriment to the program's performance. Further, members of the provider community have legitimate worries about the lack of guidance and the complexity of enrollment mechanisms. **We respectfully ask that CMS provide additional provider-specific guidance for the registration process and adopt policies that allow for flexibility of enrollment requirements so that physicians struggling to enroll remain able to participate in a meaningful manner.**

Review and Dispute Process Lacks Necessary Protections for Physicians and Teaching Hospitals

From July 14 through August 27, 2014, physicians and teaching hospital representatives can review and dispute data submitted about them before public release on September 30, 2014.

As part of that dispute resolution process, the CSRO requests an impartial process to dispute the accuracy of financial information intended for public disclosure. Specifically, the CSRO asks CMS to assume responsibility for ensuring the validity of published data as a means of both enhancing the integrity of the information and lessening burdens on providers in the absence of a uniform dispute process. Unfortunately, CMS recently made clear that the burden of disputes and adjudication falls entirely on health care providers and industry.

In the absence of a well-defined reconciliation process, the CSRO believes that CMS should safeguard the mission of the Open Payments program by taking steps to limit the publication of false or misleading information that can negatively impact the reputations of high quality physicians and impair patient decision-making. In its guidance to providers, CMS stated that information under dispute without reconciliation will nonetheless be posted online for public viewing with a disclaimer. The CSRO believes that the disclaimer offered by CMS fails to sufficiently protect the reputation of health professionals and publishes potentially false and actionable information that could impact a patient's decision to choose or not choose that provider.

As the collector and publisher of financial information, we respectfully ask that CMS take steps to enhance the fairness and accuracy of the Open Payments program by ensuring that health care providers have access to a meaningful mechanism for limiting the distribution of disputed information. Current standards fail to meet these goals by creating a reporting system where the default result of any dispute is publication, whether with or without a disclaimer. Such a process fails to fully consider the significant weight that patients may place

on the information published by CMS and the prejudicial effect that even disputed information can have on health care decision-making.

Changes to Continuing Medical Education (CME)

As I mentioned earlier in my testimony, certain educational presentations can provide valuable information regarding the latest and state of the art science. In my earlier example, our colleague relied on information from a scientific, educational lecture that led to an alternative treatment for his patient who had an inadequate response to more traditional therapy. In recognition of that valuable exchange of information, in previous rulemaking related to Open Payments, CMS clarified that speaker compensation at certain CME events is not required to be reported by an applicable manufacturer if all of the following criteria were met: (1) the CME program meets the accreditation or certification requirements and standards of the Accreditation Council for Continuing Medical Education, the American Academy of Family Physicians, the American Dental Association's Continuing Education Recognition Program, the American Medical Association, or the American Osteopathic Association; (2) the applicable manufacturer does not select or suggest the covered recipient speaker nor does it provide the third party vendor with distinct, identifiable individuals to be considered as speakers for the accredited or certified continuing education programs; and (3) the applicable manufacturer does not directly pay the covered recipient speaker. However, as part of the CY 2015 Medicare Physician Fee Schedule (MPFS) proposed rule, CMS proposed to eliminate the CME exception for certain CME activities and instead rely on a standard related to whether the applicable manufacturer "does not know" or is "unaware" of the compensation. This less defined standard does not afford clarity and fails to acknowledge the value of CME. Further, this action reverses a decision that CMS

had previously reached after reviewing hundreds of stakeholder comments in a comprehensive rulemaking process. This decision, if finalized, would significantly disrupt the practice of CME and the confidence of doctors, educators and others. For this and many other reasons, **the CSRO urges Congress and others to ask CMS to reconsider its proposal to eliminate this exception and urge CMS to opt instead to appropriately expand the list of certified CME accrediting/issuing agencies beyond the five currently cited in regulation.**

SUMMARY

As I hope I have outlined today, current practices at both the FDA and CMS may be inappropriately hampering the exchange of information and making it difficult for physicians to receive the information they need to make valuable treatment decisions. For the FDA, I hope that Congress will examine ways to allow for more proactive exchanges among clinicians with appropriate safeguards to assure that such information is truthful and not misleading. For CMS, I hope that Congress can urge specific programmatic changes to make the transparency process accurate and appropriately descriptive of the financial relationships among the various entities.

Thank you again for taking into consideration our written comments. The Coalition of State Rheumatology Organizations looks forward to working with the Committee to address these issues.

Physician-Directed Applications

A Position Statement of the Alliance of Specialty Medicine

Physician-Directed Applications

Physician-directed applications, also known as "off-label"[1] uses, are an integral component of the art and science of medical practice, particularly for specialty physicians. Using their medical expertise and judgment, physicians may choose to use approved medical products such as prescription drugs, biologics, and devices, for uses not listed in the United States Food and Drug Administration (FDA) approved or cleared labeling, as appropriate.

Background

It is not uncommon for some off-label uses of medical products to become standard of care in the practice of medicine.[2] In fact, off-label uses of certain medical devices and drugs can be found in standard textbooks for medical subspecialties. In certain patient populations, such as children and cancer patients, off-label use of medical products is extensive when appropriate therapies have not been developed or evaluated for the populations or a clinical trial is not feasible (such as in the case of rare diseases). In these circumstances, physician-directed applications provide treatments that may not otherwise be available for some of the nation's youngest and most critically ill patients.

Physicians use the best available clinical evidence, judgment, and consideration of individual patient circumstances and preferences in treating and managing disease and injury. Good medical practice and the best interests of the patient require that physicians use legally-available drugs, biologics, and devices according to their best clinical expertise and judgment.

FDA Regulatory Principles and Labeling

The FDA has broad regulatory authority over the approval of pharmaceutical, medical device, and biologic products in the United States. Products may only be labeled, promoted, and advertised for the uses that the FDA has approved or cleared. Labeling of a medical product is negotiated between the FDA and the manufacturer to ensure that the labeling accurately reflects the safety and effectiveness data presented in the manufacturer's

[1] "Off-label" use for approved prescription drugs, biologics, and medical devices means any use that is not specified in the labeling approved by the FDA. For cleared medical devices, "off-label" means any use that is not included in the cleared "indications for use." Labeling is considered as any written material, which accompanies, supplements, or explains the product.

[2] Refer to specific specialty examples document at specialtydocs.org

marketing application. Furthermore, a drug, device, or biologics manufacturer may choose, for economic reasons, not to pursue additional labeling for indications that may increase the cost of obtaining FDA approval or clearance. As a result, the label may not reflect changes in indications, contraindications, warnings, or dosage, supported by new data that become available after approval or clearance.

Practice of Medicine Exception

The Food and Drug Administration does not have the statutory authority to regulate the practice of medicine. In 1998, the US Supreme Court issued a judgment in *Buckman v. Henney* affirming physicians' right to use any FDA-approved therapies they believe are in the best interests of their patients. In addition, section 906 of the federal Food, Drug, and Cosmetic Act addresses the issue of the practice of medicine and states the following:

> *Nothing in this Act shall be construed to limit or interfere with the authority of a health care practitioner to prescribe or administer any legally marketed device to a patient for any condition or disease within a legitimate health care practitioner-patient relationship. This section shall not limit any existing authority of the Secretary to establish and enforce restrictions on the sale or distribution, or in the labeling, of a device that are part of a determination of substantial equivalence, established as a condition of approval, or promulgated through regulations. Further, this section shall not change any existing prohibition on the promotion of unapproved uses of legally marketed devices.*

Physicians may prescribe or administer any legally-marketed product for an off-label use within the practice of medicine.

Standards of Care

Standards of care change over time, and the emergence of new literature may alter treatment patterns. As a result, there are instances when the off-label use of medical products evolves to be recognized as a generally accepted medical standard. There are also instances in which the labeled uses of medical products are found to have contraindications and interactions that reduce their safety and efficacy. Specialty physicians are encouraged to notify the relevant agency or institution of adverse events related to the use of medical products.

Access to Available Information

To enhance patient care, physicians must have unrestricted access to truthful, non-misleading information about the benefits and risks of all therapies available for treatment, including medically accepted alternative uses of approved prescription drugs, biologics, and/or devices. Manufacturers must be able to provide adequate directions for use of both approved and medically accepted alternative indications of approved medicines and treatments, along with adequate disclosures regarding risks and the limitations of scientific

understanding.

Provided there is prominent disclosure that FDA does not approve such use, limitations on communications should only be related to patient risk based on factors including the approval status of the medicine, general medical acceptance of the treatment, and the level of scientific sophistication of the audience.

Informed Consent

Informed consent is the process by which the treating health care provider discloses appropriate information to a competent patient so that the patient may make a voluntary choice to accept or refuse treatment.[3] Among other things, informed consent requires a discussion of reasonable alternatives to the proposed intervention, which may include a discussion of medically accepted alternative uses of approved prescription drugs, biologics, or devices.

Physicians and medical institutions have varied practices for obtaining and documenting informed consent provided to patients that may or may not address off-label use. In some instances where an off-label use has come to be considered a standard of care in the clinical community and/or raises little risk of an adverse outcome, the use may not be discussed specifically with the patient. However, physicians should use their clinical judgment in determining the need to discuss specific off-label uses with patients and include information about such uses in informed consent materials when the off-label use could be a significant factor in the patient's decision about whether to undergo the procedure. If a patient has questions, the physician should also personally inform the patient that the product is being used in an off-label manner and discuss the benefit/risk profile for that use. This approach not only serves the patient's best interests, but might also help to limit the physician's liability risk.

Benefits and Risks of Physician-Directed Applications

Benefits and risks exist with off-label use. Benefits include the ability to provide care to patients who may not receive appropriate treatment or perhaps treatment at all without off-label use, such as many pediatric patients. Risks include the potential for limited effectiveness and unexpected side-effects from uses that have not been adequately studied for the specific indication or patient population.

It is well-established that physicians who use a product for an indication not in the approved or cleared labeling have the responsibility: (1) to be well informed about the product; (2) to base its use on a firm scientific rationale and sound medical evidence; and (3) to maintain awareness of the product's uses and effects.

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[3] Appelbaum PS. Assessment of patient's competence to consent to treatment. *New England Journal of Medicine.* 2007; 357: 1834-1840.

Conflicts of Interest

Conflicts of interest should be disclosed in compliance with all state and federal laws and regulations. Specialty physicians engaging in compensated arrangements with industry should disclose their financial arrangements in medical education, research, and professional activities. Physicians who are involved in product development and/or testing should disclose this role to patients. Physicians should avoid interactions and activities where discussions of off-label use could be considered promotional in nature.

Statement of Policy

The Alliance of Specialty Medicine maintains that a specialty physician may prescribe or administer any legally-marketed product for an off-label use within the authorized practice of medicine where the physician exercises appropriate medical judgment and it is in the best interests of the patient. If specialty physicians use a product for an indication not in the approved or cleared labeling, they have the responsibility: (1) to be well informed about the product; (2) to base its use on a firm scientific rationale and sound medical evidence; and (3) to maintain awareness of the product's use and effects. Specialty physicians should appropriately counsel patients about the benefits and risks of the proposed treatment, and whether alternative treatments might be available. Specialty physicians are encouraged to notify the relevant agency or institution of adverse events related to the use of medical products.

Mr. PITTS. The chair thanks the gentleman.

And now, Ms. Grealy, you are recognized for 5 minutes for an opening statement.

STATEMENT OF MARY GREALY

Ms. GREALY. Mr. Chairman and members of the subcommittee, thank you for the opportunity to testify this afternoon. And thank you as well for the attention you're bringing to the future of American healthcare, and the ability of the healthcare system to develop, communicate, and utilize the data that can lead to 21st century cures.

I am here today representing the Healthcare Leadership Council, a coalition of leaders from all sectors of American healthcare. I am very proud that our membership includes innovators, like Mr. Mussallem, also on today's witness panel.

Our members share this committee's goals for a healthcare system that is affordable, sustainable, and of the highest attainable quality, and that is also on path toward curing the diseases and illnesses that have cost us far too much both in lives and resources.

Each year, those involved in all aspects of healthcare generate literally trillions of decisions, communications, interventions, consultations, treatments, therapies, and clinical trials. The key to achieving progress lies in harnessing this massive amount of information and setting policies and practices in place to productively share and to use this data.

HLC members have been engaged in this challenge for some time both as individual innovative companies and collectively. What I share with you today is our broad-based, multi-sector perspective on how we can create an environment in which data can be used to strengthen the entirety of the healthcare continuum.

There are three areas where I will focus my comments today. One, the role of the HIPAA privacy law; two, the need for Federal data policies that enhance access to information to enable health system improvements and accelerated medical research; and three, the potential impact of the new Sunshine Act on the physician industry collaborations that are critical engines of healthcare advancement.

On the first point, the HIPAA privacy and security laws are generally serving patients in the healthcare system well, and should continue to be the guiding rule regarding the appropriate and effective use of patient health data. There are certain aspects of HIPAA, however, that warrant continuing review and discussion.

We need to keep in mind that HIPAA was created at a time in which policymakers were not 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 may be necessary to adjust the authorization components of HIPAA to ensure that data can be used effectively for research.

Also, in order to transmit data and collaborate in its use, we need to review the utility of having 50 separate sets of State privacy laws and regulations instead of a single national standard.

On the issue of Federal data policy, Healthcare Leadership Council members have developed a set of consensus multi-sector principles on data policy that I have submitted for the record. One of

these key principles is our belief that access to Federal health data should no longer be denied to entities perceived to have a commercial interest. The profit status of an organization should not take precedence over the larger question of how best to conquer disease and improve population health.

Any standard that restricts access to critical, federally-held health data is, in fact, detrimental to our shared goals for medical and human progress. We must put the benefit to patients first.

Finally, we believe strongly that Congress must diligently monitor the impact of the Physician Payment Sunshine Act. This is not a criticism of transparency, which our member companies practice and HLC strongly endorses. We are concerned, though, about the transparency without context. We are concerned that physicians may feel stigmatized by the Federal reporting of their interactions with manufacturers in a way that does not communicate the patient benefits of such collaborations.

Some of our member companies are already witnessing physicians withdrawing from collaborative activities, which can have a devastating impact not only on innovation, but also on product efficacy and safety. Congress should monitor the implementation of this law to ensure that both transparency and innovation are fully achieved.

Mr. Chairman, thank you again for the opportunity to testify today. We believe that this committee's bipartisan vision for 21st century cures is an achievable reality, one that can be accelerated by creating a pathway for the productive use of data that we already possess.

Thank you, and I will be happy to answer any questions.

[The prepared statement of Ms. Grealy follows:]

Testimony of

Mary R. Grealy
President
Healthcare Leadership Council

Hearing on

21st Century Cures: Examining Barriers to Ongoing Evidence Development and Communication

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

Tuesday, July 22, 2014
3:00 p.m.
2123 Rayburn House Office Building

Mr. Chairman and members of the subcommittee. Thank you for the opportunity to testify today on the importance of communication and evidence development in our drive to continually improve the quality of American healthcare and in the shared quest to develop 21st century cures for the diseases and illnesses that continue to exact an unacceptable toll on our society in both lives and resources.

My name is Mary R. Grealy and I am president of the Healthcare Leadership Council (HLC). The HLC is a coalition of chief executives representing virtually every sector of American healthcare. Our members are leaders of hospitals, insurers, pharmaceutical companies, medical device manufacturers, distributors, pharmacies, health information technology companies and other health disciplines. HLC members are united by our belief that American healthcare can be more affordable and accessible, that it can reach higher levels of quality, that it can achieve improved health outcomes and an unprecedented success in improving population health. We believe that these objectives can and must be attained through data-driven innovation, the kind of innovation that has defined private sector healthcare for generations.

The topic of this hearing goes to the heart of the challenges we face in maximizing healthcare's potential. Each year, millions of patients and consumers in the United States interact with the healthcare system. Those interactions lead to literally trillions of decisions, communications, interventions, consultations, treatments and therapies. We have a constant, never-ending cascade of real-time data that contains the secrets to entering the next era of high-quality healthcare and developing the 21st century cures that the Energy and Commerce Committee has outlined so clearly and compellingly.

The key to capturing this potential lies in putting the policies and practices in place that will allow us to harness this data. By utilizing and analyzing this massive trove of information we will catalyze more rapid progress in medical research and design the kind of health delivery improvements that will make our healthcare system more quality-driven and cost-effective.

The Healthcare Leadership Council has been engaged in this challenge for some time. Our individual members are among the early adopters and innovators in using data to enhance the entire continuum of healthcare – from treatment protocols to payment systems to the manufacturing of drugs and devices – and, cumulatively, they provide a broad-based perspective on the challenges that currently exist in the accessibility and usability of data to make further strides in healthcare advancements. As you articulated so well in the meeting notice for this hearing, "We need to make sure that patients, providers, researchers, and drug and device companies are able to communicate and collaborate in the most productive and transparent manner possible."

Because HLC represents these various sectors, we are able to provide you today with our members' broad perspectives and experiences on issues related to data accessibility and data sharing. I will divide my testimony into three areas: (1) The role of the HIPAA privacy law; (2) The need for federal data policies that strengthen access to information and enable improved care, greater healthcare value and accelerated research; and (3) The need to examine the impact of Sunshine Act laws on physician-industry collaboration and the patient-focused benefits that result from those collaborations.

Health Insurance Portability and Accountability Act (HIPAA)

In addition to bringing together the expertise of its various members, HLC also leads a multi-organizational Confidentiality Coalition, which has played an important role for more than a decade in advising policymakers on the steps needed to protect confidential health information while also making data appropriately accessible under HIPAA to strengthen care quality, improve healthcare systems and advance research.

We believe that the HIPAA privacy and security laws are, generally, serving patients and the healthcare system well and that it should continue to be the guiding rule wherever HIPAA-covered entities are involved. As healthcare payment and delivery systems evolve, and even as we gravitate toward greater use of electronic health records, we believe that HIPAA continues to be an effective policy foundation with which to govern the appropriate and effective use of patient healthcare data.

In order to achieve more rapid healthcare advancement, while still protecting patient confidentiality, there are certain aspects of HIPAA and privacy laws in general that warrant policymaker review and discussion, specifically:

- As medical research itself evolves, we must be cognizant of the limitations HIPAA imposes on research into new cures and technologies. HIPAA was created at a time when policymakers were not thinking about the knowledge that could be gained by accessing data residing in large databases. We now are in an era where researchers can harness vast amounts of data to learn at a rapid pace unlike we have ever seen. Policymakers should be aware of the need to adjust the authorization components of HIPAA as necessary to ensure that data can be used effectively in a research setting.

- Currently, in most research environments, patient data must be de-identified before it can be utilized. In general, we promote the HIPAA de-identification standard as a strong model for making data anonymous and believe this standard should be applied in appropriate circumstances to health data, inside or outside of the HIPAA schema, to effectively protect patient and consumer health data. Policymakers, however, need to be aware of circumstances in which de-identified data is not sufficiently useful to achieve particular research objectives.

