UNITED STATES OF AMERICA

DEPARTMENT OF HEALTH AND HUMAN SERVICES

FOOD AND DRUG ADMINISTRATION

+++

CENTER FOR DEVICES AND RADIOLOGICAL HEALTH

MEDICAL DEVICES ADVISORY COMMITTEE

+ + +

PUBLIC MEETING - MEDICAL DEVICE USER FEE AMENDMENTS FOR FISCAL YEARS 2023 THROUGH 2027

+++

April 19, 2022
12:00 p.m.

Via Web Stream

WORKSHOP PARTICIPANTS:

LAUREN ROTH
Associate Commissioner for Policy
FDA

ROBERT CALIFF, M.D.
Commissioner of Food and Drugs
FDA

JEFF SHUREN, M.D., J.D.
Director, Center for Devices and Radiological Health
FDA

PETER MARKS, M.D.
Director, Center for Biologics Evaluation and Research
FDA

MICHELLE TARVER, M.D., Ph.D.
Center for Devices and Radiological Health
Deputy Director, Office of Strategic Partnerships and Technology Innovation
FDA

MELISSA TORRES, M.E., M.S., C.O.A.
Center for Devices and Radiological Health
Associate Director for International Affairs
FDA

FELIPE AGUEL, Ph.D.
Center for Devices and Radiological Health
Deputy Director, Office of Clinical Evaluation and Analysis
Office of Product Evaluation and Quality
FDA

KATHRYN CAPANNA
Center for Devices and Radiological Health
Deputy Director, Division of All Hazards Response, Science, and Strategic Partnerships
Office of Strategic Partnerships Technology Innovation
FDA

BRENDAN O'LEARY
Center for Devices and Radiological Health
Acting Director, Division of Digital Health,
Office of Strategic Partnerships Technology
Innovation
FDA

JANET TRUNZO, M.S.
Advanced Medical Technology Association
(AdvaMed)
Senior Executive Vice President, Technology &
Regulatory Affairs

MARK LEAHEY, J.D., M.B.A.
Medical Device Manufacturers Association (MDMA)
President and Chief Executive Officer

PETER WEEMS
Medical Imaging & Technology Alliance (MITA)
Director of Policy and Strategy

THOMAS SPARKMAN, J.D., M.P.P.
American Clinical Laboratory Association (ACLA)
Senior Vice President for Government Affairs
and Policy

ELIZABETH RICHARDSON, M.Sc.
The Pew Charitable Trusts
Project Director, Health Care Products

S. RAYMOND GOLISH, M.D., Ph.D., M.B.A.
American Academy of Orthopaedic Surgeons

JENNIFER DEXTER
National Health Council
Assistant Vice President, Policy

CYNTHIA BENS
Personalized Medicine Coalition
Senior Vice President, Public Policy

MICHAEL ABRAMS, M.P.H., Ph.D.
Public Citizen

PAUL CONWAY
American Association of Kidney Patients
Chair of Policy and Global Affairs

AMY OHMER
International Children's Advisory Network (iCAN)
Executive Director

DYLAN SIMON, M.Sc.
EveryLife Foundation for Rare Diseases
Director, Policy

PAUL MELMEYER, M.P.P.
Muscular Dystrophy Association
Vice President, Public Policy & Advocacy

INDEX

M E E T I N G

(12:00 p.m.)

MS. ROTH: Good afternoon, here on the east coast. Good morning if you are on the west coast. And welcome to FDA'S Public Meeting to discuss the proposed recommendations for the reauthorization of the Medical Device User Fee Amendments or MDUFA. For fiscal years 2023 through 2027, I'm Lauren Roth FDA'S associate commissioner for policy, and I have the privilege of leading FDA'S negotiations during this fifth MDUFA cycle.

I want to thank you all the speakers whose perspectives we'll hear today and welcome all the participants who've joined the webcast. We look forward to the dialogue and we invite you to submit any additional feedback to us via the public docket for this meeting. The deadline for submitting comments is this Thursday, April 21st, and we look forward to reviewing the feedback that we get from the public.

