Testimony of Bill Murray
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House Committee on Energy and Commerce
Subcommittee on Health
Hearing on:
"21st Century Cures: Modernizing Clinical Trials"
Wednesday, July 9, 2014

Introduction

Chairman Pitts, Ranking Member Pallone, and Subcommittee Members: Thank you for the opportunity to testify before you this morning. My name is Bill Murray, and I am President & CEO of the Medical Device Innovation Consortium. During my 25 years in this industry, I have had the opportunity to lead multibillion-dollar global businesses at Medtronic and Applied Biosystems, as well as two venture capital-backed early-stage companies, ReShape Medical and Envoy Medical. I have also served on the boards of several other companies. While I have been fortunate to learn from a great diversity of experiences through these leadership opportunities, one core aspect has been consistent: All of these innovative businesses were founded on technology developed in the United States. In recent years, however, all of these businesses have faced a more difficult regulatory and reimbursement environment in the U.S., which is challenging our country's position as a global leader in medical device innovation. I applaud the Committee's bipartisan leadership in initiating the 21st Century Cures Call to Action, and its commitment to finding solutions that will ensure that the U.S. healthcare industry is best equipped to maintain global leadership and empowered to deliver the next generation of medical products that will help U.S. patients and the overall healthcare system.

Background on the Medical Device Innovation Consortium

MDIC is a public-private partnership between government agencies, including the NIH, CMS, and FDA; patient advocacy and other nonprofit groups; and industry. MDIC is the only such partnership focused exclusively on the medical device ecosystem. Our mission is to collaborate on advancing "regulatory science," by which I mean the tools, standards, and approaches that regulators and innovators use in the development, assessment, and review of medical devices. MDIC represents a new, collaborative approach to improving the methods used to regulate new medical device innovations. We believe that our focus on improving regulatory science will offer concrete ways to make patient access to new technologies faster, safer, and more cost-effective.

Medical devices play a unique role in healthcare. While medical devices are a small percentage of healthcare spending, they touch many different aspects of patient care. They range from surgical instruments and implantable devices to high-tech molecular diagnostic systems and imaging equipment. Today, the category of medical devices also includes emerging digital technologies and sensors that enable telemedicine and remote healthcare. The pace of new innovations far exceeds all historical precedent. Medical devices not only restore health and extend life by treating many of the most challenging chronic and life-threatening diseases; they also enable new cost-effective ways to deliver healthcare to patients, creating opportunities for improved care at lower cost.

MDIC was formed in late 2012 out of a shared desire on the part of manufacturers and the FDA to address ecosystem-wide challenges facing the U.S. medical device community. Through the vision and leadership of industry leaders and Jeffrey Shuren, director of the FDA's Center for Devices and Radiological Health, we have been successful in fostering this breakthrough model of cooperation. MDIC is designed to create a collaborative environment where industry, government, and nonprofits can share expertise and resources to advance pre-competitive medical device research, benefiting patients by speeding the rate at which important technologies reach the market.

MDIC's Work to Modernize Clinical Trials and Promote Medical Device Innovation

One of the biggest challenges in the medical device ecosystem are clinical trials. The time, complexity, and cost of conducting clinical trials, along with the uncertainty regarding outcomes, makes clinical trial design and execution a challenge for both regulators and innovators. In the past decade, the demand for high-quality clinical data and the standards by which such data are judged have risen: Our community is expected to conduct more rigorous, evidence-based clinical trials, operate with greater transparency, and do more to inform and share decision-making with patients. In many ways, these changes are benefiting both patients and the industry. However, they have also strained our traditional product development and regulatory assessment systems, which are not sustainable in light of the costs and the uncertainty of outcomes. We need new approaches to clinical development if we are to continue fostering a vibrant innovation ecosystem that is efficient, cost-effective, and economically sustainable. MDIC applauds the committee's focus on finding ways to modernize clinical trials. We

must find ways to improve the clinical development process to ensure that the United States retains our global leadership position in medical innovation.

The good news is that, through MDIC, our stakeholders are proactively collaborating on clinical trial innovation and reform. We believe that clinical trial innovation has the potential to improve the safety and effectiveness of products being introduced into the market, reduce clinical trial timelines and costs, and give U.S. patients earlier access to beneficial innovative technologies. MDIC's work currently includes several high-priority initiatives:

First, MDIC is working to improve the design of clinical trials. Medical device clinical trials are increasingly—and often unnecessarily—complicated. The reasons for this are both varied and poorly understood. They may include inefficiencies in infrastructure, such as missed opportunities for multiple studies to share platforms and resources, the frequently long review cycles and inconsistent requirements of local Institutional Review Boards, and poor subject recruitment by some clinical study sites. Many researchers and regulators also believe that we could be handling data more effectively—that we could save time and money by being more thoughtful about how much and what kind of data is collected in clinical trials, how it is organized and stored, and when it is shared across studies and with the FDA. For example, common data standards and the ability to share information between different electronic health record systems might facilitate fruitful sharing of clinical study data. MDIC is examining current trial designs to better understand which aspects of clinical trials may be needlessly complex, and we are exploring possible alternative trial designs that still supply high-quality data on the safety and effectiveness of medical devices. Our work will include a

survey of our member companies on the amount and type of data that they gather in clinical trials, how much of that data is used, and how much it costs to collect. Our near-term goal is to publish a series of case studies where alternative trial designs were used and how they worked, and to create a menu of alternative trial designs that will explain different design types and when they may be appropriate. Future work will include additional research, such as a survey of physician societies and clinical researchers about trial designs.