- The presence of 50 separate sets of state privacy laws and regulations represents an impediment that slows down medical and scientific progress. It makes little sense and does not serve the public interest for healthcare entities and research to try to untangle inconsistent, overlapping laws. In today's world, information must be transmitted across state lines and laws should enable this data sharing, not obstruct it.

We believe strongly that progress toward 21st century cures would be aided by the presence of a national privacy framework to replace the complex and burdensome patchwork quilt of current state laws. This national framework should be modeled upon the current HIPAA structure which is, again, working well in protecting patients and enabling healthcare improvement.

Federal Data Policy

More than any other public or private entity, the federal government possesses the greatest volume of health data. In recent years, there have been strides made in making more of this information available to entities outside of the federal realm. The 2009 Open Government Directive and the Department of Health and Human Services's Health Data Initiative led to the

sharing of valuable information from agencies like the Centers for Disease Control and Prevention, the Centers for Medicare and Medicaid Services (CMS) and the Food and Drug Administration.

However, the hands-on experience of our HLC member companies in multiple health sectors informs us that much more needs to be done in the area of data accessibility and quality. Toward that end, HLC members collaborated in the development of consensus, multi-sector principles on data policy. I am submitting this full set of principles as an addendum to my testimony (Attachment 1). Some of these relevant principles include:

- **As taxpayer-funded entities, it is the responsibility of government health agencies to maximize public benefit from data collected through their operations.** By allowing regular access to data at minimal cost to organizations that are subject to consumer protection laws, organizations throughout the country can develop novel ways to fight disease, improve the quality of care, reduce costs and accelerate innovation. We encourage increased coordination among federal government agencies to reduce data "silos."

- **Timeliness, format and regulatory flexibility are critical for organizations serving consumers to make the most of data held by the federal government's health programs.** Federal 'data use agreement' restrictions keep many healthcare organizations from gaining access to data that would allow them to improve care and reduce costs. These agreements should be revised to allow organizations to get preapproval for real-time access to CMS data for appropriate uses. The current practice of precluding some organizations from purchasing data at all and substantial lag time in the availability of key information slows progress that could benefit everyone.

- **Federal health data should no longer be denied to entities perceived to have a commercial interest.** Healthcare organizations are using advanced data analytics to improve healthcare quality, better manage population health and address consumer health needs using private-sector patient-level data. These organizations can enhance their work with appropriate access to federal program data. Commercial entities could easily be held to the same Data Use Agreement standards as noncommercial entities.

HLC has also collaborated with stakeholders outside our own membership to discuss the issue of access to federal government health data. Participants in these discussions include individuals representing the health sectors in our own membership, along with think tanks and academic organizations. Those we have worked with have shared insights on data exchange, current barriers to access and policies that can broaden medical and healthcare knowledge, engage patients and support essential research.

Important data policy themes have emerged from these discussions:

- **As part of the "open government" initiative, the administration should further explore and encourage government-wide policies and standards for health data sharing.** These would include uniform data access methods and usage agreements across federal agencies in order to simplify the process for organizations seeking data.

- **The federal government should convene all stakeholders for a broad discussion of situations in which there should be restrictions on data access.** This would enable government to establish a more consistent rationale for restrictions on health

data that continue to exist. It could also include reexamining the feasibility of regulating access by usage of health data instead of by type of user.

- **Federal policymakers should broaden efforts to share most federally-held health data, when appropriate.** Data collected from federal government programs, particularly those funding new and innovative care delivery models or tools, should be available for research, with appropriate privacy protections. As partners to the federal government in national efforts to improve care while lowering costs, private sector organizations should have access to the tools needed for success.

On the issue of private entity access to federally-held health data, I am also attaching to my testimony a March 7, 2014 letter to CMS Administrator Marilyn Tavenner from the Healthcare Leadership Council and the National Pharmaceutical Council. In this letter, we applaud CMS, in its proposed rule affecting the Medicare Part D and Medicare Advantage programs, for opening up the topic of access to Prescription Drug Event data by entities with commercial interests. We recommend expanding the discussion to include the long-standing HHS policy that denies access by commercial entities to data from the Medicare Part A, B, D and Medicaid programs as well as other program datasets (Attachment 2).

In the letter, we note that the profit status of the organization in question should not take precedence over the larger question of whether the research in which the organization is engaging is of high quality and has the potential to improve population health. Further, by excluding certain organizations from access to federal health data, federal policy is also excluding the deep scientific and analytic expertise that can bring improvements to the entire healthcare spectrum. Any standard that essentially bars access to this critical data is, in fact, detrimental to the larger goals of our healthcare system and our shared societal goals.

The Physician Payments Sunshine Act

The Physician Payments Sunshine Act (referred to hereafter as "Sunshine Act") requires manufacturers of drugs, medical devices and biologics that participate in federal health programs to report payments and transfers of value to physicians and teaching hospitals. This reporting of payments is already taking place and a website is expected to be launched this fall making this data available to the public.

We believe it will be essential for Congress to closely monitor the implementation and impact of the Sunshine Act to ensure that it does not have an adverse impact on physician-industry collaboration and, as a consequence, innovative healthcare progress.

Many of the most important medical developments of the past half-century have come as a result of physicians and researchers sharing their insights and expertise with product manufacturers. These lifesaving and life-transforming innovations include CAT scans, cervical disc replacements, coronary stents, deep brain stimulation, the heart and lung bypass machine, laser eye surgery, mumps and measles vaccines, penicillin, statins, total knee replacements, artificial heart valves and ultrasound diagnostic technologies. And these are just a few examples of a much longer list of benefits yielded from physician-industry collaborations. I have included a list of some of these as an attachment to my testimony (Attachment 3). This interaction between physicians, researchers and manufacturers is the inception point for so many of our cures, treatments and medical technologies – in the past, the present and, hopefully, the future.

Our concern with the Sunshine Act should not be construed as opposition to transparency. In fact, HLC launched an initiative under our National Dialogue for Healthcare Innovation (NDHI)

platform which brought together leaders from multiple health sectors, government, academia and patient organizations to thoroughly discuss the issues surrounding physician-industry collaboration. That effort led to the development of a consensus set of principles on the issue, endorsed by organizations from many of the aforementioned sectors, which emphasize transparency, research independence and patient-centeredness. I have attached those principles and additional information regarding NDHI and physician-industry collaboration to my testimony (Attachment 4).

Rather, our concern is with the possibility of transparency without proper context. If the only information conveyed to the public and media regarding transfers of value between manufacturers and physicians involves dollar amounts – without a full, adequate explanation of the benefits generated for the public as a result of those interactions – there are legitimate concerns on the part of physicians that they will be unfairly stigmatized and lose the faith and confidence of their patients and the public at large. One only has to look at the controversies surrounding the recent release of Medicare physician payment data to see that information can be easily misconstrued if not presented with full context.

We have, in fact, already heard from some of our HLC member companies that physicians who have worked with them in the past to ensure the efficacy and safety of products are now reluctant to continue doing so because they are concerned about how these interactions will be reported and interpreted. When this collaboration is discouraged, those hurt the most are current patients as well as those who will suffer from diseases and illnesses in the future because new cures and treatments were delayed or never developed. This concern is amplified by the recent decision by CMS in the proposed Physician Fee Schedule for 2015 to include the reporting of continuing medical education (CME) funding, a move that will only have a

dampening effect on physicians learning new medical science because of a perceived stigma associated with industry support of CME activities.

Again, we encourage Congress to closely monitor the implementation of the Sunshine Act and seek the input of those in the physician community as well as pharmaceutical and medical device manufacturers to get a comprehensive perspective on whether the law, in its current form, is having an adverse impact on the innovation that is critical to 21st century cures. Transparency and innovation are not and should not be viewed as mutually exclusive and we stand ready to work with Congress to ensure that both goals are achieved.

Chairman Pitts and members of the subcommittee, thank you again for the opportunity to present testimony on this important issue. The Healthcare Leadership Council and its individual members believe strongly that the diseases and illnesses that diminish and shorten too many lives can be conquered within the foreseeable future as long as we enable and incentivize the healthcare innovation that has generated countless medical miracles over the past several decades. We look forward to working with you to make this vision for 21st century cures a reality. Thank you.

Attachments (7)

[The attachments to Ms. Grealy's testimony have been retained in committee files and can be found at *http://docs.house.gov/meetings/IF/IF14/20140722/102524/HHRG-113-IF14-Wstate-GrealyM-20140722.pdf.*]

Mr. PITTS. The chair thanks the gentlelady. Thanks to all the witnesses for their testimony. I will begin questioning and recognize myself for 5 minutes for that purpose. I will start with you, Dr. Schimizzi.

Different uses for FDA-approved drugs and devices are constantly being discovered, many times for treatment of different conditions and diseases or for different populations. Manufacturers of these products have access to robust data sets and information that is not always limited to the specific indications listed in their package inserts.

Why is it important that we responsibly allow providers to have access to such information to ensure that the most appropriate treatment options are being considered?

Dr. SCHMIZZI. Well, thank you, Mr. Chairman. In rheumatology we see many patients with rare diseases and unusual autoimmune problems. And we also see patients who are referred to us by other specialists for autoimmune problems in their specialty that they do not know how to handle, so they send them to us.

In our armamentarium of medications, we have an array of medications that work very well. Some of them are of low toxicity, and some of them are of high toxicity. In the event of a new agent being brought to the United States medical arena having a high safety profile, but lack an indication for an orphan disease or a critically important problem in another organ system, like the eye, for example, use of those medications would be miraculous and have a high margin of safety if we had access to information. I am just using the eye as an example. There are other instances as well. Primary muscle disease is another one.

Medications are available, but the indications are not there, and they probably never will be because the numbers of patients who have these diseases is so small, it would take many years to discover that the indications were there and millions of dollars, perhaps tens of millions of dollars, to identify that.

So if we had access to information that these new medications might be effective in certain other small diseases that may have been gleaned from the data that was derived from the direct clinical trials, then that would be extremely helpful to us and help guide us in a direction that would increase efficacy, and increase safety, and maybe even decrease cost and poor outcomes.

Mr. PITTS. Do you believe that the current restrictions on off label communications are limiting healthcare professionals' ability to provide the most appropriate treatment to patients? And if so, what needs to happen?

Dr. SCHMIZZI. What was the last part?

Mr. PITTS. If so, what needs to happen?

Dr. SCHMIZZI. Yes, I do believe that the limitation of exchange of information is hampering the delivery of healthcare to some of these patients, especially in my sub-specialty. What needs to happen is that we need to have access to information that is locked up in vaults in pharmaceutical companies, locked up in data sets in study information.

For instance, here is a great example that I can spread to rheumatology. There is a great drug that came out many years ago to prevent ulcers and to treat ulcer disease and esophagitis called

Prilosec. The generic name was omeprazole. Prilosec was a mixture of two different mirror image molecules, D-enantiomer and an L-enantiomer. It is like a right hand and a left hand.

Well, it came to light that one of the enantiomers was much more effective at treating ulcer disease and esophagitis, so out came esomeprazole, or Nexium, which has proven to be more effective.

What if there were medications on the market right now that we have that could treat diseases but have side effects, and yet if we isolated the D-enantiomer and the L-enantiomer, we would identify which one was effective and which one caused the problems. I submit to you that there are drugs in our compendium right now that have D and L isomers, and the data sets are probably available in the vaults of pharmaceutical manufacturers that show the D isomer is more effective than the L isomer. The L isomer has more complications than the D isomer. So that would be a dramatic improvement.

So such data sets are locked up. We do not have access to them, and I do not know that we ever will.

Mr. PITTS. Quickly, Ms. Grealy, you mentioned HIPAA. What kind of changes should we consider to HIPAA to ensure that big data can be used effectively for research purposes while still protecting patient privacy?

Ms. GREALY. Well, when the HIPAA law was originally passed, there was an exemption for healthcare operations, and that included the treatment and payment for patients. But sort of outside that scope was research activity.

So I think a very simple approach would be let's include healthcare research as if it is a natural part of healthcare operations.

There are probably several other recommendations that we could make, but I think the key here is to make sure that we have an appropriate balance between protecting patient information, and we believe very strongly in that. We also do not want to erect barriers to having access to that data.

I think a big part of this is having informed consumers, educated patients, and especially as we are seeing patients engage more and more in the management of their own healthcare.

Mr. PITTS. The chair thanks the gentlelady, and my time has expired. The chair recognizes the ranking member, Mr. Pallone, 5 minutes for questions.

Mr. PALLONE. I wanted to start with Dr. Jacques, and then if I have time, ask Dr. Rising a question. But, Dr. Jacques, I wanted to better understand what you mean when you talk about the confusion created by Medicare's vague authority and lack of administrative agility in Medicare coverage and payment policies for new innovation technologies.

Could you briefly describe the statutory limitations that apply to Medicare coverage determinations, both as they relate to coverage with evidence development and local coverage determinations? And what are your recommendations for how to streamline these authorities? And then maybe how does this existing authority impact the decision making framework for CED study questions, and what data is needed to trigger and end the CED study?

Mr. JACQUES. The reasonable and necessary standard, which is essentially the coverage standard for Medicaid, those provisions are 1862(a)(1) of the Social Security Act, which is then followed by subsections (a) through (p) that parse things out for prevention hospice and things along those lines.

CED itself is not defined in the Social Security Act, so CMS has had to rely on the Agency for Healthcare Research and Qualities Research Authority under Section 1862(a)(1)(E) of the Act. Thus ARC has to approve every CED decision.

While the scope of a national coverage determination is described broadly in statute as a decision under Title 18, local coverage determinations are defined in the Act only as decisions under 1862(a)(1)(A). Thus, an LCD could not implement coverage with evidence development. So even if there were an item or service that is only furnished within one contractor region of the entire country, a national decision would be required to implement CED.

I have been told by various stakeholders that CED could be approached more eagerly if it were not tied to the formal NCD process. The current framework forces CMS to apply the CED requirement to all beneficiaries receiving the item or service in question, regardless of where they live. This is a particular challenge for beneficiaries who live in the remote parts of the country where clinical studies do not normally happen and clinical trial enrollment is, frankly, unrealistic for many.

A more agile CED paradigm would permit CED to occur in parallel with other forms of coverage rather than requiring everyone to fit through the same door.

Mr. PALLONE. Now, you also highlight a rapid decline in the number of national coverage determinations in the last few years. How has the lack of staff resources within CMS impacted that decline, and what, if any, impact has this had on coverage with evidence development?

Mr. JACQUES. I believe that staff reductions are the largest single cause of the decline in number of national coverage determinations. And the impact of this decline is broader than CED because it impacts the Agency's ability to respond to other requests for coverage.

CED itself generally requires more internal staff work to develop, and it creates an ongoing need after the publication of the final decision to interact with sponsors who might want to conduct clinical trials. By my own estimate, it takes about three times as much internal effort for CMS staff to do CED than it does to simply say yes or no.

Mr. PALLONE. All right. Thank you. And I am going to try to get this in. Dr. Rising, you note in your testimony that the taste trial conducted in Europe on heart attacks cost a tiny fraction, perhaps one-one hundredth of what it would cost in the U.S., because it was able to make use of patient registries. However, we also heard in Mr. Mussallem's written testimony that registries can be very expensive to set up and maintain, and threshold questions must be answered to determine when and how registries should be used for post-market data collection.

Now, I am familiar with registries from the law creating the 9/11 Health Program. It included a provision to authorize a registry of people who were exposed to toxic dust from the attack on the

World Trade Center on 9/11. But I do not know much about registries for assessing medical products.

Can you explain how these registries work, and can you describe what source of impediments to the use exist, including why they may be harder, expensive to set up and maintain. And I would like to know your thoughts and what can be done to facilitate their use.

Dr. RISING. Sure.

Mr. PALLONE. You do not have a lot of time to do it.

Dr. RISING. I will in 30 seconds.

Mr. PALLONE. OK.

Dr. RISING. So medical product registries, kind of like the 9/11 responder registry, will follow a group of patients with one exposure for a period of time. So, for example, we heard a little bit from Mr. Mussallem about their trans-catheter valve registry, which follows patients who have gotten a valve for a period of time in order to assess their long-term outcomes.

Now, while registries can be a tremendous source of information on the post-market performance of devices, there are some barriers to setting them up. And one of the biggest barriers is the lack of interoperability between systems. So, for example, clinical staff need to enter data in the TBT registry and then enter a lot of the same data again in electronic health records. So this kind of added burden on the staff is one of the biggest drivers for why registries are currently inefficient in the United States.

Now, in addition to these post-market benefits, registries can have tremendous benefits for innovation as well. One of the other benefits that we have seen for the trans-catheter valve registry that Dr. Shuren talked about at the first hearing was that data from the registry was used to expand an indication for the device.

So if we are able to take some steps in this country forward for registries, we should be able to see significant benefits both on the safety side and then also on the innovation side of things.

Mr. PALLONE. All right. Thanks a lot. Thank you, Mr. Chairman.

Mr. PITTS. The chair thanks the gentleman, and now recognizes the vice chairman of the full committee, Ms. Blackburn, 5 minutes for questions.

Mrs. BLACKBURN. Thank you, Mr. Chairman. I have got just a couple of questions that I want to go to, and again, I thank you all for participating with us and working with us.

There is a lot of bipartisan agreement on this. You have heard different members of the committee speak to that, finding a pathway forward so that we deal with the regulatory framework, provide some certainty, and speed up the process by which innovation and cures are going to get to our patients is a shared goal. And so, we thank you for that.

Mr. Mussallem, let us go back to the topic of the registry. We have talked a little bit about that, and you have all talked about basically the data and the value of the data that can be found within those registries and the benefit to our—to patients from being able to utilize the data in those registries.

So let us talk a little bit about risks that are there for the patients or cost that is there. And can you give me just a little bit of an articulation looking at the other side of this with risk and

cost, both the actual dollar cost, or, as Ms. Grealy mentioned, the privacy, some of the privacy concerns?

Mr. MUSSALLEM. Yes, thank you very much, Member Blackburn. And I applaud the bipartisan cooperation toward this shared goal. Registries can be a powerful tool, and we by and large think they could be very appropriately used. My only caution that I mentioned in my testimony is that there are some cases where technologies and/or therapies are well enough known that can establish a safety and effectiveness standard without going through that sort of process.

In the case of the TBT registry, in particular, that I mentioned, the large group of stakeholders ended up generating this long list of items to be collected. I mentioned 300 data fields. Maybe when a technology is brand new and unknown you want to learn an awful lot about it. The problem is that becomes quite costly. And at some point, it gets too expensive to maintain.

Ideally you would have a registry that could be whittled down to those things that are really most critical that you would like to measure, and there may be a way to populate it with electronic data that is already being generated, such that a registry could be a very cost-effective tool.

In the case of the TBT registry, it literally cost seven figures plus per year for that total cost. That is shared by a lot of constituents, including manufacturers. But a lot of the burden rests on hospitals. They have a burden where they actually pay a fee every year and additionally have to put on dedicated staff just to fill out those fields. So not something to be taken lightly.

Mrs. BLACKBURN. If you had to give us a list of guiding principles as we look at a framework for developing some of the registries, what are those three or four principles that you would articulate?