To get us started on the agenda today, I'm delighted to introduce Dr. Robert Califf. Dr. Califf was confirmed earlier this year as 25th Commissioner of food and drugs. As commissioner, Dr. Califf oversees the full breadth of FDA'S portfolio and

execution of the Federal Food Drug and Cosmetic Act and other applicable laws. This includes ensuring safety, effectiveness, and security of human and veterinary drugs, vaccines, and other biological products for human use and medical devices.

The safety and security of our nation's food apply cosmetics, dietary supplements, and products that give off electronic radiation. As well as the regulation of tobacco products.

Given the breadth of Dr. Califf's work, we're very privileged to have him join us here today. It's his second stent as commissioner. He also served in 2016 at the 22nd Commissioner of FDA and before assuming the position at that time, he had served as FDA'S Deputy Commissioner for medical products and tobacco.

Dr. Califf, thank you for joining us here today. And with that, I will kick it over to you.

DR. CALIFF: Thanks, Lauren, it's really great to be here with all of you. And I want to welcome everyone to today's meeting to discuss a proposed agreement to reauthorize and improve the Medical Device User Fee Program for the next five years.

Your participation in this conversation is essential. And I especially want to thank today's

presenters who reflect a broad spectrum of perspectives from the agency, industry, patients, consumer advocates, physicians, and academics. It's important that we all engage in these different stakeholders as we consider the current reauthorization of the Medical Device User Fee Program.

User fees have played an increasingly important role in supporting the FDA's work to promote and protect public health since Congress first authorized the MDUFA program nearly 20 years ago. User fee resources have enabled FDA to transform and strengthen the predictability and transparency of the device review process. And then provided essential support and so many ways for the work we do.

I was pleased I was able to support the agency's work and reaching the MDUFA IV agreement in 2016, during my first stint at FDA. And recognized the privilege of getting a redo opportunity here, very exciting. That agreement allowed the FDA to continue making progress on reducing review timelines in making important medical devices available to patients more quickly.

It also laid the foundation for several important programs including advancing the use of

high-quality real-world evidence to support regulatory decisions making and I emphasize high quality here because if there's one sort of pet peeve I have it's glomming all of this together and really essential that we work on defining quality for regulatory purposes.

Secondly, supporting patient input and involvement in the regulatory process. And thirdly helping agency develop our capabilities to foster responsible high-quality innovation and digital health technologies. One of the most rapidly advancing areas of med-tech innovation.

Those are just a few of the examples of the areas that thanks to the MDUFA program the FDA continues to lead the way in regulatory science innovations that improve the process for device review and ultimately the lives of patients. It's my great honor to be back at the FDA for the second turn as commissioner to once again support the agency in reauthorizing MDUFA.

The current agreement provides a significant infusion of resources. These resources will help address critical gaps and allow the FDA to strengthen its capacity to assess new medical device technologies and provide a more predictable

transparent path to market. The agreement also looks to the future with new investments to expand FDA's programmatic capabilities in critical areas.

I want to highlight one aspect of the commitments in the agreement concerning patient involvement and how we incorporate their perspectives and experiences into the regulatory process.

One of the areas that's been the focus of my work not just from my previous tenure at FDA but as a clinician and researcher, has been how we strengthen the quality, quantity, and methods of inquiring the evidence necessary to make the best-informed regulatory decisions. And the full participation of patients and consumers is a key piece of this process. Patient input and the development of clinical studies and other forms of evidence generation helps ensure that evidence reflects what matters most to patients and caregivers. And patient access to useful, understandable information about medical devices helps ensure that they have the tools to inform their healthcare decision-making.

I also want to underscore the essential need to include diverse patient populations in the development of medical products including devices.

In order to ensure the best and most effective

treatments for the broadest number and diversity of people. There must be meaningful participation by populations most likely to use the products if approved.

FDA has worked for many years to advance clinical trial enrollment practices that help foster diversity in the evidence generation process. Last week we issued a new draft guidance to industry for developing plans to enroll more participants from underrepresented racial and ethnic populations in the U.S. into clinical trials.