Second, MDIC is investigating ways to reduce the barriers to conducting early feasibility studies in the United States. Early feasibility studies, which are also called first-in-human studies, mark the first point at which a new treatment is tested on human subjects. These studies are a critical step in the approval process of many new medical devices, but increasingly, they are performed outside the United States. The reasons for this include powerful economic incentives offered by countries other than the United States for companies to invest abroad, but also a pervasive perception that the regulatory approval process is slower and less predictable in the United States than it is in many other countries. As a result, U.S. patients often have to wait longer than patients elsewhere for access to new medical devices. MDIC feels strongly that American patients should be the first to benefit from cutting-edge American technologies.

The FDA recognizes this need and, in response, issued a new policy in 2012 to make it easier for innovators to start early feasibility studies in the U.S., to do so earlier in device development, and to make certain changes to devices and re-study them without having to receive FDA approval. The FDA has also created a medical device clinical trials program with an acting director in the Center for Devices and Radiological Health

to facilitate these and other innovations. The goal is to reduce the time and cost of the clinical trial enterprise, including the early feasibility phase, while assuring adequate patient protections. Some companies are already taking advantage of the new early feasibility clinical trial policy.

To help address the issue of early feasibility studies, MDIC is conducting an industry survey to help identify the specific barriers that discourage companies from performing these studies in the U.S. We are also exploring new methods and tools to support early feasibility studies, such as templates and best-practice guidelines that could help both innovators and regulators by clarifying how the process should work.

Third, MDIC is conducting research to better understand patient preferences, with the goal of integrating these preferences into the development and regulatory approval of medical devices. 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 treatment. It makes sense for innovators and regulators to consider patient perspectives as they develop and assess medical devices. After all, one of the most important questions we ask is whether the clinical benefit of a device outweighs its risk. Patients and their families have a deep and personal understanding of what it is like to live with a disease, and they often have valuable insights on how a device could affect their quality of life. In the end, it is patients who must take the risks of medical interventions to obtain the benefits, so their perspectives on benefit-risk tradeoffs should be central to the benefit-risk assessments that are the basis of regulatory approval.

The FDA has acknowledged the potential value of patient preference information in regulatory benefit-risk determinations. In 2012, the agency's Center for Devices and Radiological Health issued guidance[1] for manufacturers on how it makes benefit-risk determinations during the pre-market review of certain medical devices. Significantly, FDA emphasized that "patient tolerance for risk and perspective on benefit" is an important consideration. However, this important guidance document does not discuss how such information on patient tolerance of risks and valuing of benefits can be collected or presented to the FDA.

One of MDIC's first major efforts will be on how to measure information on patient preferences and incorporate that data into the regulatory assessment of new medical devices. This work is being funded by the FDA and builds upon the findings of a public workshop hosted by the agency last fall. MDIC's Patient-Centered Benefit-Risk (PCBR) Project will have three major deliverables: First, we will develop a catalog of scientifically valid ways to reliably assess patient views on the potential risks and benefits of specific devices. Second, we will develop a framework for thinking about how to incorporate patient preferences into regulatory benefit-risk assessments. Third, we will produce an analysis of gaps in our current ability to collect and use patient preference data, with a research agenda to address those gaps. The PCBR Project team working on these deliverables includes knowledgeable participants from CDRH, industry, patient advocacy groups, and academia. MDIC plans to share our work on patient preferences

[1] "Guidance for Industry and Food and Drug Administration Staff - Factors to Consider When Making Benefit-Risk Determinations in Medical Device Premarket Approvals and De Novo Classifications." FDA. March 28, 2012. http://www.fda.gov/medicaldevices/deviceregulationandguidance/guidancedocuments/ucm267829.htm.

publicly in early 2015, with the goal that CDRH might choose to build off this work in future guidance documents and in regulatory decision-making. We also anticipate that others, including innovators, payers, patient care organizations, advocacy groups, and academics, will find our catalog of methods for obtaining patient preference information, our thinking about how to use patient preference information in benefit-risk assessment, and the agenda that comes out of our gap analysis helpful in their efforts to improve patient outcomes and make healthcare more patient-centered.