Mr. MUSSALLEM. I think it is most important to have a clear risk benefit analysis and also have clear goals set out by the registry. There should be a set of rules around the registry and some governance guidelines around it.

Dr. Rising spoke to the work that Pew Foundation has done in this area. It is actually very thoughtful, done with a broad group of stakeholders about the value of registries. And I think that is not a bad guidepost.

Mrs. BLACKBURN. OK. And let me ask you this. Do you envision any of these registries moving to the point where the patient could populate some of those cells and fields themselves?

Mr. MUSSALLEM. Ideally registries would not be expensive to populate, and any time that they could be filled out automatically in an electronic patient record or even, as you suggest, that a patient could do it themselves, this is important. I mean, some simple things. Is the patient alive? Is the patient going through a routine of exercise, or what is the patient's diet? All these things are very potentially powerful variables that could provide insight to the value of technologies.

Mrs. BLACKBURN. Well, we would hope that anybody populating one of these with an app on an iPad would indeed be alive and not have their avatar doing it for them.

Mr. MUSSALLEM. Well said.

Mrs. BLACKBURN. So, oK. Dr. Schimizzi, let me ask you just a couple of things on off label use. You had mentioned that, and I am intrigued by this. I think this is an area that it holds some promise. It is a legal practice, correct?

Dr. SCHMIZZI. Yes.

Mrs. BLACKBURN. OK. Do you consider it a best practice to inform a patient that a therapy that is being prescribed is off label?

Dr. SCHMIZZI. I think that is best practice, yes, and I always do.

Mrs. BLACKBURN. If informed doctors can legally prescribe off labeled patients who are also well informed, what would be the current barriers to that practice?

Dr. SCHMIZZI. Well, the barrier is we need the information to pick which drug to use in a difficult situation. And that information is not always available to us.

In the immune system, there are different cells that are at work, we know that a certain cell is active in one disease. And if you suppress that cell, we can suppress the disease or cure the disease. And that agent might be available for a cancer, but if we can transpose and use that in this patient, that would probably work. It would be very nice to have that information from the pharmaceutical company or manufacturer or innovator who developed that product that, yes, this is very, very likely an effective way to use this medicine, but we are never going to study it because they probably never will.

Mrs. BLACKBURN. OK. I will yield back. I am over time. Thank you, Mr. Chairman.

Mr. PITTS. The chair thanks the gentlelady, and now recognizes the ranking member of the full committee, Mr. Waxman, 5 minutes of questions.

Mr. WAXMAN. Thank you, Mr. Chairman. The Affordable Care Act strengthened our movement away from a healthcare system that rewards providers for the volume of services they provide and toward a system that fosters and promotes high quality, high value care. The bipartisan SGR legislation is to make this committee's perspective a permanent change in the reimbursement rate for physicians under Medicare. That legislation passed out of this committee and the other committees' jurisdiction and then furthered that aim by incentivizing care delivery that is coordinated in alignment with consensus guidelines and best practices, and as efficient as it is appropriate.

Dr. Jacques, in your testimony you speak of the broad national goals of Federal health agencies to improve public health and protect beneficiaries' access to products and services that demonstrate genuine benefit. You suggest that FDA approval for drugs and devices puts products on the shelves, but a prudent purchaser should not blindly pay for those products without regard to how useful or appropriate they are.

Could you speak to this point: should Medicare really be paying for products that have no real value or paying more for products that have no added value? How do we balance a desire for rapid adoption of new technologies with ensuring that providers can be confident in the safety and benefit of new technologies as they are held accountable for their use?

Mr. JACQUES. New technologies remind me of teenagers, and both of my children are adults, so I survived raising teens. We see glimpses of their future promise, but we also recognize that not all of them are going to be good drivers as soon as we put them behind a wheel. As a society, we accept this and we balance their independence with our risks through a variety of mechanisms, whether it is a learner's permit or a prohibition on consuming alcohol or driving with friends.

I believe in an ideal health technology system. We would have one where lessons are learned quickly and disseminated broadly. That depends on reliable collection, analysis, and publication of real world data that arise from using patients who are more typical than those studies in pivotal trials and who are treated in their communities by their own physicians.

Mr. WAXMAN. OK. But what does that mean if a doctor wants to use a new technology, and he has to be confident that this is going to be safe and it is going to benefit the patient?

Mr. JACQUES. I am sorry if I was obtuse. What I was trying to convey is that the timing of calling the question is as critical as the content of the question itself. And especially for new technologies, the issue is being asked to call the question arguably prematurely to give it a thumb's up or a thumb's down when, in fact, what you actually have is an adolescent technology that has promise, but you do not really know the final answer.

Mr. WAXMAN. And should we be paying for that through the Medicare system when we do not know whether it is going to add any value to what we already have available to us?

Mr. JACQUES. There are people who feel strongly on both sides of that question, sir.

Mr. WAXMAN. So when we do we call the previous question to get their vote?

Well, we hear a lot of concern raised from manufacturers on the cost of data collection to the healthcare system both in real terms and in delays of bringing new technologies to patients. However, as you suggest under the 510(k) paradigm, some devices may be cleared for marketing with no relevant clinical trial evidence at all. Could you discuss your concerns with that program and the potential risk to the healthcare system of Medicare covering such technologies even under its coverage with evidence development authority?

Mr. JACQUES. Yes. While that paradigm is appropriate for many low-risk devices, I would focus my own attention on that subset of cleared devices where untested claims of enhanced benefit are made beyond the predicate device, or where subsequent evidence may raise questions about the fundamental impact of the technology.

I think the premise of the 510(k) program makes it more difficult for a sponsor to articulate an enhanced value proposition for a technology when it has been found to be substantially equivalent to an old technology.

And that to me is the critical point in terms of paying for value. That value proposition that you are essentially better than something is hard to make if you have not actually been compared to anything else.

Mr. WAXMAN. So we may have a 510(k) to get the device approved, but we ought to know before we start paying a lot of money for it that it is going to work.

Mr. JACQUES. Yes, sir.

Mr. WAXMAN. Thank you. Thank you, Mr. Chairman.

Mr. PITTS. The chair thanks the gentleman, and now recognizes the vice chairman of the subcommittee, Dr. Burgess, 5 minutes for questions.

Mr. BURGESS. Thank you, Mr. Chairman. And I want to thank the ranking member for his generosity in mentioning H.R. 4015, which was the repeal of the sustainable growth rate formula, which did come through this committee. We are about at the 1-year anniversary of that 51 to zero vote. That was a landmark occasion for the committee. And in many ways, the development of that SGR policy was very similar to what is happening with the Cures Initiative. So I think that provides a template that ultimately could speak to success for the Cures Initiative that as we opened the doors up, we took information, asked for information from physicians, from patients, as to what they needed to see in the repeal of the sustainable growth rate. As a result, nobody got exactly what they wanted, but we got a product that was ultimately supportable by both Republicans and Democrats on this committee, and ultimately did pass the floor of the House, though we are still waiting on the Senate.

Ms. Grealy, I need to ask you a question about—there is a bill that Donna Christensen and I have done, H.R. 2663, which deals with CBO scoring, because oftentimes it seems like there are good ideas that are developed within the healthcare sphere, but then CMS will say, but you know what? All we can do—or the CBO will say CMS just tells us about the cost, so all we can do is report to you on the cost. So the ability to implement this new regimen is, in fact, a cost driver for the system, and cannot be regarded as a cost saver.

And, in fact, in this committee, even though I did not support the Affordable Care Act, on this committee I recognized a great deal of anxiety on the part of my Democratic counterparts in dealing with Mr. Elmendorf at the Congressional Budget Office. Wait a minute, we get no credit for all of the savings we are going to get from treating things in a more timely fashion.

So in your role at the Healthcare Leadership Council, have you studied that issue at all?

Ms. GREALY. Yes, Mr. Burgess, and we strongly support the legislation——

Mr. BURGESS. I felt you would say that.

Ms. GREALY. You think so.

Mr. BURGESS. That is why I asked you.

Ms. GREALY. And delighted that it is bipartisan legislation as well. But as you know, innovation plays a strong role in wellness and prevention. And what our members have seen are long-term savings when you make that investment in wellness and prevention.

And as you point out, unfortunately CBO, in their traditional scoring methods, does not give you credit for those long-term savings. And we know that 70 to 80 percent of healthcare costs today

are going towards the treatment of preventable chronic disease, and we know that if we make an investment over the long term, we will see a dramatic reduction in those healthcare costs. So your legislation would not mandate that CBO have this longer scoring window, but at least we would have that option so that you as members of Congress could see that information and then make your decision on making those investments, which may have a short-term cost, but we know in the long term will result in better health and lower costs for the healthcare system.

Mr. BURGESS. Well, oftentimes it seems today we only end up talking about the costs of a therapy and we do not recognize the fact that, my goodness, we have beaten one of the big scourges of people's health. The hepatitis C treatment comes to mind. Instead of talking about the victory over hepatitis C, a disease that did not even have a name when I was in residency. We called it non-A/non-B hepatitis. And now there is a treatment for it that is, in fact, a cure. That is pretty big news.

So it is my hope that the Cures Initiative will be able to focus on those things. Yes, we will talk about price and we will talk about cost as we go through. But the big news, the headline is hepatitis C vanquished in our lifetimes, and that is a big deal.

Dr. Schmizzi, I wanted to ask you a question on the Sunshine Act and the Sunshine Act provision that was contained in the Affordable Care Act. It does seem like it was written pretty broadly, and now the implementation is or runs the risk of hindering communication and information sharing among physicians.

So a rule that came out over the 4th of July weekend may prevent some of the country's most qualified physicians from giving lectures to fellow physicians through continuing medical education. Have you heard of providers that are having difficulty getting access to medical studies or even finding it more difficult to access continuing education because of the way this law is being implemented?

Dr. SCHMIZZI. Excuse me, Congressman. I do not hear of anything yet, but I can certainly see it coming that the Sunshine Act provision, the way it is written, can actually inhibit speakers from wanting to attend or be participants in a medical conference because of the information that will be published about them being paid and where the money comes from.

Most institutions, most professional associations get their funding from member dues, but they also get funding from industry support in the form of gifts or donations. And those gifts and donations, if they are identified to be tied to CME credits, can actually impair the desire of academicians and thought leaders in medicine to give those presentations in front of those societies. So it can have a real negative impact on that. I do not believe it has happened yet in my sub-specialty, but it certainly is possible.

Mr. BURGESS. Thank you for that answer. Mr. Chairman, I hope that is something that this committee will keep in mind and continue to monitor as we go forward. I will yield back.

Mr. PITTS. The chair thanks the gentleman, and now recognizes the gentleman from Texas, Mr. Green, 5 minutes for questions.

Mr. GREEN. Thank you, Mr. Chairman. And again, thank our witnesses for being here. A central component in improving the

quality of our healthcare system and developing 21st century cures must be data-driven innovation. Mr. Mussallem, in your testimony you talked about the coverage for evidence development CED determination, how it can be useful if used appropriately.

However, the challenge of ensuring CED is a tool for the reimbursement system to give patients access to groundbreaking therapies rather than the burden that ultimately limit innovation remains before us. Can you tell us how we might be able to handle that?

Mr. MUSSALLEM. Sure. Particularly the use of CED, I think, is valuable for therapies that are new and really have not been evaluated in the past. In many cases, the therapies that can be reimbursed are ones that are well understood, and you could establish the safety and a safe and necessary threshold. But in the case where you just do not know much because they are novel, it is helpful to be able to apply CED.

It is not always clear in the beginning of the CED process exactly what evidence is going to be collected and how much is necessary. And one of the things that is also not clear about CED is when does it come to an end? At some point in the initial stages of a technology, it is very valuable to learn as much as you can and collect that evidence. But once you have done that for some period of time, it is appropriate for CED to sunset so that it does not just become another layer of cost that sits on the healthcare system.

And so, it is important, I think, to define CED more thoughtfully and carefully as we think about using it as a tool. But it has great promise for entering areas that are unknown.

Mr. GREEN. OK. Do you have any mechanisms you would suggest to enhance the coverage of these innovative therapies?

Mr. MUSSALLEM. It is not a simple question. In the case of transcatheter technology, CED was used, and it was used that allows for this important aspect of medical device development to be evaluated, different than a drug.

Medical technology is one that is an iterative process. Because we make tools for physicians, often we get a lot of feedback from physicians and they say, could you make it better? Could you make it smaller? Could you make it do things that it does not do today? And we respond to that. And through those changes, therapy improves dramatically.

And so, a coverage evidence development tool that is flexible, and this is what was done in the case of trans-catheter heart valves, allowed for the system to accommodate new generations, new indications, as the evidence supported it. So that is a powerful use of that tool.

Mr. GREEN. Dr. Jacques, is there any other mechanism available to provide coverage to these new innovations?

Mr. JACQUES. There are other mechanisms, including regulations concerning Medicare coverage for FDA-approved Category B investigational device exemption trials, the challenge being that aside from CED and those IDEs, there is not an obvious path for other sorts of valuable research.

Mr. GREEN. OK. Anyone else on the panel for those issues or those questions?

If not, our entire healthcare system is shifting to a model that embraces shared decision making by informed patients whose views are valued and considered at every stage of the treatment. We have heard a great deal about the potential value for patient preference information and regulatory risk benefit determinations, particularly in the context of medical device pre-market approval.

The FDA has emphasized that patient tolerance for risk and perspective on benefit is an important consideration. It makes sense for the innovators and regulators to consider patient perspectives as they develop and evaluate medical devices.

Mr. Mussallem, again, what potential benefit do you see from incorporating patient preference information in regulatory determinations, and do you have any suggestion on how it could be incorporated in the process?

Mr. MUSSALLEM. Sure. All medical technology and all medical advancements are not created equal. Some can have a profound impact on patients' lives. In our case, sometimes it is the only difference between life and death for these patients. So when you are making that sort of a consideration as a regulator, you would really love that the regulators, they have the ability to apply a risk benefit analysis when they are thinking about what they should do in terms of allowing this technology to come to patients.

If you keep the bar too high in the pre-market approval setting, what you might do is in an effort to achieve great science, again allow patients to not benefit and, in fact, die or live very poor quality of life. And sometimes it would make some sense to allow a certain element of risk, certainly to safeguard against safety concerns, and have a basic level of evidence, but to study in a post-market setting the true depth of efficacy in a real-world setting, and then apply that and make adjustments.

So this is one that you would not want to be unfettered, but to give regulators, in effect, not only the ability to, but the mandate to take a risk benefit analysis I think would be a powerful enhancement for the system and make it a learning system rather than what we have today.

Mr. GREEN. Thank you, Mr. Chairman.

Mr. PITTS. The chair thanks the gentleman, and now recognizes the gentleman from New Jersey, Mr. Lance, for 5 minutes for questions.

Mr. LANCE. Thank you, Mr. Chairman, and good afternoon to you all. Ms. Grealy, in your testimony you state that the key to harnessing the potential of real time data lies in putting the policies and practices in place that allow us to harness this data. You then go on to state the importance of protecting confidential health information while also making data appropriately accessible under HIPAA.

In several of our recent hearings, witnesses have pointed out the challenges that arise in ensuring that this innovative technology is HIPAA compliant. Are there ways in which HIPAA inappropriately restricts the sharing and use of patient data by researchers and medical providers?

Ms. GREALY. Well, I think it is an ongoing challenge. And really what are trying to do is find the appropriate balance between pro-

tecting that patient information, but not stifling the innovation or access. And so we constantly have to keep that in mind.

And periodically proposals are put forward that really would consume a lot of resources and time, and really do not create any particular value for the patient. I will use an example of that disclosure of everyone who has had access to the patient information, whether they are within that healthcare operations that I discussed, which is reasonably expected by patients.

So I think it is all about making sure that we do not try to micromanage this, and we really put the patient at the center of it. And how can we create better value for that patient? And so, as we are looking at new ways and new access to information, and I will use as an example of that, as I mentioned earlier, patient engagement and the new tools for that, the mobile apps.

And we are spending a lot of time, those of us that have been minding the HIPAA world for many, many years, working with those app developers and telling them, as you are approaching this, we do not want to hinder your innovation, but try to build into your system up front the appropriate patient protections and information protections. But again, the key is let us not stifle that innovation by them, and let us not defer a whole lot of resources that could be going towards patient care and treatment and innovation by getting caught up in too much compliance activity.

Mr. LANCE. Is there something we should be doing here in Congress to make this a better situation?

Ms. GREALY. I would almost say do not do too much.

Mr. LANCE. First do no harm.

Ms. GREALY. Yes, first do no harm.

Mr. LANCE. To coin a phrase.

Ms. GREALY. Yes. And as we are looking, we have heard a lot today about registries and how we can use that information. I would say, again, let us not stifle that access and the use of that information.

And the other very powerful thing that we are seeing that I think is going to be make all of this much more available and much more usable is what is happening with health information technology. I do not think any of us could have imagined even 5 years ago how rapidly we are nowable to build these databases and how rapidly we are not able to access that information. And more importantly, get those best practices to the physicians right as they are treating the patients and having those practice guidelines, which is going to go a long way towards creating that value that we have all talked about in our healthcare system.

Mr. LANCE. Thank you. The Physician Payments Sunshine Act, usually known as the Sunshine Act, requires manufacturers of drugs and medical devices that participate in Federal health programs to report payments to physicians in teaching hospitals. In your judgment, has that data sharing in this regard been beneficial to medical innovation? Ms. Grealy?

Ms. GREALY. Again, I would caution, and I think we heard a lot today on this panel. We all believe in transparency. We think that is important, and having the disclosures about collaborations between physician and industry.

What we are most concerned about, and what we have actually seen already is the chilling effect, that physicians are concerned. Wait a minute, this is a minimum amount of money. It is just not worth it to have my name on a list when there is no context about what was the value of that collaboration.

And I think you heard Mr. Mussallem talk about their interactions with physicians as they are developing new cures, new devices. It is absolutely critical. And their partnerships with academic health institutions, absolutely critical.

So again, it is about balance. We think there should be reporting this information, but it needs to be in context so that people know what is the value of having physicians working with manufacturers, and how does that benefit patients.

Mr. LANCE. Thank you. My time has expired. Thank you, Mr. Chairman.

Mr. PITTS. The chair thanks the gentleman, and now recognizes the gentleman from Florida, Mr. Bilirakis, 5 minutes for questions.

Mr. BILIRAKIS. Thank you, Mr. Chairman. I appreciate it very much. First question is for Dr. Schmizzi. There are about 7,000 diseases and only about 500 treatments available. Patients with rare diseases frequently have no approved treatments. This forces these patients to find off label usage for medication to treat their condition. If the FDA has rules limiting information to doctors and patients, this could harm a patient's health. And I know this was touched on earlier.

Our health system should be patient-centered type of care, in my opinion. Given that, how can we ensure that patients and their physicians have access to information, whether it be on label or off label uses, so that it can determine the best course for treatment?