We must continue to focus on this issue and expand those included in clinical trials as far too many groups including racial and ethnic minorities in rural populations are still underrepresented.

I'm pleased that the proposed MDUFA V agreement will support these and other efforts that strengthen the FDA's continued work with industry, patients, and other stakeholders, and improve patient engagement across device development and evaluation process.

MDUFA V will build on the success in a number of ways including supporting first supporting FDA's efforts to use innovative technologies to capture patient input and reduce patient burden, to inform the design and conduct of clinical studies. Second,

holding a public meeting to explore ways to use patient-generated data to help advance remote clinical trial data collection and support clinical outcome assessments. Third, advancing FDA's work to foster the inclusion of diverse patient perspectives in the medical device development and evaluation process. It's a very strong and forward-looking agreement that will allow us to continue the progress we've made. In closing, I want to thank all of you for being here today to provide your perspectives on what the next five years of the MDUFA program should include. We look forward to your feedback.

MS. ROTH: Thank you again, Dr. Califf. And now I would next like to introduce Dr. Jeff Shuren, the Director of FDA Center for Devices and Radiological Health or CDRH at FDA. Dr. Shuren has served at center director since 2009 but has held other policy and planning positions within the agency since 1998. And his involvement in the MDUFA process has lasted nearly as long as his tenure at FDA. Dr. Shuren has been involved in each of the MDUFA reauthorizations beginning with MDUFA II, so to say he brings a reservoir of experience with this undertaking would be an understatement. Dr. Shuren, welcome.

DR. SHUREN: When FDA began the MDUFA

reauthorization process in October 2020, we asked ourselves how the medical device ecosystem should continue to evolve in the future and what FDA and industry could do through the MDUFA program to help make that future a reality. The medical device ecosystem in the United States is characterized by tremendous breadth. FDA regulates more than 230,000 different devices, which are manufactured by more than 18,000 firms in more than 25,000 medical device facilities worldwide.

The ecosystem is also characterized by tremendous innovation. Progress in science and technology offers extraordinary opportunities to develop innovative medical products that can save lives, lead to better treatments, better diagnostics, and better care for patients, but only if they are safe and effective. In considering this landscape, FDA established three overarching goals for the MDUFA V reauthorization.

First, we sought to continue enhancing the operational success of the program, reducing device development times and further accelerating patient access to high-quality, innovative, safe, and effective devices.

Second, we sought to optimize the agency's

infrastructure, staffing, and resources to keep pace with scientific development, including addressing funding shortfalls during the MDUFA IV due to higher than anticipated workloads. And third, we sought to improve device safety across the total product life cycle. I'm pleased that we have reached a proposed agreement that will help us succeed in all three goals.

Notably, the agreement reflects a commitment to the foundation of the MDUFA program by infusing more resources and people to support review of device pre-market submissions. And during MDUFA IV, FDA's workload reached some of its highest levels in key areas. In particular, we saw tremendous growth in the pre-submission review program and the breakthrough devices program. And these increases show no signs of plateauing in the foreseeable future.

MDUFA V will provide critical resources to ensure the operational success of the program as we handle this rising workload. Not only that the agreement includes an innovative new accountability mechanism for add-on payments, unique to MDUFA V. If FDA meets specified performance goals for certain types of pre-market submissions, in fiscal years 2023

to 2025, we'll apply additional user fees in fiscal years, 2025 to 2027 to support improvements in those goals. This will enable us to review an even larger number of pre-market submissions within the predictable review timeline specified in the commitment letter or to further expedite review.

The agreement also reflects critical investments in the future of the program to assure that FDA has the scientific and technical expertise we need to handle oversight review of the robust pipeline of new technology. MDUFA V includes investments to strengthen FDA's expertise, to evaluate real-world evidence, digital health technologies, and patient-generated health data as part of the device review process.

In addition, the agreement reflects a commitment to process and programmatic enhancements that are designed to lead to more timely patient access to new devices, including devices expect to be safer than currently available treatments and diagnostics while continuing to uphold FDA standards.