Fourth, MDIC is convening experts to help the medical device industry harness the power of computational modeling and simulation. Modeling and simulation have the potential to revolutionize the field, enabling medical device developers to generate more groundbreaking ideas, test them with greater confidence and at lower cost, and bring them to patients more safely and quickly. Moreover, with accelerated use in development and evaluation, it is conceivable that modeling and simulation will play a larger role in treatment planning and fully realizing personalized medicine in the clinic. Currently, though, the medical device industry lags behind such fields as aerospace and automotive engineering in the use of these tools.

MDIC members share a vision of using modeling and simulation to accelerate medical device innovation. We are working to achieve the consistent application of validated computational modeling and simulation in device development and regulation. We aim to use these tools to evaluate new and emerging technologies, and to develop state-of-the-art preclinical methods for assessing device safety and performance. We are studying how the incorporation of virtual patients might inform clinical trial design, making clinical trials more efficient and potentially reducing their size. To achieve these

goals, we are working to define, standardize, and educate the medical device community on validation requirements for the use of modeling and simulation in device development and regulatory submission.

Recommendations to Support Medical Device Innovation

While we are very pleased with the progress MDIC is making on these important initiatives, much more needs to be done. Regulatory science is a nascent field that will benefit from a sustained strategic investment throughout the design, development, regulatory, and reimbursement product lifecycle to ensure that U. S. citizens have timely access to high-quality, safe, and effective American innovations. We encourage Congress to support these efforts to strengthen regulatory science to help improve the environment for medical innovation here in the United States.

There are three key steps Congress could take to support this work and work by other innovative partnerships. First, create grants for public-private partnerships that effectively harness the brainpower of both the public and private sectors to address a public health need. Second, remove barriers to public health agency participation in these types of partnerships. Currently, the Federal Advisory Committee and Paperwork Reduction Acts, together with technology transfer statutes, increase the time and complexity involved in establishing and managing organizations like MDIC. Finally, allow federal, industry, and nonprofit researchers to collaborate freely on work that is supported in part by industry. Hesitation to use federal gift authority means that it is often difficult for our best minds to work together unless the partnership was specifically

established by Congress, as in the Foundation for the NIH, or funded by a federal agency. Without requiring Congressional action, stakeholders and government experts should be liberated to identify a need, come together to address it, and then dissolve the partnership once the public health need has been addressed. Public-private partnerships can be nimble, efficient, and responsive, but only if government, nonprofit, and industry participants are allowed to participate freely, work together closely, and invest wisely. These partnerships are unique and address a unique need. There are no easy questions left in medicine. We need big collaborations to conduct big science, and to rapidly and efficiently improve human health. Partnerships allow each sector—patients, foundations, industry, and the government—to vote with their feet, spending their time, ideas, and resources only on those partnerships that accurately identify the outstanding problems and creatively search for the solutions.

Thank you again for the opportunity to testify and educate the Committee and stakeholders about MDIC's collaborative efforts to advance pre-competitive medical research that will benefit patients.

I will be happy to answer any questions.

Summary of Testimony on 21st Century Cures: Modernizing Clinical Trials
Bill Murray
President & CEO, Medical Device Innovation Consortium (MDIC)
Wednesday, July 9, 2014

MDIC is an unprecedented public-private partnership between government agencies, patient advocacy and other nonprofit groups, and industry. MDIC is the only such partnership focused exclusively on the medical device ecosystem. Our mission is to collaborate on advancing regulatory science. We believe that we can offer concrete ways to make patient access to new technologies faster, safer, and more cost-effective.

In recent years, medical device businesses have faced a more difficult regulatory and reimbursement environment in the United States, which is challenging our country's position as a global leader in medical device innovation. I applaud the Committee's bipartisan leadership in initiating the 21st Century Cures Call to Action.

Clinical trials are among the biggest challenges in the medical device ecosystem. The time, complexity, and cost of conducting clinical trials, along with the uncertainty regarding outcomes, makes clinical trial design and execution a challenge for both regulators and innovators. We need new approaches to clinical development if we are to continue fostering a vibrant innovation ecosystem that is efficient, cost-effective, and economically sustainable.

MDIC's stakeholders are proactively collaborating on clinical trial innovation and reform. We believe that clinical trial innovation has the potential to improve the safety and effectiveness of products being introduced into the market, reduce clinical trial timelines and costs, and give U.S. patients earlier access to beneficial innovative technologies.

Our high-priority initiatives include:

- Improving the design of clinical trials.
- Reducing the barriers to conducting early feasibility studies in the United States.
- Conducting research to better understand patient preferences, with the goal of integrating these preferences into the development and regulatory approval of medical devices.
- Convening experts to help the medical device industry harness the power of computational modeling and simulation in clinical trials.

To support medical device innovation in the United States, we recommend that Congress:

- Support efforts to strengthen regulatory science.
- Create grants for public-private partnerships that effectively harness the brainpower of both the public and private sectors to address a public health need.
- Remove barriers to public health agency participation in these types of partnerships.
- Allow federal, industry, and nonprofit researchers to collaborate freely on work that is supported in part by industry.