Dr. SCHMIZZI. Thank you, Congressman. I think the best way to do that is to ask the FDA or direct the FDA by legislation, or statute, or regulation changes, to allow that communication to go forward. Right that now communication is badly stifled, and much of the information that pharmaceutical manufacturers and innovators have is hidden from our view.

Rheumatology treats many diseases that have no defined treatment. There is no medication that is defined to treat Sjogren's syndrome. There is not defined treatment, no medication specifically defined to treat chondrocalcinosis. Some of these unusual diseases that are not really rare. We see a lot of people with that, but we have no defined mechanism or medicine that is approved for the use in these diseases.

But things like Sjogren's syndrome, I am certain that the pharmaceutical company that has manufactured some of the medications available today have had crossovers with patients who have Sjogren's syndrome, and they have data on how those patients' Sjogren's symptoms improved or worsened, which is also important to know. Did a particular medication make that particular subset of symptoms worse?

Those things are important for us to know, but those things may not be readily available to us. And those would be very helpful to have.

Mr. BILIRAKIS. Thank you. Would it improve the standard of care to have these indications on the label, such as ensuring correct dos-

age and access to insurance reimbursement? And should we incentivize sponsors to do the additional studies to get these off label uses on label?

Dr. SCHMIZZI. I think incentivization to do some of these studies on these small diseases would be very, very helpful. It took 15 years to define that dermatomyositis was treatable with a medication that has been on the market for 20 years. It took that long to get a large enough sample size to prove that the medication really worked. And dermatomyositis is a devastating inflammatory disease of the muscle that destroys muscle tissue and skin.

So incentivizing those types of things would go a long way. And the National Institutes of Health already does that, and they were the ones who sponsored the actual study on dermatomyositis. But incentivizing manufacturers to go forward with some of these smaller diseases would be very helpful, yes.

Mr. BILIRAKIS. Very good. Thank you very much. Mr. Mussallem, you mentioned that it was 4 years after the EU approval before the FDA approved the SAPIEN valve. Is the U.S. typically behind the EU for device approval? Why, and how can we accelerate the process?

Mr. MUSSALLEM. Yes, it was 4 years' difference. I would say generally in medical devices and medical technology, manufacturers would introduce their products first in Europe. The burden of proof to be able to introduce in Europe is much lower than the burden of proof required by the FDA.

There is a level of safety that needs to be established in Europe, but a much lower level of efficaciousness that is required that is required before it moves to the marketplace. And it is left to the judgment of physicians and patients on whether it should be used, and the FDA requires a much higher level of science to bring it to the United States.

Mr. BILIRAKIS. How can we accelerate the process here in the United States?

Mr. MUSSALLEM. Well, there are several ideas, and a number of them have actually been mentioned by Jeff Shuren, who is responsible for CDR–8, including trying to think more carefully and take a risk benefit analysis, and think carefully about what might be collected in a pre-market setting versus a post-market setting.

In the cases where patients really need the benefit, if you were to have a pre-market setting that was not so onerous, but rather have more extensive study in the post-market setting, what you could do then is potentially speed these cures to people that really need it when in the judgment of FDA it was the right thing to do. And at the same time, make sure that you are collecting the evidence so that therapies that are winners get supported and losers get stopped.

Mr. BILIRAKIS. Well, thank you very much. Appreciate that. And I yield back, Mr. Chairman.

Mr. PITTS. The chair thanks the gentleman, and now recognizes the gentlelady from North Carolina, Ms. Ellmers, 5 minutes for questions.

Mrs. ELLMERS. Thank you, Mr. Chairman. And thank you to our panel, especially Dr. Schimizzi—welcome—from Wilmington, North

Carolina, one of my very favorite places and just down the road from Dunn, North Carolina.

Dr. Jacques, I have a question for you. You state that CMS needs unambiguous authority to review clinical trials when claims related to these trials will be submitted for Medicare payment. In what ways is CMS authority in this respect limited now, and how does this impact the search for cures?

Mr. JACQUES. At a fundamental level, one would expect that the Medicare program or any insurance company would know what it is paying for as opposed to paying blindly. And my understanding is periodically Congress asks Medicare how much research are you paying for, and my understanding is the Agency has been unable to actually produce a number. So that would be helpful to know.

I think that Medicare engagement on research would actually serve a number of purposes because I have found much to my own frustration while I was a civil servant that there would often be times when companies would have come in with the data that they had, and we would sit there around the table going, if only 2 years ago you had made this small change it would have made a very large course direction in where you came up.

So the challenge is that Medicare covers routine costs in clinical trials based on a White House executive order from the end of the Clinton Administration. There is then a distinct regulation on FDA Category B investigational device exemptions, and then there is CED. And in any particular trial, there may be an overlap of those things, so CED would include routine costs, for example. There may be CED that might also be combined in the context of an FDA IDE approval study.

And because all of these things are siloed, it is very, very difficult at the staff level when a prospective investigator comes in and says, oK, here is my clinical trial. These are all the things I want to do. Can you tell me if it is covered or not. And that can be a conversation that takes months to get to all the details.

And I believe that if CMS simply had a singular authority that would relate to this, it could then publish an actual integrated policy where all the pieces actually fit, and you were not running all over the place trying to find different parts of an answer.

Mrs. ELLMERS. Mr. Mussellum and Dr. Schimizzi, it looked like you were very intrigued by Dr. Jacques' answer. Is there anything that either one of you would like to comment on?

Mr. MUSSALLEM. Yes. We think that just by listening to comments of Dr. Jacques and others in CMS, we have heard that there are limitations to what policy allows CMS to do. And also that there are limitations associated with their staffing levels, and that is concerning to us. We are dependent on payment to be able to bring these technologies to patients.

One of their particular things that are most noteworthy is much of the great medical breakthroughs come from individuals, or very small companies, or somebody that just has a great idea. And being able to take that from a napkin to reality is becoming longer and longer and more and more costly.

And to be able to have a conversation with CMS that clearly defines a predictable process would be very powerful to those organi-

zations. And this unpredictability has a chilling effect on innovation, so that kind of clarity would be very positive.

Mrs. ELLMERS. Dr. Schmizzi?

Dr. SCHMIZZI. I have found that what Dr. Jacques mentioned about staffing problems being an issue with national coverage determinations and local coverage determinations, contrasting one another, conflicting, very intriguing especially because that is a topic that has really hit us very hard this last year when we have a patient who may live in North Carolina part of the year and New York another part of the year, and they have Medicare. They may be able to get the medication in North Carolina, but when they go to New York the medication is denied because the carriers are different and the coverage determination is different.

It would be really nice to have a uniform set of rules.

Mrs. ELLMERS. Centralized.

Dr. SCHMIZZI. Yes. I mean, that is essentially what the national coverage determination was meant to do. But I can now understand, given what I have heard today, that it might indeed be a staffing problem that does not allow CMS to act on the national level, and allows individual carriers to make different determinations in different States, which makes it difficult for a patient to get the same care in different areas of the country.

Mrs. ELLMERS. Right. Well, thank you. And my time is about expired, so I will leave it at that. Thank you, Mr. Chairman.

Mr. PITTS. The chair thanks the gentlelady, and now recognizes the gentlelady from Colorado, Ms. DeGette, 5 minutes for questions.

Ms. DEGETTE. Thank you very much, Mr. Chairman, and thank you again for your commitment to these hearings.

Mr. Mussallem, in your written testimony you mentioned some of the advantages of registries to help with post-market surveillance. And you talk about the American College of Cardiology and the Society of Thoracic Surgeons working collaboratively to create the STSACCTVT registry. Can you talk to us a little bit more about that registry, when it was formed, the cost, and who can access that data and information?

Mr. MUSSALLEM. Yes, thank you. It was a remarkable collaboration. And again, routinely when a new technology is approved, FDA would mandate a post-approval study. In this case, the idea of mandating a post-approval study took a couple of forms, and an alternative was presented to collect data in a registry rather than collect more extensive data on a smaller group of people.

Through conversations with CMS—as a matter of fact, this actually became one where CMS also became partners in this discussion as well as a number of other stakeholders. It actually became part of the national coverage determination because the national coverage determination said we will pay for this new procedure if you are collecting evidence. And they did that under the provisions of coverage with evidence development.

So this registry became multi-purpose, and it did a few things. One is it became the post-approval study for FDA and to follow patients on a long-term basis with this new therapy, and every patient gets this, so it is a very large and powerful database. It became the tool used for evidence collection for CMS in terms of their

ultimate decision on coverage for evidence development. And it just became very powerful to the medical community because here was a set of data that rather than being managed by a company——

Ms. DEGETTE. Excuse me. Excuse me. They only give me 5 minutes.

Mr. MUSSALLEM. I am sorry.

Ms. DEGETTE. And I appreciate every minute of that. Who can access that data and information from that registry?

Mr. MUSSALLEM. Yes. That is exactly where I was going.

Ms. DEGETTE. OK.

Mr. MUSSALLEM. This data is managed by the American College of Cardiology and the Society of Thoracic Surgeons as a matter of fact. So it is housed within their organization, and so they have access to it. There is an advisory board that includes many of the members of those societies that actually ride herd over that data and publish results from that data on a routine basis.

Ms. DEGETTE. OK. Now, there are some limitations, I think, that you and others have pointed out with registries. And I am wondering, do you think it is just because we do not have a lot of experience with it?

Mr. MUSSALLEM. I think there is concern that we do not have experience with registries, and that is certainly true. We have no experience, for example, in our field of heart valves.

Ms. DEGETTE. Right. Right. So we just need to learn. Dr. Rising, I wanted to ask you quickly along these lines, in your testimony you talked about the Sentinel Initiative as a possible alternative or supplement to registries. Can you talk about how those can work for data collection?

Dr. RISING. Sure. I would be happy, thanks. So Congress instructed FDA to establish the Sentinel Program in 2007 to proactively monitor for problems with drugs and biologics on the market. And in 2012, Congress instructed FDA to expand Sentinel to include medical devices.

Now, what Sentinel is it uses claims data, almost exclusively claim data, housed by payers to look for associations between exposure to a particular product and then a particular health outcome.

Now, Sentinel could be expanded to devices except that right now there is no specific information on a device that is used in care on the claims form. So a payer might have information that they did a hip replacement that they are paying for, but they have no information on the specific hop replacements that were used.

Ms. DEGETTE. Right. Right.

Dr. RISING. So to expand Sentinel to include devices, a new field needs to be placed on the claims form. And in general, and we are a big supporter of using existing structures, such as claims forms, to capture new information like this.

Ms. DEGETTE. And, Mr. Mussallem, in your testimony you said that we need more resources and support for FDA. And I am wondering what types of resources you think we need. We have heard others talking about CMS. I am wondering about FDA.

Mr. MUSSALLEM. You know, I think the leadership at FDA has a pretty clear vision of some things that need to change, and they have done a pretty good job of articulating that through their strategic plan.

Ms. DEGETTE. Right. They have also told us about it, too. Thank you very much. Thank you, Mr. Chairman.

Mr. PITTS. The chair thanks the gentlelady. I have a unanimous consent request. I would like to insert a letter from the Lupus Foundation of America into the record.

Without objection, so ordered.

[The information appears at the conclusion of the hearing.]

Mr. PITTS. This has been another very informative and important hearing. Thank you very much for testifying today. We will have a lot of follow-up questions I am sure from members. We will send those to you. We ask that you please respond promptly. Members will have 10 business days to submit their questions for the record. That means they should submit their questions by the close of business on Tuesday, August the 5th.

Without objection, the subcommittee is adjourned.

[Whereupon, at 4:39 p.m., the Subcommittee was adjourned.]

[Material submitted for inclusion in the record follows:]

PREPARED STATEMENT OF HON. FRED UPTON

When we first launched the 21st Century Cures initiative in April, we had a pretty good idea that learning about the benefits and risks of a drug or device doesn't end when FDA initially approves or clears the product for use in certain patients with a specific disease or set of conditions. Since then, we have heard repeatedly that in many ways it is only just beginning.

Different uses for drugs or devices are constantly being discovered by physicians, researchers and scientists in academia and industry. Particularly in the context of devices, improvements are continually made to products based on new evidence being developed about how certain patients are responding to certain treatments, technologies, or combinations thereof. We must work to ensure that our regulatory and reimbursement policies encourage this iterative process and do not stifle innovation.

This type of ongoing evidence development, collaboration, and communication must be facilitated, not hindered by any policies in place that do not ultimately benefit patients. As part of the 21st Century Cures initiative, I am committed to evaluating how Congress can play a role in breaking down any of these legal or regulatory barriers and encouraging communication and collaboration between and among patients, doctors, and scientists regarding new data, research, and results.

At our digital healthcare roundtable, we learned about the many exciting opportunities to capture and analyze data in real-world delivery settings to generate meaningful insight and specific evidence about which type of treatments are working better on which type of conditions or diseases in which type of patients-often right down to the molecular level. During our joint hearing of the Health and Telecommunications subcommittees, we learned more about the role electronic health records and increased data sharing can play in that process, but also heard about the challenges and privacy issues we must address in order for such potential to become reality.

As we stated from the outset in our first Cures white paper, the policies we have in place must allow for health care delivery to serve as a platform for new discovery and development. This hearing will provide a great opportunity to learn how we can encourage and reward ongoing evidence development and not unduly limit how such evidence is discussed or communicated to patients and providers.

PREPARED STATEMENT OF HON. FRANK PALLONE, JR.

Thank you Chairman Pitts. Today's hearing is a broader effort to better understand how data collection and the exchange of patient information can be improved to help facilitate 21st Century Cures.

The development process of medical products, as we have learned, has many layers. Throughout our meetings on this initiative we have heard that FDA and NIH are driving medical advances and innovative approaches to clinical trial designs. NIH also develops and funds the basic research that makes medical advances pos-

sible. FDA, meanwhile, has made full use of early indicators of effectiveness, when the science justifies their use, to enable it to approve drugs based on more limited data than would otherwise be possible.

But there are still challenges to taking full advantage of these advances. For example, we've heard that there are obstacles to patient recruitment for clinical trials. Today I hope we can better understand about what methods can be used to facilitate initial product development but also allow for further evaluation of the effects of drugs and devices already on the market. I am particularly interested in the role patient registries and electronic health records can play.

We all want the best cutting edge medicines and treatments to get to the patients who need them. But we must also ensure that we have good tools for post market monitoring. So I'm also interested in how electronic health records can facilitate such monitoring and enable greater participation in clinical trials, while also safeguarding patient privacy under HIPAA.

Another topic we will hear extensively about today is how drugs and devices, once developed, get reimbursed—highlighting the process by which new drugs and devices under federal health programs like Medicare gain coverage. Clinical trials don't always provide the necessary clinical evidence to enable the Medicare program to determine whether the coverage of a particular drug or device is reasonable and necessary for its particular patient subpopulation. With the inability of Medicare to negotiate prices and the increasing price of new drugs and biologics, it is incumbent upon Medicare to be very diligent in its coverage decisions.

Getting a treatment or a cure to a patient has implications for industry, payors and patients alike. So how do we ensure access to these products? In addition, medicines and treatments alone will not ensure the best outcomes. Providers have a critical role to play in the quality of care patients receive.

Mr. Chairman, these are complicated issues. I want our researchers and scientists to have access to the funding necessary to make discoveries; I want our companies to operate in an environment where innovations can flourish; and I want patients to have access to safe and effective treatments. I'm not entirely sure a package of laws is needed to accomplish all of these goals, but I'm hopeful that Democrats and Republicans can work together moving forward to accomplish these goals.

Thank you.

July 22, 2014

Help Us Solve
The Cruel Mystery
LUPUS
FOUNDATION OF AMERICA

The Honorable Joe Pitts
Chairman

The Honorable Frank Pallone, Jr.
Ranking Member

Committee on Energy and Commerce
Subcommittee on Health
2125 Rayburn House Office Building
Washington, DC 20515

RE: July 22nd Hearing on the 21st Century Cures Initiative: Examining Barriers to Ongoing Evidence Development and Communication

Dear Chairman Pitts and Representative Pallone:

Thank you for the opportunity to continue the important dialogue about the Committee's *21st Century Cures* Initiative. The Lupus Foundation of America is the only national force dedicated to improving the quality of life for all people affected by lupus through programs of research, education, awareness, and advocacy. Lupus is an unpredictable and misunderstood autoimmune disease that ravages different parts of the body. It is difficult to diagnose, hard to live with, and a challenge to treat. Lupus is a cruel mystery because it is hidden from view and undefined, has a range of symptoms, hits out of nowhere, and has no known cause and no known cure.

The Subcommittee's July 22nd hearing memo discussed that "different uses for drugs and devices are being discovered constantly, many times for treatment of new conditions and diseases or for populations of patients other than for which they were initially approved." In the case of lupus, this statement is on target. The most commonly prescribed medications for treating lupus are prescribed "off-label," and it is critically important for all stakeholders to have the ability to freely share peer-reviewed information about the safety and efficacy of medications used to treat lupus.

Currently, there are only four FDA-approved medications to treat lupus – Benlysta®, hydroxychloroquine (an antimalarial), prednisone (a steroid), and aspirin. Benlysta®, approved by the FDA in March 2011, is the first and the *only* drug designed specifically to treat lupus. All other medications used to treat lupus are drugs approved for other indications such as chemotherapies, used to treat cancer, and immunosuppressants, used post organ transplantation.

2000 L Street NW, Suite 410 Washington, DC 20036 Toll Free: 1 800 558 0121 Main: 202 349 1155 Fax: 202 349 1156 lupus.org

The above treatments, other than Benlysta®, have never been specifically tested in lupus. For example, Rituximab was developed and approved for use in rheumatoid arthritis, but it has shown efficacy in some lupus patients and is prescribed "off-label" as a treatment for lupus.

Another example is related to the treatment of lupus nephritis or lupus kidney disease, one of the most serious forms of lupus. Lupus nephritis is most often treated by two different therapies - CellCept and Cyclophophamide. Neither have been fully tested in lupus nor approved for use in lupus by the FDA; yet, both are considered the standard of care in lupus nephritis. Interestingly, Cellcept was tested against the chemotherapy, and Cellcept was found to be equivalent but not superior; thus, never approved by the FDA.

The real-world provides the perfect opportunity to learn more about the richness of medications and their other potential uses beyond their FDA designation. People with lupus must be treated effectively and in a timely manner, with the best resources available-regardless of whether it is indicated for lupus or not. Otherwise the consequences can be costly and life threatening --including lupus flares, organ failure and long-term hospitalizations. It could be considered unethical not to communicate vital off-label information to physicians treating diseases like lupus.

It is the Foundation's position that the prescription of off-label medications to people with lupus may already be the standard of care and the communication of these uses may advance public health. If you have any questions or wish to discuss further, please contact Kim Cantor, Vice President of Advocacy and Government Relations at (202) 349-1150 or at cantor@lupus.org.