In particular, MDUFA V pilots an innovative program, the Total Product Lifecycle Advisory Program Pilot, or the TAP pilot for short, to provide earlier more frequent, and more strategic engagement with

sponsors of products designated under the breakthrough devices program or included in the safer technologies program.

The TAP Pilot will build upon lessons learned, not only from these programs but also our experience engaging with sponsors through the pre-EUA process as part of our COVID-19 pandemic response efforts.

The pilot is designed to help innovators avoid pitfalls in early product development to better ensure a clear predictable path to market and to continue to foster the innovation pipeline. FDA will implement the pilot during MDUFA V and provide a public report on progress no later than January 2026. Importantly, the MDUFA V agreement also reflects enhancements to programs designed to improve the overall efficiency and predictability of the device review process.

These enhancements include for the first time, user fee resources to support FDA's work, to improve the efficiency of global regulatory systems for medical devices through international harmonization and convergence of regulatory requirements.

They also include continuation of the accreditation scheme for conformity assessment pilot, which was launched under MDUFA IV to encourage

medical device sponsors to use FDA recognized voluntary consensus standards in their product submissions, as a means of both reducing regulatory burden and fostering product quality. Under MDUFA V, FDA aims to complete the pilot and transition to a permanent program.

With these as well as other enhancements, the proposed MDUFA V agreement will help sustain and bolster the success of device review program in the years to come. FDA has demonstrated time and time again, that we do our best to meet and even exceed our commitments. And the fact that there are more safe and effective medical devices on the market, more options for patients than at any other time in US history, is a testament to these ongoing efforts.

Although, FDA will continue to face significant challenges due to COVID-19 pandemic's continued impact on our day-to-day work. The MDUFA V agreement will be instrumental in getting the program fully back on track, allowing patients to continue to benefit from the robust innovation pipeline for medical devices in the United States. I want to echo Dr. Califf's thank you for your participation in the meeting today. And I want to thank my center colleagues for their efforts in organizing this

meeting. We look forward to the dialogue. Thank you.

MS. ROTH: Thank you, Dr. Shuren. Next, I'm pleased to introduce Dr. Peter Marks the Center Director for FDA Center for Biologics Evaluation and Research or CEBR. This is the center responsible for ensuring the safety and effectiveness of biological products including vaccines, allergenic products, blood, and blood products and cellular tissue, and gene therapies. Including devices.

Dr. Marks joins the FDA in 2012 as Deputy Center Director for CEBR and he became center director in 2016. Dr. Marks is Board certified in internal medicine hematology and medical oncology as well as being a fellow in the American College of Physicians we're really pleased to have you here today, Dr. Marks. And I'm going to turn it over to you.

DR. MARKS: Thanks very much. So I also want to echo Dr. Califf and Dr. Shuren's thanks for your participation in the MDUFA reauthorization process. And I especially thank the presenters on today's agenda who reflect a spectrum of perspectives from the agency, industry, patients, consumer advocates, physicians, and academics. My remarks otherwise will

be brief.

CEBR regulates only a small percentage of devices including those involved in screening of the blood supply, certain in infectious disease diagnostic tests, and those involved in the preparation of blood components or tissue products. However, those devices have significant importance for public health, and we take our responsibilities seriously for helping to facilitate their development and appropriate regulation.

During MDUFA IV we aimed to achieve and remain an excellent alignment with CDRH in executing new policies and achieving the necessary commitments and we look forward to doing the same as we move into MDUFA V.

So as with CDRH, we'll do our absolute best to meet and exceed the commitments outlined in the agreement and that will most benefit the medical device ecosystem and the American public. So thank you once again for taking the time out of your day to participate in this session.

MS. ROTH: Thank you, Dr. Marks. And so with that, we are really again very pleased to have had our colleagues Dr. Califf, Dr. Shuren, and Dr. Marks join us here today. And what we would like to do

next is turn to the rest of the agenda for today.