Sincerely,

Sandra C. Raymond

Sandra C. Raymond
President & CEO
Lupus Foundation of America

August 11, 2014

Dr. Louis Jacques
Senior Vice President and Chief Clinical Officer
ADVI
1050 K Street, N.W.; Suite 340
Washington, D.C. 20001

Dear Dr. Jacques:

Thank you for appearing before the Subcommittee on Health on July 22, 2014, to testify at the hearing entitled "21st Century Cures: Examining Barriers to Ongoing Evidence Development and Communication."

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.

To facilitate the printing of the hearing record, please respond to these questions with a transmittal letter by the close of business on August 25, 2014. Your responses should be mailed to Jessica Wilkerson, Legislative Clerk, Committee on Energy and Commerce, 2125 Rayburn House Office Building, Washington, D.C. 20515 and e-mailed in Word format to Jessica.wilkerson@mail.house.gov.

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 Frank Pallone, Jr., Ranking Member, Subcommittee on Health

Attachment

Louis B Jacques, MD Responses to: Additional Questions for the Record from The Honorable Joseph R. Pitts – August 25, 2014

Q1. "In previous hearings...FDA approval is not the last hurdle...predictability of the CMS coverage process?"

It is important to acknowledge that some medical technologies are not intended for use in Medicare beneficiaries and thus CMS coverage policy is not determinative for them. In this context CMS may have no particular interest after FDA approval or clearance. However, CMS is appropriately expected to know what it pays for, and to have assurance that such items and services are reasonable and necessary as described in the statute. Unfortunately, some small and medium sized innovator companies seem focused on the requirements of FDA review and unmindful that Medicare and other payers have their own requirements for coverage and payment. Issues that could have been readily addressed well before FDA approval or clearance are therefore seen by some parties as last minute hurdles.

The most efficient way to address these issues for small and medium size companies is to support their earlier engagement with CMS and other payers regarding the questions that will arise in the context of coverage and payment. Both FDA and CMS have signaled interest in such a new paradigm, exemplified by the FDA-CMS Parallel Review pilot and other initiatives.

I believe the Medicare coverage process itself is predictable and transparent in comparison to some other Federal administrative processes and those of private companies. The challenge is that many small companies do not seem to be aware of the many published materials that describe and demonstrate examples of the coverage process and its outcomes. Similarly they seem unaware of the interest of CMS coverage staff to meet with innovators. In summary, they don't know what they don't know.

While the Parallel Review pilot is demonstrating positive results as recently as the August 11, 2014 proposed Medicare coverage of a DNA based screening test for colorectal cancer, the formal Parallel Review paradigm may be more than what is needed by some innovators. Given the geographic proximity of CMS and FDA offices, the establishment of a jointly supported interagency "innovator engagement space" could make these resources more obviously available and apparent to innovators. FDA could, as a standard procedure, inform these innovator companies of the availability of such voluntary early stage engagements.

Q2. "The most recent SGR patch legislation..."

Some members of the venture capital community have expressed interest in the establishment of a PAMA-like paradigm for truly innovative medical technologies, similar to provisions for advanced diagnostic tests. This could entail several PAMA-like components.

- A statutory definition that would define these technologies and distinguish them from incremental technologies. This might entail a combination of a) FDA PMA status; b) evidence of high likelihood of substantial new clinical benefit in a Medicare beneficiary population; and c) other factors established by the Secretary of HHS.

- Manufacturer product specific coding that would uniquely identify the item in the claims process.
- Payment calculation based initially on invoice amounts followed by a calculated payment rate that reflects commercial contracts.

Q3. "You state that CMS needs unambiguous authority to review clinical trials..."

Medicare serves beneficiary populations that are commonly excluded from enrollment in clinical studies because they have comorbid conditions associated with permanent disability, end stage kidney disease and advanced age that can complicate the interpretation of clinical trial results. In this context, the reported outcomes of some clinical trials do not describe how the studied technologies would impact these Medicare beneficiaries. Clearer incentives to enroll beneficiaries in clinical studies could support the development of better treatments for patients who have complicated chronic conditions, particularly the frail elderly patient.

As noted above in the response to the first question, some technologies are not intended for use in Medicare beneficiaries, and Medicare would not be asked to cover their clinical trials. However, CMS is appropriately expected to know what it does pays for, whether in clinical trials or usual clinical care. It is also reasonable to expect that Medicare coverage policy for research will reflect attention to the needs of the program and its beneficiaries. This will not happen if CMS has to rely on a piecemeal approach.

Current CMS authorities regarding coverage of items and services in clinical trials are siloed in three distinct vehicles: a) the June 2000 Executive Memorandum on coverage of routine clinical care costs in trials, i.e. usual care that the patient would get if not enrolled in a trial; b) statutory establishment of coverage of FDA approved IDE trials; and c) Coverage with Evidence Development (CED) under AHRQ's authority. This lack of integration stymies the development and publication of unified policy on the matter and unnecessarily lengthens the time to address coverage issues for clinical studies. Innovators seeking Medicare coverage for clinical trials don't have a unified set of criteria for reference. This engenders a reluctance to seek Medicare coverage for trials, with the consequence that innovators need to seek funding from other sources or conduct a more limited clinical study that may leave important questions unanswered.

The current paradigm does not address coverage in clinical studies for innovative technologies that may be studied in research sponsored by other Federal agencies or outside of an IDE or CED. Thus there may be missed opportunities to use the additional support that would be furnished by Medicare coverage to address important beneficiary-centric research questions.

Q4. "You state in your testimony that Local Coverage Determinations..."

Small device companies have historically expressed a preference to engage with local Medicare Administrative Contractors (MACs) as an alternative to opening a national dialog with CMS. As a practical matter, this approach represents a lower cost and lower risk strategy and may work well for a company whose facilities are entirely within a single MAC jurisdiction. However, the technology may be supported by preliminary evidence insufficient for coverage in the Medicare beneficiary population. A

broader LCD authority could permit earlier Medicare coverage on the condition of additional evidence development specifically to address the clinical impact of the device in the beneficiary population.

The current statutory definition of a Local Coverage Determination (LCD) is described in Section 1869(f)(2)(B) as:

> For purposes of this section, the term "local coverage determination" means a determination by a fiscal intermediary or a carrier under part A or part B, as applicable, respecting whether or not a particular item or service is covered on an intermediary–or carrier–wide basis under such parts, in accordance with section 1862(a)(1)(A).

This has the practical result of impeding the development of LCDs on any of the other "reasonable and necessary" provisions contained in Section 1862(a)(1)(A) of the Act. This also applies to clinical studies that might be supported under Coverage with Evidence Development, which is articulated by CMS as a decision under 1862(a)(1)(E) of the Act.

August 11, 2014

Mr. Michael A. Mussallem
Chairman and Chief Executive Officer
Edwards Lifesciences Corporation
655 15th Street, N.W.; Suite 385
Washington, D.C. 20005

Dear Mr. Mussallem:

Thank you for appearing before the Subcommittee on Health on July 22, 2014, to testify at the hearing entitled "21st Century Cures: Examining Barriers to Ongoing Evidence Development and Communication."

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 Frank Pallone, Jr., Ranking Member, Subcommittee on Health

Attachment

Mussallem QFRs
Health Subcommittee Hearing
July 22, 2014

The Honorable Joseph R. Pitts

1. ***In your testimony, you note that the U.S. innovation ecosystem is eroding, can you elaborate as to what is contributing to this weakening? What actions should Congress take to reverse this trend? Based on your experience as an executive in the medical device industry, what do you think are the ingredients to ensure we have a robust innovation ecosystem?***

The U.S. innovation ecosystem is eroding: As this Committee has heard in prior "21st Century Cures" testimony, there are a variety of factors contributing to the weakening of the innovation ecosystem. Recent data from the National Venture Capital Association help to illustrate the decline. Between 2007 and 2013, medical device investments fell by a total of 40 percent. In 2007, there were 98 companies amassed approximately $576 million in initial venture capital. Since then, there has been a 50 percent reduction in the number of device companies receiving initial venture capital investment and an approximately 70 percent drop in the amount of capital invested. In 2013, the U.S. witnessed the lowest level of medical device initial funding activity in more than two decades. Last year, only 44 new venture device companies raised a total of $163 million compared to of which there was 98 companies in 2007.

Strict regulatory requirements and lengthy review processes have contributed to this erosion: From Edwards Lifesciences' viewpoint, the regulatory approval process and the U.S. reimbursement system have been significant barriers to timely market access for new, innovative technologies. The increasingly burdensome scientific inquiries from FDA require more robust and longer clinical trials, which are costly and delay opportunities for firms to recoup their investment. This hampers the ability to have innovative technologies available in the US in a timely fashion. For example, we've invested more than 10 years in the pursuit of U.S. approval for the SAPIEN transcatheter heart valve platform, dedicating time, resources and significant funding to product development, clinical trials and data collection and analyses. Due to the large amount of clinical data required for approval in the US, the SAPIEN valve was obsolete in Europe and was replaced by the next version of the device (SAPIEN XT) in Europe two years before the original SAPIEN valve was approved in the US. The ability of the patients in the US to obtain life sustaining medical devices lags significantly from other countries due to the large clinical studies that are required for approval in the US.

Inspections of manufacturing sites should be a more collaborative effort between FDA and manufacturers. FDA should be encouraged to take a more risk-based approach to compliance inspections. In other countries, governments partner with the local companies to help them comply with regulations.

Additionally, heightened scrutiny of the economic value of new technologies at the earliest stages of their development create a significant risk that a technology may not meet third-party payers' (e.g., Medicare) requirements for coverage and adequate payment. It is imperative to recognize that medical device innovations become more effective and more efficient with time, experience and device improvement. If we hold new innovations to the same unforgiving standard that we hold well-established technologies that have been honed to near perfection over decades, we will miss opportunities to help American patients with new and transformational technologies. We need a system that takes into account the healthcare system's learning curve, and does not shut the door to evaluation on day one, while always maintaining patient safety along the way.

Costly and time-consuming data gathering requirements, combined with uncertainty regarding reimbursement amounts and coverage, yield uncertainty and delays in a company's ability to begin sales of its product and recoup its investment. These barriers and risks created by the U.S. regulatory and reimbursement system discourage investment in new, breakthrough technologies.

Necessary ingredients to a robust innovation ecosystem: Based on the Edwards experience, there are essential elements to fostering an ecosystem that incentivizes curiosity and rewards innovators who develop new therapies for patients, including: patient/physician needs clearly communicated and ascertainable, ready access to capital and supportive economic climate, functional, timely and predictable regulatory processes, a reimbursement system that welcomes novel therapies as they undergo a continuous improvement process, and strong intellectual property protection.

Actions Congress should take to reverse the erosion of our innovation ecosystem: Congress can provide FDA greater support and – where necessary – changes to statute to provide the agency authority to streamline the product approval process. Some areas where FDA has begun to make improvements is through proposals to streamline the clinical trial IDE approval process, reduce legal complexity between the different hospital IRBs, incorporate patient preferences and tolerance for risk into the decision-making process, and allow for the use of surrogate endpoints or data from sources outside the U.S. during the safety and efficacy evaluation. In addition, FDA has proposed to shift some of the pre-market review process to the post-market setting. And efforts have been made to improve reviewer training, better align the pre and post-market surveillance functions at FDA, and allow for expedited appeals of FDA decisions. We support these initiatives, and they need to move faster. Congress can help in that regard, particularly in the form of providing additional financial resources for FDA to expedite these new initiatives.

Additionally, CMS's authority could be expanded to allow for the encouragement of medical technology innovation. Our healthcare system must be willing to pay for promising new technologies, even though they may appear costly in the near term. If there is a significant potential that a new technology can improve clinical outcomes, quality of life, and overall healthcare economics, then CMS should develop policies to allow for coverage and payment of qualifying technologies. It would stimulate innovation if CMS provided coverage and adequate payment for a fixed period of time to allow a technology to develop before measuring the technology's cost-effectiveness.

2. ***We've heard that the amount of data that has to be collected to gain U.S. regulatory approval and reimbursement is substantial. Can you give us an idea on how much clinical and economic evidence Edwards had to generate to obtain regulatory approval and reimbursement in the U.S.?***

Leading up to the initial FDA approval of the Edwards SAPIEN transcatheter aortic heart valve, our company generated a substantial amount of clinical evidence including a large, complex randomized controlled clinical trial in the US. Extensive study of this valve – including an unprecedented four *New England Journal of Medicine* papers – has demonstrated the "triple win": a substantial and sustainable clinical benefit, extraordinary quality-of-life improvement, and cost effectiveness in inoperable patients. In fact, the SAPIEN valves are the most studied heart valve in history, with more than 300 peer-reviewed, published articles on clinical outcomes associated with the valves. There are also more than 120 cost-effectiveness and quality of life

articles related to transcatheter aortic valve replacement (TAVR). Subsequent indications and different access routes (used when a direct percutaneous approach is not possible) for SAPIEN were studied in registries, and we conducted a second large trial in the US – PARTNER II – for SAPIEN XT, a much improved and lower profile device that was approved by FDA in June. Accompanying these large randomized trials have been cost effectiveness and quality of life studies supporting the value of the SAPIEN family.

Following FDA approval and the Medicare National Coverage Decision (NCD) that provided reimbursement for TAVR through Coverage with Evidence Development (CED), Edwards and other TAVR manufacturers are required to support the TVT patient registry. Created by The Society of Thoracic Surgeons (STS) and the American College of Cardiology (ACC), the TVT Registry is designed to monitor and benchmark patient safety and real-world outcomes related to the TAVR procedure. The burden and cost of complying with registry requirements is not insignificant. For example, the patient data registry form for the STS/ACC TVT Registry for TAVR procedures is eight pages long and consists of more than 300 separate fields, requiring special staffing, and dedicated personnel, and hours of work to complete this exhaustive form. Many physicians have told us that it takes longer to fill out the TVT Registry form than it does to perform the procedure. In addition to the significant financial commitment manufacturers must make to support the development and ongoing operations of registries, hospitals are charged ongoing fees to participate.

3. ***Based on your experience with an innovative medical technology, what improvements to the premarket approval process can be made from an evidence generation perspective?***

There are improvements that should be made to the premarket approval process and FDA is already taking on a number of initiatives to improve the regulatory processes to help improve patient access to innovative therapies. Thanks to the Food and Drug Administration Safety and Innovation Act (FDASIA), FDA has agreed to improved review and approval performance metrics tied to dramatic increases in manufacturers' user fees, and we are just beginning to see positive performance.

FDA has recently proposed a number of improvements to the premarket clinical trial process that hold promise, including:

- Streamlining the investigational device exemption (IDE) approval process
- Reducing the legal complexity and inconsistency between each hospital Institutional Review Board (IRB) through the creation of a centralized or standardized review process
- Incorporating patients' voices and tolerance for risk into the regulatory decision making process
- Addressing potentially duplicative clinical evidence through the consideration of surrogate endpoints and greater use of data developed outside of the U.S.
- Providing a more risk-based application of FDA requirements during inspections prior to an approval

Congress could encourage FDA by providing additional support to help expedite these changes and give them room to innovate.

4. As the sole manufacturer involved in the support and development of the TVT registry, what are the benefits of a registry?

Edwards Lifesciences was the first medical device manufacturer to have a transcatheter aortic valve replacement device approved by FDA. Since the approval of Edwards' first TAVR device, another company has gained FDA approval of a TAVR device. Both device companies are part of a Stakeholder Advisory Committee that meets to get updates and provide advice to the TVT Executive Committee.

There are many benefits to a registry, including real-world data collection that helps further refine appropriate patient populations. Registries can also potentially be used to remove pre-market data collection hurdles with enhanced post-market data collection. In the case of registry used with CED, it enables reimbursement in cases where payers have questions regarding the "generalizability" of the clinical data to their populations.

Other potential benefits of registry include the ability to accelerate future indication expansion utilizing registry data and the opportunity for more streamlined surveillance, if done appropriately and not in addition to existing (and generally ineffective) systems.

5. There has been a lot of positive, supportive discussion regarding patient registries. Are there any risks or costs to them? What are they?

In addition to the many benefits of patient registries, there are some risks and costs. Specifically, there is a danger that registries could become the de facto data collection mechanism for all technologies when in certain cases, level of evidence or risk is well-established and additional data collection is not needed. In some cases, the quality of data collected by the registry may be incomplete or poor, leading to inaccurate conclusions.

The centralized control of registry data could prevent independent or different research approaches from participating in the clinical and academic dialogue about the medical device. Registries may pose a barrier to access for patients who don't wish to consent to their data being collected or who refuse to participate in follow-up.

Irresponsible use of data could threaten patient access. For instance, there could be inappropriate comparison of device-to-device performance. Cherry-picking data may lead to "sensational" findings and headlines.

Registries have financial and administrative burdens. The financial burden on the healthcare system for the creation and maintenance of registries can be high, and they can be a factor in driving up the cost of healthcare. They often pose significant burdens on providers in terms of time and the cost of data management. There is also the potential for duplicative information collection.

Finally, it is important to recognize that registries cannot be left open in perpetuity – once evidence is mature, requirements need to stop so that resources can be directed to help generate evidence for new therapies.

6. What guiding principles should be applied when deciding when and how to develop a registry?

Registries can help improve patient outcomes by providing greater understanding of the effects of products in the real-world and can facilitate patient access to new therapies by efficiently collecting accurate data to support expanded device use and indications. Additionally, registries can provide regulators with alternative methods to monitor the performance of technologies, allowing them to shift the burden of pre-market data collection to the post-market setting. But to realize these benefits, it is important that medical device registries be carefully designed, implemented and maintained.

A key component of the recently released AdvaMed registry principles is a series of "threshold questions" intended to assure that creation of a registry is the appropriate mechanism for meeting the defined objective:

- Are there reliable data collection instruments available to collect the data needed to achieve the objectives?
- Will the registry have a stable and diverse source of funding to promote long-term sustainability?
- Is using a registry the least-burdensome means to collect the necessary data to achieve the scientific objectives?
- Do the objectives warrant the level of investment required to develop and maintain a registry?

In addition, the principles outline several key elements that should guide the development of any medical device registry, including: establishment of a data governance committee to oversee issues on ownership, access and use of any data collected; prospective registry design, to establish clear objectives and data analysis plans; policies for sharing the data collected with qualified scientific or medical researchers; and policies for the use and publication of registry data.

Registries need to be flexible on how and what is collected. As new information and methods are developed the older systems need to be replaced striving for lower costs through better efficacies. Once adequate information is obtained on an outcome, that information should no longer be collected.

Registries should also not be redundant tools for postmarket safety monitoring. If a registry can successfully perform device surveillance functions for FDA, then the devices being monitored by the registry should be excluded from other surveillance mechanisms.

The medical technology industry is committed to the principles of evidence-based medicine. Registries can be an important tool for gathering useful information about the safety and effectiveness of interventions involving medical devices and diagnostics, but only if they are designed and executed properly.