We were going to kick things off with FDA colleagues from CDRH. Can I ask the studio to show the slides? I think they are being shown at this point. All right. We're going, to begin with remarks from CDRH colleagues and I am very pleased that I'll be joined on this initial discussion by Dr. Michelle Tarver, CDRH'S Deputy Director for the Office of Strategic Partnerships and Technology Innovation.

I'm also joined by Melissa Torres the Associate Director for international affairs, Dr. Felipe Aguel, Deputy Director for the Office of Clinical Evaluation and Analysis, and the Office of Product Quality Evaluation and Quality. Katie Capanna the Deputy Director for the division of all hazards, response, science, and strategic partnerships from the Office of Strategic Partnerships and Technology Innovation. And finally Brendan O'Leary the acting director for the Division of Digital Health.

We can go to the next slide, please. Following this presentation we'll be joined by our colleagues from industry to provide their perspectives on the MDUFA reauthorization, we'll have a short break, and then we'll be joined by our colleagues and advocates

from the Patient Consumer Scientific Academic and Health Professional Communities to provide their perspectives on the agreement.

We'll conclude with an open public comment period, and we appreciate those members of public who registered to provide comments during that period.

Finally, a few closing remarks as well as during that period an opportunity for questions that -- for the FDA panel that we will be collecting during the course of the meeting. Next slide. So without further ado, the MDUFA V reauthorization -- next slide, please. Each cycle of MDUFA has built on successes and we've learned from the challenges revealed by the previous cycles as was mentioned at the outset, this agreement represents the fifth MDUFA reauthorization. And as you can see from the graphic here, each one of the agreements has expanded and has enhanced, and really stood on the foundation of the prior MDUFA agreements.

And there's really a lot for the FDA and for industry to be proud of in terms of what we have been able to achieve as part of these MDUFA agreements. And to some extent, this achievement is reflected in the independent assessments that are commissioned as part of the MDUFA agreements. And so during MDUFA

IV, we commissioned an independent assessment, it was handled by Booz Allen Hamilton. And I just wanted to call out one the findings from the agreement, which you can see here, overall "CDRH'S actions have positively impacted efforts to enhance and improve medical device submission review." And that's what we hope to continue and to build upon in the MDUFA V reauthorization. Next slide.

Seeing that the MDUFA V agreement included the following high-level categories. Of course, review performance, the MDUFA V agreement introduced a new goal structure with opportunities for add-on payments as Dr. Shuren mentioned. As well as improving goals for PMA total time to decision, 510(k) time to decision, pre-submissions, and De Novo submissions.

Program improvements, as Dr. Shuren mentioned, we will be launching a TPLC Advisory Program Pilot during MDUFA V as well as enhancing programs that support patient, science, and engagement. Real-world evidence, consensus standards, digital health, and international harmonization.

Hiring and retention. The MDUFA V agreement includes a keen focus on the agency's hiring and retention practices. In particular, it provides needed resources to help support the agency's efforts

to bring on new staff to help administer the MDUFA program. It also includes goals to enhance hiring and the retention of world-class scientific and technical expertise.

Performance accountability is also a strong focus of the MDUFA five agreement. And both FDA and industry shared a goal as part of these MDUFA V -- as part of the MDUFA V reauthorization to ensure that we have a high-quality program that was managed by quality and focus on the performance of the program itself. And part of the way in which we do that is we have regular audits that are undertaken by CDRH'S quality management team as well as I mentioned, independent assessments by an outside auditor.

Finally, financial transparency. The MDUFA V agreement included new accountability mechanisms around financial transparency for the program including enhanced reporting. We're going to go through each of these categories in a bit more detail and as I mentioned I'm joined by my colleagues from CDRH to talk about some of the areas in which the staff from the center will be -- they have their subject matter expertise, and they will be responsible for implementation of some of the enhancements under MDUFA V. Next slide.

So starting with review performance, as I mentioned MDUFA V really builds on the foundation that was established through hard work of industry and FDA in the prior MDUFA agreements. So in many respects, the review performance goals were already optimized coming into MDUFA V.

However, there were a few areas that needed additional focus as part of the negotiations given either rising center workload or other outside impacts such as the impact of the COVID-19 pandemic on the agency's workload.

As I mentioned the first one of those categories pre-submissions. The pre-submission program as most of you may know, provides an opportunity for sponsors to engage with FDA before they submit a marketing submission, whether it's 510(k), De Novo, or PMA. They can come to the agency with questions and in those questions are channeled through by and large the pre-submission program.

During MDUFA IV we saw a significant rise interest by the industry in participating in the pre-submission program. And so it is an important going into MDUFA V that the program be adequately resourced to handle the rising volume in this workload.

It was also important to improve the

performance goals. The pre-submission program was really in its early stages as the agency and industry were negotiating MDUFA IV, the last MDUFA agreement.

But now with five additional years of that program under our belts, we can see the benefit of trying to bring the pre-submission goals more in line with other performance goals in the agreement and having a percent-based structure for those goals. And so we worked with industry colleagues to develop a goal structure that would help address the rising volume of pre-submissions as well as to bring goal structure more in line with other parts of the MDUFA agreement.

In addition, another focus of the agreement was on the total time to decision goals. Total time to decision is a shared outcome goal between FDA and industry and there are two. One relates to 510(k)'s and one to PMA'S. Industry and FDA worked very closely and very hard to ensure that we were optimizing the 510(k)and the PMA total time to decision goals.

In addition, we talked about the need and the opportunity to optimize the De Novo decision goal. This is one of FDA's review performance goals. In all three areas as we looked at the MDUFA agreement,

in addition to pre-submissions, we saw opportunity for improvement.

And so what we agreed to do, as you all will see and as you can see in the agreement, is to both maintain and improve upon this goal structure. And we did that through a mechanism -- a novel mechanism, in the agreement that provides guaranteed funding to the agency. As well as the opportunity for add-on payments if in the early years of MDUFA were successful in meeting the goals. And so we have this structure which provides stability for the program, as well as accountability for meeting the goals through a structure that provides for add-on payments for 510(k), shared outcome total time to decision, PMA shared outcome total time to decision, the De Novo decision day goal as well as for the pre-submissions goal.

Finally, FDA and industry worked together to establish a new performance goal related to communicating clearly in FDA deficiency letters. What we've agreed to is to establish an escalating role whereby a percent of the deficiency and additional information letters will include a statement for the basis of the deficiency during the course of MDUFA V. Consistent with our guidance.

Next slide, please.

In terms of program improvements, Dr. Shuren gave an overview of the TAP Pilot, but I'll spend a minute here, and then I will also turn it over to some of my colleagues to talk about other aspects of the program improvements that are included in the MDUFA draft commitment letter.

So what is the TAP Pilot? As Dr. Shuren mentioned, this is an opportunity for FDA to launch a voluntary pilot program in order to provide an opportunity for earlier and more frequent interaction between sponsors of breakthrough and STeP designated products and the FDA. The idea here is to facilitate coordination of earlier and more strategic input from the agency as well as from other stakeholders. It will focus on breakthrough and STeP as I mentioned and the concept for this pilot is to employ lessons learned from the existing breakthrough program. As well as from the agency's experience with COVID-19 submissions in the pre-emergency use authorization process that we had in order to help expedite, review of COVID-19 related products.

FDA and industry are going to explore how this program works during MDUFA V, assess the impact of the pilot on these products, and look for where there

may be opportunities for further improvement as we move into MDUFA VI. Next slide, please.

Patient science and engagement also an incredibly important focus of the agreement as Dr. Califf mentioned and for that, I'd like to turn it over to my colleague Dr. Michelle Tarver.

DR. TARVER: Thank you very much, Lauren. As you heard from the opening remarks, patients are truly the bedrock of the work done at FDA. Our commitment to foster patient science and engagement at CDRH began during those 2010 townhalls where our Director Dr. Jeff Shuren traveled the nation speaking to and hearing from patients as well as other stakeholders. It was solidified in our 2012 vision statement where patients as the first word because they are our most important customers.