7. Does the reimbursement system hold new, game-changing innovations to unrealistic evidentiary standards?

The reimbursement system's heightened scrutiny of the economic value of new technologies at the earliest stages of their development create a significant risk that a technology may not meet third-party payers' (e.g., Medicare) requirements for coverage and adequate payment. It is imperative to recognize that medical device innovations become more effective and more efficient with time, experience and device improvement. If we hold new innovations to the same unforgiving standard that we hold well-established technologies that have been honed to near perfection over decades, we will miss opportunities to help American patients with new and transformational technologies. We need a system that takes into account the healthcare system's learning curve, and does not shut the door to evaluation on day one, while always maintaining patient safety along the way.

8. Has the FDA taken specific steps that have enhanced evidence development mechanisms and how can they be improved? If so, what are they?

April 2014, FDA released draft guidance entitled "*Balancing Premarket and Postmarket Data Collection for Devices Subject to Premarket Approval; Draft Guidance for Industry and Food and Drug Administration Staff.*" In this guidance, FDA has made efforts to clarify and update its policies concerning the balance between premarket and postmarket data requirements.

While not specifically addressed in the draft guidance document, there typically is little flexibility in requirements of IDE study design, data requirements, and follow-up for postmarket studies. Risk profile, targeted patient population, OUS clinical data, and non-clinical testing results should be taken into consideration in determining premarket IDE study design, and any postmarket studies should be designed accordingly. For example:

- Where the performance of a particular device type is well-studied, documented, and understood, the clinical data collection should be able to rely on OPCs, Patient Reported Outcomes (PROs), or other data instead of requiring a randomized controlled clinical trial.
- Similarly, if a product has excellent long-term OUS clinical performance data, this should be taken into consideration when determining clinical data collection. This is a mechanism that could be used to strike an evidence-based balance between premarket and postmarket studies. In short, IDE study designs should focus on unanswered questions rather than requiring collection of data answering questions that are well understood.

9. Have other countries created reimbursement incentives for innovation? If so, what are they and could you seem them working here in the U.S.?

Yes, other countries have created reimbursement incentives for innovation. For example, when Germany – which prides itself on its innovative climate – introduced its DRG system, policymakers there recognized the potential for hospitals to focus on costs to the exclusion of potential longer-term benefits of innovation. In response, Germany created an "innovation clause," which allows for hospitals to apply for additional funds each year to pay for innovative therapies. In addition, Japan provides medical technologies a 5% bonus reimbursement payment if the product is introduced in Japan before the U.S.

Mussallem QFRs
Health Subcommittee Hearing
July 22, 2014

The U.S. healthcare system is unique, and other countries' incentive mechanisms may not be effectively adopted in the United States exactly as designed. As the current world leader in medical innovation, the U.S. should be cautions in following the example of competitive countries. Instead, the U.S. should identify creative approaches to better valuing and driving faster innovation that will provide Americans not only transformational healthcare technologies, but also drive business growth and job creation.

The Honorable Renee Ellmers:

1. ***Mr. Mussallem, in your testimony, you mention "reducing the legal complexity and inconsistency between each Institutional Review Board (IRB) through the creation of a centralized or standardized review process". In my district, I represent one of the largest clinical trial service providers, Quintiles. It is my understanding that without the use of a centralized IRB, clinical trials can be hindered because of the current excessive review process, where clinical trials are referred to many IRB's. What can be done to promote or help expedite the IRB review process?***

Under current law, hospital Investigational Review Board (IRB) approval is necessary for conducting a clinical trial. In the case when a company has a large clinical trial with several trial sites, that company will have to seek IRB approval from each trial site. This can be a lengthy, complex and costly process. Additionally, if a significant change is approved by FDA during the trial, additional IRB approvals have to be obtained.

FDA and others have proposed creating an optional centralized or standardized review process, which could make the IRB approval process more efficient and reduce unnecessary cost and burden for innovation. However, this still requires centralized IRB approval, which can be time consuming. Additionally, it may not solve the problem, as hospitals frequently will not accept a centralized IRB approval, believing they are legally required under statute, regulation and policies outlined by the National Research Act of 1974. There may be alternative approaches to improving this process, and we would be happy to work with the Committee to identify and develop alternatives.

2. ***Mr. Mussallem, as a committee, we've heard that the amount of data that has been collected to gain U.S. regulatory approval and reimbursement is substantial. Can you give us an idea on how much clinical and economic evidence Edwards had to generate to obtain regulatory approval and reimbursement in the U.S.?***

Leading up to the initial FDA approval of the Edwards SAPIEN transcatheter aortic heart valve, our company generated a substantial amount of clinical evidence including a large, complex randomized controlled clinical trial in the US. Extensive study of this valve – including an unprecedented four New England Journal of Medicine papers – has demonstrated the "triple win": a substantial and sustainable clinical benefit, extraordinary quality-of-life improvement, and cost effectiveness in inoperable patients. In fact, the SAPIEN valves are the most studied heart valve in history, with more than 300 peer-reviewed, published articles on clinical outcomes associated with the valves. There are also more than 120 cost-effectiveness and quality of life articles related to transcatheter aortic valve replacement (TAVR). Subsequent indications and different access routes (used when a direct percutaneous approach is not possible) for SAPIEN were studied in registries, and we conducted a second large trial in the US – PARTNER II – for SAPIEN XT, a much improved and lower profile device that was approved by FDA in June.

Accompanying these large randomized trials have been cost effectiveness and quality of life studies supporting the value of the SAPIEN family.

Following FDA approval and the Medicare National Coverage Decision (NCD) that provided reimbursement for TAVR through Coverage with Evidence Development (CED), Edwards and other TAVR manufacturers are required to support the TVT patient registry. Created by The Society of Thoracic Surgeons (STS) and the American College of Cardiology (ACC), the TVT Registry is designed to monitor and benchmark patient safety and real-world outcomes related to the TAVR procedure. The burden and cost of complying with registry requirements is not insignificant. For example, the patient data registry form for the STS/ACC TVT Registry for TAVR procedures is eight pages long and consists of more than 300 separate fields, requiring special staffing, and dedicated personnel, and hours of work to complete this exhaustive form. Many physicians have told us that it takes longer to fill out the TVT Registry form than it does to perform the procedure. In addition to the significant financial commitment manufacturers must make to support the development and ongoing operations of registries, hospitals are charged ongoing fees to participate.

August 11, 2014

Dr. Gregory F. Schimizzi
Co-Founder
Carolina Arthritis Associates, P.A.
1710 South 17th Street
Wilmington, N.C. 28401

Dear Dr. Schimizzi:

Thank you for appearing before the Subcommittee on Health on July 22, 2014, to testify at the hearing entitled "21st Century Cures: Examining Barriers to Ongoing Evidence Development and Communication."

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.

To facilitate the printing of the hearing record, please respond to these questions with a transmittal letter by the close of business on August 25, 2014. Your responses should be mailed to Jessica Wilkerson, Legislative Clerk, Committee on Energy and Commerce, 2125 Rayburn House Office Building, Washington, D.C. 20515 and e-mailed in Word format to Jessica.wilkerson@mail.house.gov.

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 Frank Pallone, Jr., Ranking Member, Subcommittee on Health

Attachment

Responses to Questions for the Record

Gregory F. Schimizzi, MD
Coalition of State Rheumatology Organizations (CSRO)

House Energy and Commerce Subcommittee on Health Hearing Titled:
"21st Century Cures: Examining Barriers to Ongoing Evidence Development and Communication"

Tuesday, July 22, 2014

Questions from the Honorable Joseph R. Pitts

1. Advances in data collection, recordkeeping and technology are helping to facilitate the development of tools and are presenting new opportunities to communicate truthful and non-misleading clinical information that will result in more precise clinical decision-making. It is important to note that when talking about "off-label" uses, it doesn't just refer to completely different indications of use, but also can include slight variances around the same indication that could include dosing, route of administration, subpopulations, previous therapies used by the patient, etc. Do you believe the current restrictions on off label communications are limiting health care professionals' ability to provide the most appropriate treatment to patients? If so, do you have recommendations on how to address these barriers?

As outlined in my written testimony, my understanding is that the FDA does not allow pharmaceutical companies to actively distribute any key information, even if it is related to the on-label indication, unless it is explicitly referenced in the package insert. Therefore, observational data, subpopulation information, comparative data derived from clinical trials other than randomized controlled trials, and pharmacoeconomic or comparative cost data cannot be proactively shared with clinicians unless such data is directly referenced in the package insert. Further, for medically acceptable alternative uses, such as those which may be referenced in various compendia, publicized in medical presentations and meetings or practice guidelines as an appropriate treatment, that data can only be shared if a clinician directly and specifically requests such information from the manufacturer. By limiting the sharing of information, physicians are hampered in their ability to access all available sound medical evidence and firm scientific rationale necessary to treat patients with difficult problems.

For example, one of our distinguished colleagues attempted to proactively request information to aid in the treatment of a patient with sclerits, which is an inflammatory disease of the eye that can occur in diseases such as rheumatoid arthritis. Left untreated, this condition has potentially devastating consequences including complete loss of vision or even perforation of the eye itself. Due to current regulations and limitations that require a physician to explicitly request information, effective treatment of this patient's condition was delayed. This particular patient did not immediately respond to traditional therapy options, but our colleague remembered a presentation suggesting that rituximab may be a suitable physician-directed application. After several failed attempts to contact the speaker, he contacted the pharmaceutical company directly and requested any specific data that the

manufacturer possessed relating to this specific potential use. He received the required information, and the product helped his patient. However, the 2-3 weeks required to obtain all of the necessary information, patient consent, and then insurance authorization, caused unnecessary delays in treating his patient and impacted the outcome by delaying access to safe effective care.

It would be preferable to allow the pharmaceutical company with its wealth of information to share key data in order to inform and assist in decision–making. That is not to say that I would recommend a change in all of the current requirements that the FDA to review such information to ensure that it is truthful and not misleading. **The CSRO urges the FDA to expand the current process of review of materials beyond what is included in the package insert to also cover other key data.** The FDA review process should occur in real-time and not potentially delayed for a year or more. **In addition, CSRO urges the FDA, through a public process, to develop standards for qualifying real world data, so that clinicians can be better informed.** With additional comparative effectiveness research, the focus on quality outcomes, and other health care reforms, Congress and the FDA should be encouraging the exchange of scientific information, not hampering it. Blocking access to data on medically acceptable alternative uses seem to countermand these new requirements and complicate my ethical responsibility to provide patients with information on risks, benefits and alternatives to medical treatments as part of the informed consent process. As we move closer to newer, alternative payment models (APMs), where shared decision-making tools will likely be a key component, I am concerned about how this lack of information will impact my ability to truly educate and manage patients with difficult problems on their treatment options and give them a fair opportunity to engage in the establishment of their care plan.

2. While the goal of manufacturers is to run appropriate clinical studies that will enable FDA approved labeled indications, the reality is that it is impossible to accomplish this for all clinical variations due to a multitude of factors including limitations on clinical trial enrollment and cost. Manufacturers have access to robust data sets and information about their products for on and off-label uses. How can we find a way for patients and providers to have access to appropriate, material information about the product to ensure the best clinical treatment decision is made?

It would be helpful to allow pharmaceutical companies to collect and distribute, upon request from providers, insurers and other stakeholders, pertinent post marketing information that is publicized at medical meetings, academic conferences and investigator presentations regarding non-approved indications for their pharmaceutical products. The pharmaceutical companies usually have fist hand knowledge regarding physician directed clinical trials, compassionate use initiatives, and off-label uses of their products but are prohibited by the FDA to discuss or distribute the pertinent information. The company representatives and science liaisons usually attend many academic subspecialty meetings but are also prohibited by FDA regulations from discussing the conclusions or findings from these meetings. Sharing this information with clinicians who have difficult-to-treat patients failing conventional therapies in an open manner (perhaps with FDA oversight) would facilitate treatment, help in educating the patient and help to streamline the acquisition process. Physicians need to feel confident about the medication, need provide necessary information to affected patients and have to produce scientific information to insurers when prescribing the medication deemed to be medically necessary.

3. Are there examples of times when patients would have benefited from information about treatments outside of the PI?

Yes, thank you for asking such an important question. Patients always have concerns about the treatment being recommended or prescribed. Most are wary of the experimental uses and off-label/non-approved indications. Along with a discussion of risks and benefits of the individual medication, the availability of scientific information and data – even if limited - is extremely useful in creating confidence in patients when using an agent in a non-approved condition.

4. Currently manufacturers cannot actively distribute any key information, even if it's related to the on-label indication, unless it is explicitly referenced in the package insert. You note that the information these companies have could be invaluable to practitioners, can you please explain further how having access to this information would benefit practitioners and patients?

There are varying types of data being collected by manufacturers that are currently unavailable to providers. This is because FDA's definition of "off-label" captures all information outside the four corners of the package insert. The types of data currently being collected by manufactures that could benefit the practice of medicine if physicians were able to access it include:

- ***"Real World"/Observational Data:*** Randomized, controlled clinical trials are the gold standard for approval of a product, but the responsibility to study that product does not end with the completion of a Phase III trial. During the post-market phase of a product's life, manufacturers collect "real world evidence" based on observations and patient experiences. Such evidence may be collected through medical records, including electronic health records, adverse event reporting, registries, and observational studies. These data sets can be invaluable in learning how subpopulations traditionally underrepresented in trials are responding to the product. This type of real world evidence provides crucial insights for physicians, yet FDA regulations limit the dissemination of these data and analyses.
- ***Comparative Data:*** Biopharmaceutical companies perform so-called "head-to-head" studies comparing two drugs approved to treat the same condition. These studies may show that one drug is safer or more effective in treating the condition in question, which is key information for a physician to have when (s)he is faced with a choice between the two products. However, companies may not proactively provide this type of information to physicians, if it is supported "only" by one trial. FDA requires that the information be supported by *two* adequate and well-controlled studies.
- ***Sub-Population Data:*** In the course of a clinical trial, a manufacturer may learn that, for example, women respond to the product differently than men. There may be differential effects on patients of different racial or ethnic backgrounds that could be gleaned from data stratification from pivotal trials. Some of the data can be indicative of potential benefits in other areas of medicine and may warrant further study. Such findings (for example, a noticeable decline in the number of myocardial events in the treatment group African–

American subpopulation compared to the placebo group African-American subpopulation) may not be the objective of the trial and are therefore not included in the labeling. As such, a company cannot communicate that information to physicians unless specifically requested– yet this type of outcomes data could be useful to providers, especially providers who work with the subpopulation in question.

- ***Meta-Analyses:*** Researchers often "pool" the results of a group of studies because trends may emerge that would not be apparent in a single, standard trial. These "meta-analyses" contrast and combine results from a range of investigations and identify relationships across the results. In addition, meta-analyses may also detect patterns in prescribing, dispensing, or administering drugs, which can be helpful in prevention of inappropriate use.
- ***Medically-Accepted Unapproved Uses or Combination Uses:*** For the treatment of some diseases, FDA may not have approved a particular use of a medicine, even though it is medically accepted by virtue of its inclusion in major compendia or treatment guidelines. Similarly, the combination of two FDA-approved products may not be included in either product's label, but may be the standard of care. In such cases, manufacturers are prohibited from informing providers that compendia or treatment guidelines support those uses of a medicine, unless FDA finds that use safe and efficacious based on two adequate and well-controlled trials.

In essence, current FDA regulations assume that health care providers cannot be trusted to correctly interpret data relevant to their field and to leverage that data to make the best care decisions for their patients. Physicians should have access to information that is truthful and non-misleading, regardless of the source. Under the current FDA paradigm, a patient can Google a drug and obtain inaccurate and potentially dangerous information from anyone with access to a computer, yet a physician cannot receive information from the one party with the most comprehensive data set about a particular product. This is nonsensical. FDA should revise its regulations to allow companies to share truthful, scientifically accurate, and data-driven information with health care professionals.

5. What is the effect on physician practice when FDA makes it difficult for you to receive information and data from drug and medical device companies?

Essentially, patient care is hampered especially for those with rare diseases (orphan diseases) and those who fail all other conventional therapies. Going back to my previous example, by making it difficult for a physician to receive the key information, there was a delay of nearly 2-3 weeks before the patient could receive the appropriate care. Such an unfortunate delay has the potential of having a negative impact on outcome for an individual patient.

6. One component of the Alliance's position on Physician-Directed Applications is that if specialty physicians use a product for an indication not in the approved or cleared labeling, they have the responsibility to (1) be well informed about the product, (2) to base its use on a firm scientific rationale and sound medical evidence, and (3) to maintain awareness of the product's use and effects." How do physicians learn about these additional indications and what resources do they utilize to keep themselves informed and aware of the latest developments?

As outlined in my written testimony, many non-approved indications can be found in standard textbooks of medicine and surgery in all specialties and subspecialties for patients of all ages and are the generally accepted standard of medical care. In some instances the lack of information is harmful and forces physicians to choose between using an agent off label without the advantage of useful information, using only approved medications that do not work well in a specific patient or doing nothing at all. In addition, in some patients with orphan diseases or illnesses that are poorly understood, non-approved therapies are the only treatments available. Management modalities for these are frequently publicized in scientific meetings, peer-reviewed literature, and other compendia. Publicizing these uses is an important method of communicating effective treatments in the medical community and a source of investigational stimulation to academicians and clinicians into new areas of research and development.

August 11, 2014

Ms. Mary Grealy
President
Healthcare Leadership Council
750 9th Street, N.W.; Suite 500
Washington, D.C. 20001

Dear Ms. Grealy:

Thank you for appearing before the Subcommittee on Health on July 22, 2014, to testify at the hearing entitled "21st Century Cures: Examining Barriers to Ongoing Evidence Development and Communication."

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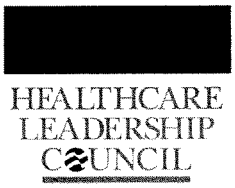
Thank you again for your time and effort preparing and delivering testimony before the Subcommittee.

Sincerely,

Joseph R. Pitts
Chairman
Subcommittee on Health

cc: The Honorable Frank Pallone, Jr., Ranking Member, Subcommittee on Health

Attachment



August 29, 2014

The Honorable Joseph R. Pitts
Chairman
House Energy and Commerce Subcommittee on Health
2125 Rayburn House Office Building
Washington, DC 20515

Dear Chairman Pitts:

Thank you again for inviting me to appear before the House Energy and Commerce Subcommittee on Health on July 22, 2014 to testify at the hearing entitled, "21st Century Cures: Examining Barriers to Ongoing Evidence Development and Communication." I welcome your additional questions for the record, and I have attached my responses.

I have also included, for your reference, a recent publication by the Healthcare Leadership Council and the Bipartisan Policy Center addressing the challenges of federal health data access. This publication builds upon a roundtable discussion of experts the two organizations convened this spring.

Thank you for your continued leadership on these important topics. We look forward to continuing our work with you and your staff to identify existing barriers to allowing healthcare data to drive better care quality and value. Please feel free to reach out to Tina Grande, Senior Vice President, at tgrande@hlc.org or (202) 449-3433, with any additional questions or for clarifications on any of the responses detailed in this letter.