It was further actualized in the 2016-2017 strategic priority, entitled Partnering with Patients. Where we began cultivating a culture of engagement and proactively incorporating patient perspectives in our regulatory activities. This early work laid the foundation for the formal establishment of the Patient Science and Engagement Program under MDUFA IV

As we have witnessed over the years we've

accomplished much and grown the program. Under the proposed MDUFA V agreement we will expand and mature work on patient science and engagement.

Specifically, CDRH will facilitate patient engagement for the generation of patient-friendly educational content. By ensuring that patients understand the considerations and medical device development, evaluation, and surveillance we can better empower them to be active agents in regulatory process. We will also transparently communicate our recommendations to industry and other stakeholders on approaches to generate robust, well-defined measures of the patient experience through guidance.

Specifically, we will work collaboratively to issue draft guidance on incorporating clinical outcome assessments into premarket studies as well as updating patient preference information guidance. Building on the theme of clear recommendations and training, we will train and hire additional experts to review patient experience data. This includes patient-reported outcomes, patient preference information, and patient-generated health data. Over the years we have seen that sharing concrete examples and lessons learned with the public can help improve the regulatory predictability and impact of patient

science. Whether it's examples of tools to collect patient experiences, or studies reflecting diverse patient preferences, we will continue to share examples where possible.

Lastly, our country has increasingly become aware that health inequities exist in various population and that the medical products used to diagnosed and treat health conditions are not always developed or evaluated in a manner that includes diverse patients or their experiences. CDRH'S 2022-2025, strategy priorities reflect our commitment to advance health equity as does our proposed MDUFA V agreement.

We will further explore ways to advance health equity by incorporating data and perspectives from diverse patients. One exciting opportunity where we are seeing an explosion of innovation is with patient-generated health data. This data which can be collected by wearables, consumer products like smartphones, and other sensors has the potential to provide texture and richness to the understanding of the patient experience living with their condition and the impact of interventions on their day-to-day lives.

We plan to hold a public meeting on patient-

generated health data for collecting clinical outcome assessments as well as for remote clinical trials. We see this technology as a bridge to inclusivity promoting diversity and equity in clinical research and clinical care.

The MDUFA V agreement as proposed will allow CDRH (indiscernible 00:38:33) to make strides in ensuring patients, all patients, have measures that reflect their experiences with medical devices. And with that, I would like to turn it to Melissa Torres to talk about international harmonization. Next slide.

MS. TORRES: Thanks, Michelle. So we are certainly pleased that we are able to negotiating goals surrounding international harmonization efforts emphasizing the importance of harmonization and convergence of regulatory requirements. Under the proposed MDUFA agreement we plan to advance international harmonization efforts by committing to several goals.

First, we commit to expand our engagement in international harmonization and convergence efforts through participation with international regulators and other key stakeholders in various forms, working groups, projects, and committees to be able to

promote alignment with international best practices.

Some of these examples of efforts include IMDRF which we're currently engaged in, but with additional resources, we would be able to expand engagement in other initiatives, such as global harmonization working project, WHO, APAC, and other regional harmonization initiatives and various international projects.

Our second goal is to be able to support regulatory convergence by creating a mechanism of which FDA will be able to work with our regulatory partners to be able to inform and align our international regulatory strategies.

For example, this can include sharing of our practices, policies, related to scientific clinical or other technical types of information. Another goal is to be able to support the creation of a forum to engage relevant stakeholders including industry and other regulators to look at ways and opportunities for us to be able to leverage regulatory approaches which became very important during the COVID-19 pandemic.

We also hope to be able to increase our participation outreach activities to be able to encourage other regulatory authority to rely either

in whole or in part on our FDA marketing authorizations.

And finally, to be able to enhance the clarity and transparency of our international harmonization activities we plan to issue a draft strategic plan in fiscal year 2023 and to be able to then publish annual assessments of our activities to ensure that we are really enhancing clarity and transparency of our goals and efforts to surrounding regulatory harmonization and convergence efforts.