Sincerely,

Mary R. Grealy
President

cc: The Honorable Frank Pallone, Jr., Ranking Member, Subcommittee on Health
The Honorable Renee Ellmers

Attachment(s)

Additional Questions for the Record

1. **Ms. Grealy, you mention in your testimony that HIPAA was created at a time when policymakers were not thinking about the knowledge that could be gained by accessing data residing in large databases. How does HIPAA need to change in order to ensure that data can be used effectively for this vital research?**

The HIPAA Privacy and Security Rules generally work well for the covered entities and their business associates who are under its jurisdiction. We see no reason for significant change in these rules based on new developments in technology or otherwise. These rules – particularly the Security Rule – have been drafted to accommodate technological change on an ongoing basis.

At the same time, there are details of the HIPAA research rules that can be modified to improve the overall ability of the health care system to benefit from health information in the research context. The Department of Health and Human Services (HHS) already has begun a proceeding to modify the existing HIPAA rules related to research. HHS published an "Advance Notice of Proposed Rulemaking" in July of 2011. We attach the comment letter prepared by the Confidentiality Coalition (convened by HLC) on the advance notice. We have encouraged HHS to move forward with a proposed rule that will streamline the existing HIPAA research processes to permit a broader and easier use of health information in connection with research.

Furthermore, HIPAA establishes a perverse disincentive for covered entities to use health data to pursue "generalizable knowledge" – that is, for research. The HIPAA Privacy Rule defines "health care operations" to include, for example:

> *"conducting quality assessment and improvement activities, including outcomes evaluation and development of clinical guidelines,* **provided that the obtaining of generalizable knowledge is not the primary purpose of any studies resulting from such activities;** *patient safety activities (as defined in 42 CFR 3.20); population-based activities relating to improving health or reducing health care costs, protocol development, case management and care coordination, contacting of health care providers and patients with information about treatment alternatives; and related functions that do not include treatment" (emphasis added).*

Pursuant to this provision, covered entities (such as hospitals and medical practices) can use patient information for "internal research," to improve their own protocols and develop appropriate standards, but are limited in their ability to then publish or disseminate these results to others for broader public purposes, even if no patient information whatsoever is disclosed during that publication. We believe that, in line with the current effort to streamline the research requirements, covered entities who engage

in permitted "health care operations" activities should then be permitted to publish results "for generaliziable knowledge," so long as no patient identifiable information is disclosed during the publication. This goal could be accomplished through a revised HIPAA Privacy Rule provision or, more directly, through guidance from HHS that addresses this idea of "primary purpose" and makes clear that a health care provider that develops useful information from its patient data may then disclose the results to others.

2. **You stated that in most research environments, patient data must be de-identified before it can be utilized but note that there are circumstances in which de-identified data is not sufficiently useful to achieve particular objectives. Would you expound upon this a little farther and explain how we should take this into account in any policy changes we consider as part of this initiative?**

   While there are various processes by which patient information can be used for research purposes, one option involves the "de-identification" of "protected health information" or "PHI," using a defined HIPAA standard, so that this "PHI" is no longer identifiable to a particular patient. At that time, the data can be used for research purposes without the need for patient permission or any other HIPAA compliance steps. However, data that has been "de-identified" according to the HIPAA standard also may not be particularly useful in a research context, because so many identifiers have been eliminated. For example, de-identified data does not contain dates associated with the individual, which makes any sort of longitudinal or chronological research nearly impossible. There are other mechanisms whereby patient data can be used for research purposes, such as the "limited data set" that can include dates in data that otherwise has been de-identified. We encourage HHS to develop additional rules and/or guidance that permits a broad use of this data where primary identifiers have been removed, either through a broader "limited data set" provision or by encouraging privacy review boards to permit disclosure of a broader range of data for research purposes without the need for additional and burdensome patient authorizations. Where appropriate procedures have been implemented (such as those required in connection with a limited data set), and where research entities have developed appropriate security procedures and means of ensuring that patient identities are not disclosed, we believe that patient privacy can be protected while still permitting more effective research. Privacy Review Boards (who have authority under HIPAA to approve research projects without the need for specific patient authorization) should be given broader guidance and additional encouragement to approve research projects where appropriate protections for patient data are in effect.

3. **You note that there are 50 separate sets of state privacy laws and regulations that can be incredibly difficult to navigate. You believe strongly that a national privacy framework should replace this current patchwork of state laws. Would you explain what you mean by a national privacy framework and why you think it is necessary?**

The complexity of the legal structure regulating privacy is a monumental barrier. Even where HIPAA applies, it is not the only rule to abide by. States have hundreds of different, inconsistent, and overlapping laws that create meaningful compliance and operational challenges. This complexity, by itself, works against appropriate use and disclosure of information, as health care providers and others do not know how to act in many circumstances. Most of these laws (although not all) were passed before the HIPAA rules went into effect. Many of these state laws do not appropriately address any kind of electronic technology or the broader levels of cooperation and information-sharing that are common and beneficial component of the current health care system today. Many of these laws do not clearly permit the use of any vendors to assist an entity in performing services. Interpreting how these states laws compare to HIPAA is exceedingly difficult, confusing and time consuming. There is virtually no guidance on these laws, and little enforcement of these provisions. In fact, this patchwork of laws has made it extremely difficult, if not impossible, for organizations such as Health Information Exchanges (HIEs), to share data across state borders. I do not think I am exaggerating in saying that this unaligned patchwork of state privacy laws is a reason that HIEs have failed to flourish. While the HIPAA rules create a federal baseline for privacy protection, we encourage Congress to make this baseline the applicable standard nationwide, by preempting these other state laws. The HIPAA standard should be the governing standard for any entities (covered entities and business associates) that are covered by the HIPAA rules. A national standard would facilitate nationwide information exchange, interoperability, and help patients by allowing the right information to reach their providers whenever and wherever they need it.

4. **You state in your testimony that federal health data should no longer be denied to entities perceived to have a commercial interest. What is preventing agencies from making this data available now?**
    a. **How would clarifying and modernizing any such laws and policies benefit federal public health agencies?**
    b. **Are there operational or organizational changes that could help enhance collaboration within and between federal public health agencies?**

There is standing HHS policy that prohibits the sharing of certain federal program data with entities that have a "commercial interest." Entities with commercial interest can access public use files and limited dataset files. However, direct access to Research Identifiable Files (RIFs, which includes the Part D Prescription Drug Event data) is generally prohibited for these entities. Entities that are presumed or determined to have a commercial interest are denied access to RIFs that contain person-level, protected health information (PHI). The exact origin of this policy is unclear, however it is referenced in various CMS documents.

The Centers for Medicare and Medicaid Services (CMS) recently reopened discussion of this important distinction in the January proposed rule on Medicare parts C and D (Medicare Advantage and Prescription Drug Benefit Programs for Contract Year 2015). In our rapidly evolving healthcare sector, the way in which data are being used has changed dramatically. Patient level information is needed to achieve the very care transformation CMS seeks. The lines are blurred with respect to which types of entities have commercial interest – commercial purposes could encompass much more than just a product or tool. Because the quality and efficiency of all physician groups, health plans, hospital systems and suppliers can be enhanced using data, any notion that commercial interest is limited and discrete is outdated.

Within organizations currently excluded, there is deep scientific and analytic expertise which enables a broader understanding and knowledge of public health issues across the entire healthcare ecosystem. Ultimately, any standard that essentially bars access to important data is detrimental to the larger goals of our healthcare system and our common goals for the evolution of that system.

It is not fair nor does it make sense in an era when all stakeholders, regardless of their tax status, are vital partners in improving the healthcare system. We believe that federal health data should no longer be denied to entities perceived to have a commercial interest. Healthcare organizations are using advanced data analytics to improve healthcare quality, better manage population health and address consumer needs using private-sector patient-level data. These organizations can enhance their work with appropriate access to federal program data.

HLC believes that all researchers should be subject to the same rules of data access for PHI. Current rules for access include:

- *Strong research design*
- *Research question must assist CMS in managing programs/improving services*
- *Researcher must have expertise and experience*
- *Researcher must sign a Data Use Agreement generally concerning handling and use of the data*
- *Researcher will not disclose research findings if such findings can be linked with other data where an individual's identity can be deduced*
- *Researcher will adhere to CMS cell size policy.*

These rules are sufficient to ensure quality, patient-serving research and should be applied to all organizations, regardless of "commercial interest."

There are a host of important public policy considerations that should lead to a revision in how CMS and HHS view access to RIF by a broad range of researcher requestors. The goal is a high-functioning, efficient, quality healthcare system. It will take all stakeholders in that system to reach that important goal within the foreseeable future.

5. **Would you explain what is current federal policy with respect to allowing innovative companies to access such data and why this is an impediment to additional discovery and development?**

   Please see the attached publication by HLC and the Bipartisan Policy Center for details on current data access requirements and how changes in these restrictions could lead to innovative new healthcare options for consumers.

6. **Are there more collaborative data sharing policies or initiatives in place in other countries that we could learn from?**

   This is a great question, but is outside our area of expertise. The question is very complicated due to the significant structural differences between how various nations deliver healthcare.

7. **You attach a number of examples in your testimony about lifesaving and life-transforming innovations that are the direct result of collaboration between physicians and drug and device companies. How could misinterpretation of the Sunshine Act impact this critical type of interaction and how can we proactively avoid any such unintended consequences?**

   Two points. One, I think people give short shrift to the importance of educating physicians on the purpose, impact and potential side effects of new pharmaceuticals and medical devices so that they can safely and effectively make the right decisions for their patients. What some pejoratively describe as enticing physicians to use a drug or device is actually this essential education. But, second, we are seeing no indication that the Sunshine Act will explain the purposes for these transactions between physicians and manufacturers. Maybe a payment is for education on the proper uses of a new product, maybe another is for the hands-on insights that lead to new innovations in organ transplantation. This is the importance of context, to which I referred in my testimony. Less important than the dollars involved in these interactions is the impact on patient care and medical progress. Information without context can have a chilling effect on the willingness of providers to engage in collaboration and, in fact, we're already receiving reports of physicians disengaging from their prior working relationships with manufacturers. We believe it is imperative that Congress as well as all sectors of the healthcare community insist that the Centers for Medicare and Medicaid Services fully explain the nature and patient benefits of the transactions of value between physicians and innovative healthcare companies. Congress must make it clear that it will not abide any action that slows or halts medical progress that is vital to millions of patients and consumers.

8. **Data analytics of huge bodies of data holds the potential to spur innovation and development in disease areas that haven't seen a new drug in 50 years. In your testimony, you state that "we are now in an era where researchers can harness vast amounts of data to learn at a rapid pace unlike we have ever seen." We all support the need to protect patient data: is the potential you see in big data spurring development of new treatments limited by HIPAA?**

   As discussed above, we encourage a streamlining of the HIPAA rules related to research to permit a broader ability to take advantage of the broad range of health care data that can be available for research purposes. We believe strongly that these improved research practices will result in improved quality of care delivery, reduction of costs, and will lead to other benefits that we cannot yet imagine. The ongoing HHS regulatory process addressing these potential changes may be the appropriate vehicle for this effort, and we encourage HHS to move forward with this activity. We are not implying that researchers have carte blanche access to all identifiable information without any oversight, but rather, to thoughtfully develop an approach through HIPAA that makes it easier to access data to do research that improves individual and population health, benefiting society for this and future generations. We also encourage appropriate development of rules and/or guidance to govern research proceedings that relate to useful data that is outside the current structure. Researchers within the healthcare industry can utilize data from other sources where there are appropriate protections for this data.

9. **We are entering an age where technological innovation and data have the potential to reinvigorate cures discovery and development in this country, but only so far as the regulation of these technologies allow us to go. In your opinion, do we need to review the current HIPAA and privacy paradigm in this country to ensure it is truly protecting patients – both from a privacy but also from an accessibility perspective?**

   As discussed above, the current HIPAA structure strikes the appropriate balance between data sharing and the protection of patient confidentiality where it applies. At the same time, there has been a substantial growth in the volume of health related data that is available outside of the HIPAA structure (and other data that is not considered "health data" but that may be useful or relevant to appropriate research). Much of this information can be valuable for research and innovation purposes. There are various ongoing efforts to review potential regulation of this "non-HIPAA" healthcare data. While we encourage development of appropriate standards related to this "non-HIPAA" healthcare data, we must be vigilant that new laws and regulation do not stifle data-driven innovation, which is dependent on the ability to access and share data.

Question from the Honorable Renee Elmers

1. **Ms. Grealy, in North Carolina we have academic medical centers like Duke and UNC, and joint-partnerships between the individual physicians, the bio-pharma companies and the teaching hospitals are crucial for innovation. Therefore, I'd like to know, what is the role that academic medical centers play in supporting innovation?**

   Academic medical centers play a vital role in finding new treatments and cures. Nearly 85% of NIH's budget is awarded to medical schools and universities, which has resulted in many medical advances, such as in the treatment of leukemia. At one time 80% of children diagnosed with leukemia died. Today, the survival rate stands at 90% thanks to research funded by NIH and conducted in academic medical centers. Also, Mr. Mussallem mentioned in his testimony the Transcatheter Aortic Valve Replacement. The development of this lifesaving device came about due to a partnership between Edwards Lifesciences and New York Presbyterian hospital, another HLC member and premier academic medical institution.

Health Program
Health Innovation Initiative

Access to Federal Health Data: A Key Imperative for Improving Health and Health Care

Meeting Proceedings

On April 3, 2014, the Healthcare Leadership Council (HLC) convened a roundtable of public- and private-sector leaders in collaboration with the Bipartisan Policy Center's (BPC) Health Innovation Initiative to explore the benefits of federal health data, current challenges associated with access and use, and the policy changes needed to support both the availability and utility of such data, while effectively managing and maintaining privacy.

The roundtable included more than 35 leaders representing numerous sectors of the health care industry, including academic and research institutions, hospitals and health systems, health plans, life sciences organizations, technology companies, and the federal government.

To lay the foundation for the discussion, representatives from the Centers for Medicare and Medicaid Services (CMS), the National Institutes of Health (NIH), and the Department of Veterans Affairs (VA) provided an overview of current agency policies and procedures governing data-sharing and access.

Insights offered by participants in the roundtable discussion are summarized in this report.

Key Take-Aways

Benefits of Federal Data Access

Access to federal health data helps clinicians and other providers make better clinical decisions. It also supports emerging delivery system and payment models that have been shown to improve health and health care. Access also plays a key role in supporting consumer decision-making and improving population health.

Key Challenges

Challenges associated with federal health data identified by participants fall into three primary categories:

- Limitations on access to Medicare data
- Lack of flexibility in Data Use Agreements
- Restrictions associated with those who have a commercial interest

Policy Considerations

1. Further explore and encourage government-wide policies and standards for health data-sharing

2. Engage in a broad public discussion regarding situations where restrictions on health data access are appropriate

3. Expand access to federal data sets for health and health care improvements, with appropriate protections

BIPARTISAN POLICY CENTER

HEALTHCARE LEADERSHIP COUNCIL

Benefits of Federal Data Access

American health care is moving at an unprecedented pace toward a data-driven, information-based system that will improve health outcomes, increase efficiency in health care delivery, and improve the quality of care. Health care data plays a critical role in these transformation efforts.

The use of health data:

- Helps clinicians and other providers make better decisions, leading to higher-quality, more cost-effective care;
- Powers rapidly emerging delivery system and payment models that have been shown to improve both health and health care;
- Supports efforts to improve population health, including clinical and comparative effectiveness research, monitoring and responding to public health and safety threats, and measuring outcomes to support improvements;
- Empowers consumers by helping them make better health care decisions as well as understand and manage their own health.

Given the promise of big data, the federal government has begun to promote new levels of data transparency and access for public and private entities. However, these current efforts are not robust enough to address the significant barriers that remain in appropriately accessing data that will allow these goals to be achieved.

Current Federal Policies Associated with Federal Data Access

The "open government initiative" was created in 2009 by the federal government to establish a system of transparency, public participation, and openness in government.[1] Aimed at addressing multiple broad issues, its impact on health care is tangible. As part of this effort, several health-related federal agencies are currently engaged in increasing access to federal health data, including the Agency for Healthcare Research and Quality (AHRQ), the Centers for Disease Control and Prevention (CDC), CMS, the Food and Drug Administration (FDA), NIH, the Substance Abuse and Mental Health Services Administration (SAMHSA), and the VA. An overview of a subset of these efforts is provided below.

Centers for Medicare and Medicaid Services

As the nation's largest payer of fee-for-service claims, representing 35 percent of total national health expenditures, CMS is the largest source of data that could be used to improve the quality and cost-effectiveness of care.[2] According to CMS, it already shares "more data in more formats" than any similar organization. With respect to questions about data reuse, CMS clarified that it allows reuse of data on a frequent basis, despite public misconceptions.

CMS has specific rules and procedures governing the release of Medicare and Medicaid data, summarized in more detail below. Access restrictions vary depending upon the type and cost of data, the applicability of certain

privacy-related laws and regulations, and availability of CMS resources.

The agency only allows access to data after applicable legal procedures are followed, regardless of the type or urgency of request. However, legal procedures have evolved and will continue to evolve over time to make data more accessible for legitimate needs. CMS has specified that research using certain data must benefit CMS in its effort to monitor, manage, and improve the Medicare and Medicaid programs or the services provided to beneficiaries.

CMS maintains a list of all the data that is collected within the Systems of Records (SOR).[3] Any data with specific personal health identifiers is subject to the Privacy Act of 1974, the Health Insurance Portability and Accountability Act (HIPAA), and other federal government rules and regulations.[4,5]

CMS data falls into one of the three categories listed below:

- *Research Identifiable Files (RIFs)* contain protected health information (PHI). RIF requests are subject to review by CMS' Privacy Board to ensure that the beneficiary's privacy is protected and the need for identifiable data is justified. CMS requires all RIF requestors to sign a Data Use Agreement (DUA).[6]
- *Limited Data Sets (LDS)*, which contain PHI from which certain specified direct identifiers of individuals and their relatives, household members, and employers have been removed. LDSs also require DUAs.[7]
- *Public Use Files (PUFs)*, which have been stripped of any personal identifying information.[8]

Embracing the administration's open government initiative, CMS engages in the following key efforts:

- *Qualified Entity (QE) Program*: Created under the Affordable Care Act, the QE program provides a framework for improved access to Medicare Part A, Part B, and Part D data wherein compliant QEs are expected to combine Medicare data with data from other payers to create more accurate provider performance reports.[9]
- *CMS' Virtual Research Data Center (VRDC)*: A subscription-based tool for conducting research using CMS data. The VRDC offers researchers several advantages, such as less costly data and access to more timely data.[10]
- *Proposed Rulemaking*: In January 2014, CMS issued a proposed rule that invited comments on a number of aspects of Part D data access, including whether its current ban on access to Part D Drug Event data for commercial purposes should be revised to allow access for research with a commercial purpose.[11] The agency will review the comments received as it contemplates reforms to data access policies.

The Department of Veterans Affairs

The VA participates in the Open Data Initiative which is intended to make information easier for the public to find and to facilitate its reuse by developers, non-profits, and other third parties to improve the quality and cost of health care.[12] By serving as both a payer and provider for a high number of individuals with mental health or behavioral disorders, the VA operates amid heightened concerns about record privacy and consent. Also, data from veterans' health records carry a higher risk for being re-identified (after de-identification) than other records because, in part, veterans are a smaller population. Due to such sensitivities, the VA generally releases data only to investigators with a VA affiliation, rather than entities outside of the VA.

The VA does have an interest in facilitating greater data-sharing, particularly for the purposes of collecting more data on the care that veterans seek outside the VA system.

National Institutes of Health

NIH has taken steps to increase access to federal data. For example, it funds research that generates a greater volume and wide range of data in genome wide association studies (GWAS) and has extended the current policy to encompass data from a broader spectrum of human and non-human genomic research as part of this effort.[13] In 2014, NIH developed an online database of genotypes and phenotypes to which researchers have access.[14] The White House Office of Science and Technology Policy's (OSTP) request to formalize policies on data-sharing sparked NIH's current process of drafting internal policies governing different types of data.[15] Such policies are expected to be released soon.

NIH notes that future policies on data-sharing regarding genomic data will allow researchers to access sensitive data for legitimate uses.

Discussion Summary

Access to Medicare Data

Ensuring adequate access to Medicare data is a widely held concern. CMS has specified that research using certain

data must benefit CMS in its effort to monitor, manage, and improve the Medicare and Medicaid programs or the services provided to beneficiaries. Many roundtable participants believe that broadening this interpretation will create further benefits to both CMS programs and patients by dramatically increasing the bandwidth for research leading to increased care quality, system efficiency, and consumer satisfaction. While many restrictions are important and necessary, other current restrictions inhibit the true potential of data analysis in health care.

For example, access to Medicare Part D Program data must be considered differently than Part A and Part B data because CMS placed new and significant restrictions on the use of Part D data when implementing the program. Under the Part D Program, private prescription drug plan sponsors must submit to CMS a Prescription Drug Event (PDE) record that contains comprehensive information for every prescription filled under a Part D plan, which includes more than 25 million Medicare Part D beneficiaries. When linked to other Medicare claims for hospitalizations and physician services, these data are a rich source of information about patterns of drug treatment, health outcomes, and adverse events among the elderly and disabled that, to date, have not been available. Currently, access to RIFs, which include the Medicare Part D data, is not allowed under a variety of situations—including when the researcher is associated with a commercial enterprise. CMS will consider reforming the program after it reviews comments received in response to its January 2014 Proposed Rule.[16] In addition, the forthcoming proposed rule on accountable care organizations (ACOs) may be another opportunity to address access to federal data for Medicare Shared Savings Program participants.

Data Use Agreements

CMS requires external researchers to sign a DUA that outlines certain restrictions placed on the data. Several challenges are created by DUAs required by CMS. First, in ACOs, DUAs prohibit data-sharing outside of the requesting organization. In an ACO, this might restrict the appropriate sharing of health data among a beneficiary's multiple providers.

Second, DUAs generally require that the data be destroyed at CMS' request, which can interfere with HIPAA tracking and compliance requirements. CMS is currently assessing ways to facilitate data access while preserving CMS control of its data.

Restrictions Imposed on Those With Commercial Interest

Currently, restrictions to federal health care data access are imposed on organizations with a "commercial interest." Entities with commercial interest can access public use files and limited dataset files.[17] However, direct access to RIFs, which includes the Part D PDE data, is generally prohibited for these entities. The genesis and rationale for restricting commercial entities' access to data is not well documented. Data access restrictions on commercial entities prevent these entities from using data for research that benefits the public, such as improving clinical trial design or studying the use and effectiveness of a treatment. Academic organizations have greater access to federal data because historically these organizations have tools in place—such as peer review procedures—that create limits on their use of the data. CMS acknowledges that academic organizations can also use data for commercial purposes rather than purely academic purposes and that the distinction between commercial and academic entities for the purposes of data access may need to be reconsidered.

A more structured definition of commercial interest that focuses on the use of the data as opposed to the organization that uses the data may be more appropriate. Roundtable participants encouraged CMS to expand the discussion of appropriate access to PDE data by entities with commercial interests to the broader, long-standing Department of Health and Human Services policy that denies access by commercial entities to federal Medicare A, B, D, Medicaid, and possibly other program datasets. Many believe it is time to reconsider this overarching policy that affects access to federal program RIFs in Medicare, including Part D, and in other federal health programs.

These concerns are relevant for more than just government and commercial entities. Other efforts to leverage health data for system-wide improvement, such as those through the Patient Centered Outcomes Research Institute (PCORI), face possible challenges due to restrictions on data access and use. PCORNet—PCORI's large, widely representative, national network for conducting clinical outcomes research—is designed to help a wider audience access health data in order to perform comparative effective research studies.[18]

Several potential approaches to improve the current data restrictions imposed on commercial entities and other users were proposed during the HLC-BPC roundtable discussion, including:

- Improving and expanding the current peer-review process used for academic research to commercial research;
- Educating patients about the benefits of data-sharing and expanded data access to facilitate higher levels of patient consent and cooperation;
- Issuing requests for information and holding future roundtable meetings to explore revisions to current data-sharing restrictions in a way that balances research needs and privacy protections; and
- Basing data access on considerations such as whether the entity is using data for the public good and whether the entity has appropriate data security measures in place.

Policy Considerations

Based on insights shared by meeting participants and previous policy work, HLC and BPC offer the following policy considerations.

1. **As part of the administration's open government initiative, the government should further explore and encourage government-wide policies and standards for health data-sharing**. These would include uniform data access methods and usage agreements across federal agencies in order to simplify the process for organizations seeking data. Consistency across federal agencies could reduce confusion among data users and allow third parties to more efficiently analyze the U.S. health care system.
2. **The federal government should convene all stakeholders for a broad discussion of situations where restrictions on data access are appropriate**. As a product of this discussion, government could establish a more consistent rationale for restrictions on health data that continue to exist. This discussion should revisit the feasibility of regulating access by intent of the researcher, rather than by the type of organization involved.
3. **Broaden efforts to share most federally held health data, when appropriate**. Data collected from federal government programs, particularly those funding new and innovative care delivery models or tools, should be available for research, with appropriate privacy protections. Private-sector organizations should have access to information on programs and services they deliver—particularly when this information supports decision-making. As partners to the federal government in national efforts to improve care while lowering costs, private-sector organizations should have access to the tools needed for success.

Conclusion

Discussions during the HLC and BPC roundtable shed new light on key policy issues surrounding increased access to federal health data for improving health and health care in the United States. This meeting report touches briefly on the role of federal data and current strategies for increased access and sharing, and also offers crucial insights into some of the greatest challenges to future progress. Ultimately, it is clear that the federal government, along with additional public- and private-sector leaders and policymakers, must continue to foster and engage in the kind of rich dialogue that occurred during this roundtable discussion in order to move the nation forward toward better care and better health for all citizens.

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About the Healthcare Leadership Council

The Healthcare Leadership Council (HLC), a coalition of chief executives from all disciplines within American health care, is the exclusive forum for the nation's health care leaders jointly to develop policies, plans, and programs to achieve their vision of a 21st century system that makes affordable, high-quality care accessible to all Americans. HLC members advocate measures to increase the cost-effectiveness of American health care by emphasizing wellness and prevention, care coordination, and the use of evidence-based medicine, while utilizing consumer choice and competition to elevate value. HLC works to accelerate the growth of health information technology in order to promote quality improvement and improve care through patient information-sharing while also protecting important patient privacies.

Based on the interest of its member CEOs, HLC has convened leaders from all disciplines within American health care to consider the challenges and opportunities of "big data" health policy. HLC envisions a future in which public- and private-sector health care organizations securely share information in an efficient, effective manner that is accessible and useful for all stakeholders. HLC members have already proved that they can harness data to improve care and value in health care. Improved accessibility and quality of health data can accelerate progress in medicines, improve the quality of care delivery, reduce costs, and will lead to other benefits that cannot yet be imagined. See www.hlc.org.

About the Bipartisan Policy Center

Established in 2007 by former Senate Majority Leaders Howard Baker, Tom Daschle, Bob Dole, and George Mitchell, BPC is a nonprofit organization that drives principled solutions through rigorous analysis, reasoned negotiation, and respectful dialogue. With projects in multiple issue areas—such as democracy, economic policy, energy, housing, immigration, national security, and health care—BPC combines politically balanced policymaking with strong, proactive advocacy and outreach.

The BPC Health Innovation Initiative conducts research and collaborates with experts and stakeholders to advance recommendations that promote innovation and drive improvements in the cost, quality, and patient experience of care. BPC's work in supporting the use of data to improve health and health care includes convening leaders and releasing numerous reports that address the electronic information sharing needs of both individuals and new models of care and the policies and strategies required to accelerate information sharing. See www.bipartisanpolicy.org.

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Disclaimer

This report is a product of the HLC and BPC. This meeting summary was prepared by HLC and BPC staff as a factual summary of discussions that occurred during the meeting hosted by HLC in collaboration with the BPC Health Innovation Initiative on April 3, 2014. The statements made are those of the authors or individual meeting participants and do not necessarily represent the views of all of the meeting participants. Also, the findings and recommendations expressed herein do not necessarily represent the views or opinions of the Bipartisan Policy Center, its founders, or its board of directors.

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National Pharmaceutical Council

Joe Selby,
Patient Centered Outcomes Research Institute

Kristen Axelsen,
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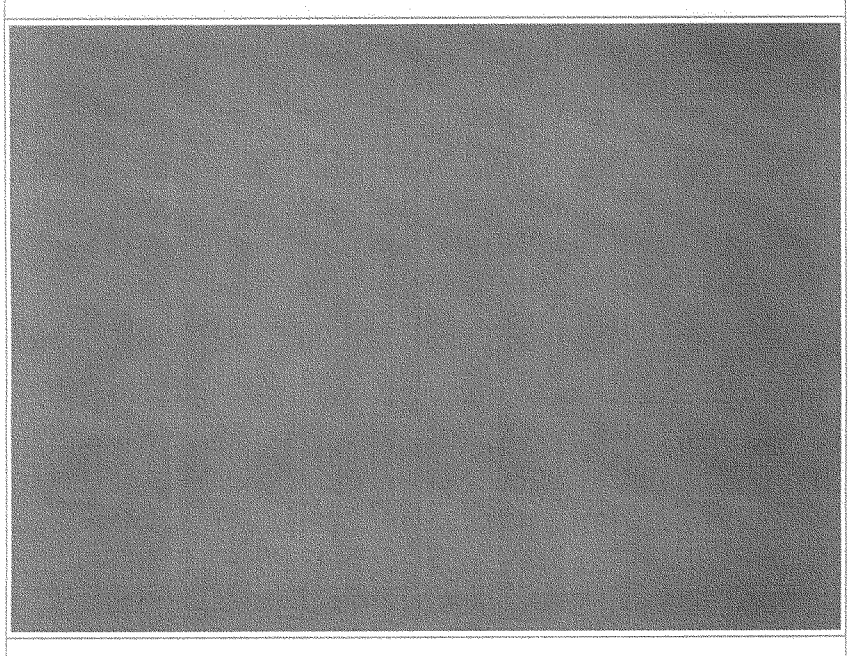

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October 26, 2011

Jerry Menikoff, M.D., J.D.
Office for Human Research Protections
Department of Health and Human Services
1101 Wootton Parkway
Suite 200
Rockville, MD 20852

Re: HHS-OPHS-2011-0005 (Advanced Notice of Proposed Rulemaking on Human Subjects Research Protections: Enhancing Protections for Research Subjects and Reducing Burden, Delay, and Ambiguity for Investigators)

Dear Dr. Menikoff:

The Confidentiality Coalition respectfully submits these comments in connection with the Advanced Notice of Proposed Rulemaking related to Human Subjects Research Protections: Enhancing Protections for Research Subjects and Reducing Burden, Delay, and Ambiguity for Investigators, published in the Federal Register on July 26, 2011 (the "ANPRM"). In this response, we (i) provide background on the Confidentiality Coalition; and (ii) offer comments on certain limited aspects of the ANPRM that relate to the privacy and security of patient information.

Background

The Confidentiality Coalition is composed of a broad group of hospitals, medical teaching colleges, health plans, pharmaceutical companies, medical device manufacturers, vendors of electronic health records, biotech firms, employers, health product distributors, pharmacies, pharmacy benefit managers, health information and research organizations, clinical laboratories, patient groups, and others[1] founded to advance effective patient confidentiality protections.

The Coalition's mission is to advocate policies and practices that safeguard the privacy of patients and healthcare consumers while, at the same time, enable the essential flow of patient information that is critical to the timely and effective delivery of healthcare, improvements in quality and safety, and the development of new lifesaving and life-enhancing medical interventions. The Confidentiality Coalition is committed to ensuring that consumers and thought leaders are aware of the privacy protections that are currently in place. And, as healthcare providers make the transition to a nationwide, interoperable system of electronic health information, the Confidentiality Coalition members believe it is essential to replace the

[1] A list of the Confidentiality Coalition members who have signed on to this letter is attached.

current mosaic of sometimes conflicting state healthcare privacy laws, rules, and guidelines with a strong, comprehensive national confidentiality standard for healthcare information.

Comments

- **The Coalition supports the premise of matching HIPAA's protections to the IRB/Human Subject Research Environment.**

Rather than mandate that Institutional Review Boards (IRBs) assess informational privacy risks each time a research project is proposed, the Department through this ANPRM is proposing to standardize privacy and security protections in the research environment, using the HIPAA privacy and security rules as the baseline standard. We wholeheartedly support this approach.

There are two aspects of this approach that are important to recognize. First, the HIPAA Privacy and Security Rules provide significant privacy and security protections to all protected health information. These safeguards – even with the upcoming regulatory changes from the HITECH Act – are well understood in the healthcare industry and have provided substantial protections to all patient information.

Second, there have been concerns throughout the healthcare industry and among our members that some of the interpretations (and misinterpretations) of the HIPAA Rules – including how they have been applied by IRBs and others in the research context – have sometimes created material impediments to effective research. We are aware of repeated instances where a lack of understanding of some of the provisions of the HIPAA rules and the protections they provide have resulted in unnecessary burdens that have not created additional or meaningful new privacy protection. Therefore, we also support the idea of removing the obligation from IRBs to address these informational privacy risks, by applying a common privacy standard across these research projects. We believe this will permit IRBs to focus on the healthcare risks that are the primary focus of their attention and their expertise, while providing meaningful privacy protections to research subjects consistent with other areas of the healthcare industry.

Therefore, we support the intent of the ANPRM – to align the definitions and requirements of HIPAA and the Common Rule, and to impose consistent privacy and security standards. We believe this is a "win-win" approach. Patient privacy and security will be protected in a consistent fashion. IRBs can focus their attention on areas that are more appropriate to their expertise. And researchers and others involved in research projects can follow a consistent approach throughout their activities.

- **We have strong concerns about adding new patient consent requirements.**

While we support the overall approach of the ANPRM, we also have strong concerns with the primary exception to this approach – the effort to impose a new patient consent requirement in

certain situations related to the use and disclosure of de-identified data in connection with research studies.

The ANPRM proposes new requirements for individual consent for the research use of data, including for the use of limited data sets, and even de-identified data – that would go far beyond HIPAA requirements. We do not believe that this step is necessary or appropriate. Instead – contrary to the overall approach taken in the remainder of the ANPRM – this step would provide new impediments to research and a different set of legal rules, in situations where the patient privacy interests are limited at best. In fact, the ANPRM purports to require new patient consent in situations where the HIPAA Rules have deemed the patient privacy concerns to essentially have been eliminated through the de-identification of healthcare data. We see no significant advantage to patients in this situation, and believe that this new requirement will create significant burdens on research projects. In fact, to obtain this consent, the provision may force research entities and others to re-identify patient data simply in order to try to obtain consent – where no such re-identification would have been permitted or appropriate in the normal course of business. Unlike the remainder of the ANPRM, we view this approach as a "lose-lose" situation. Patient privacy interests (a) could actually be harmed by forcing re-identification of patient data and (b) no significant new protection will be provided through a new and burdensome consent requirement. At the same time, this new requirement will create substantial (and perhaps insurmountable) new obligations on research entities, with significant detrimental effects on research projects. We do not believe that this is a step that makes sense in any way.

Accordingly, we believe that extending the overall approach of HIPAA's privacy and security protections to the research environment should be applied consistently.

Conclusion

The Confidentiality Coalition appreciates the Department's efforts to revise the Common Rule standards to make the requirements consistent with HIPAA. We believe that this approach will benefit the public, by improving overall healthcare research, without creating any material privacy or security concerns for patients.

The Confidentiality Coalition appreciates this opportunity to comment on this ANPRM. Please let Tina Grande at tgrande@hlc.org know if there are any comments or questions about the comments in this letter.

Sincerely,

Mary R. Grealy
President, Healthcare Leadership Council
On Behalf of the Confidentiality Coalition

Enclosure

CONFIDENTIALITY COALITION
Advancing Health Care, Safeguarding Trust

Steering Committee Signatories

Aetna
American Hospital Association
America's Health Insurance Plans
Association of Clinical Research Organizations
Blue Cross Blue Shield Association
CVS Caremark
Federation of American Hospitals
Healthcare Leadership Council
Health Dialog
IMS Health
Marshfield Clinic
Mayo Clinic
McKesson Corporation
National Association of Chain Drug Stores
Pharmaceutical Care Management Association
Pharmaceutical Research and Manufacturers of America
Premier healthcare alliance
Surescripts
Texas Health Resources
VHA
Walgreens
WellPoint
Wolters Kluwer Pharma Solutions

General Membership Signatories

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American Academy of Nurse Practitioners
Amerinet
AmerisourceBergen
American Pharmacists Association
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Baylor Health Care System
BlueCross BlueShield of Tennessee
Cardinal Health
CareFusion
Caris Life Sciences
Catalina Health Resources
CIGNA Corporation
Cleveland Clinic
Care Continuum Alliance
College of American Pathologists
Covidien
C.R. Bard
Eli Lilly
Franciscan Missionaries of Our Lady Health System
Fresenius Medical Care
Genentech, Inc.
Genetic Alliance
Health Care Service Corporation
Health Industry Distributors Association
Healthways
Humana, Inc.
Ikaria
Intermountain Healthcare
Johnson & Johnson
Kaiser Permanente
Lahey Clinic
Medical Group Management Association
MedAssets
Medtronic
MemorialCare Health System
Merck
MetLife
National Association of Health Underwriters
National Association of Manufacturers
National Association of Psychiatric Health Systems
National Community Pharmacists Association
NewYork-Presbyterian Hospital
NorthShore University HealthSystem
Novartis
Novo Nordisk
Pfizer
Press Ganey
ResMed
SAS
sanofi-aventis
SCAN Health Plan
SCHOTT
Siemens Corporation
Society for Human Resource Management
State Farm
Theragenics
Vanderbilt University School of Nursing
U.S. Chamber of Commerce
Wal-Mart
Weight Watchers International