In addition to a show additional accountability we'll also explain the extent of our FDA implementation of our IMDRF draft technical documents. Now, I'd like to turn it over to Dr. Felipe Aguel to cover real-world evidence goals. Thank you.

DR. AGUEL: Thank you, Melissa. User fee revenue will be used by FDA for the continued development of methods and policies to advance regulatory acceptance of real-world evidence in premarket submissions. This includes premarket submissions seeking expanded indications for use. As well as clear and sort of approval of new devices. To this end, FDA has committed to update the 2017 Real World Evidence Guidance document to provide more

clarity and what's needed to demonstrate a real-world dataset is fit for purpose for premarket regulatory acceptance. Provide examples of previously used and accepted methodologies and provide best practices for review of real-world evidence.

FDA will also continue to advance its training program for review staff, including medical reviewers. FDA will provide transparent program development updates and transparent financial accounting of user fee revenue, used for this purpose. This includes updating stakeholders at two or more public meetings during the course of MDUFA V and tracking the reporting on experts hired to support review of real-world evidence and submissions.

Finally, user fee funds could be used to continue to support the National Evaluation System for Health Technology or NEST. If FDA chooses to do so, the letter stipulates how the funding should be you'd by NEST. FDA will also solicit public input on how the funding would be used if FDA were to choose to fund other external organizations to advance the use and acceptance of real-world evidence in premarket submissions. Thank you. With that, I'll turn it over to Katie Capanna.

MS. CAPANNA: Thank you, Felipe. And good afternoon, everyone. Thank you for joining us. I'm presenting on behalf of our standards program here at FDA. And before I walk through the commitments, I'll provide just a little bit of background to help you understand the important role that consensus standards play in FDA's review. And also in helping to assure the safety and effectiveness of medical devices around the world.

So standards are essentially test methods or guidelines that are developed through consensus-based process run by national or international organizations. And they cover fundamental aspects such as basic safety or essential performance, for example, biocompatibility, or standard requirements for medical, electrical equipment.

And there are currently over 1,000 consensus standards that are officially recognized by the FDA which essentially means that when manufacturers follow the standard correctly, they meet FDA's requirements in that area. And during the COVID response, FDA relied heavily on international standards.

As the nation was dealing with widespread shortages of essential devices such as covid

diagnostic tests and supplies, respirators, other types of PPE, and other devices. FDA was using every tool we could to increase the availability of appropriate alternatives in the United States.

And by leveraging international standards we were able to much more rapidly assess alternative manufacturers for potential Emergency Use Authorization which is essential for admitted gating the effects of the shortages.

The MDUFA V commitment centered around voluntarily ASCA Program or Accreditation Scheme for Conformity Assessments. The goal of this program is to significantly streamline the reviews for submissions that involve ASCA standards to help ensure timely access for patients to safe, effective, and high-quality medical devices.

Programs does this by raising confidence among manufacturers and amongst the FDA reviewers and the testing that's completed by ASCA accredited testing laboratories. During the pilot we conducted under MDUFA IV we developed strong relationships with the test labs and the accreditation bodies, and at the same time, the stakeholders are upping their game which will have positive ripple effects for safety and device quality.

Based on early successes and industry's work -- industry's interest in this work under MDUFA V we proposed to take the lessons learned from the ASCA pilot to transition to a sustainable expanded program by FY24 and report on the performance of the pilot.

We'll continue to work with stakeholders to inform how we will enhance and expand the program. We will continue to train testing laboratories as well as FDA reviewers to execute the process as designed and intended. We will incorporate existing international conformity standards and practices where appropriate. We will continue to report publicly on the progress of the program. And with that, I'll thank you for your attention and turn it to Brendan O'Leary.

MR. O'LEARY: Thanks, Kate. I'm excited to speak with you a little bit today about the progress we'll be building on for MDUFA IV in our digital health program.

Under our MDUFA IV commitments, we established the Division of Digital Health as centralized unit of digital health experts who can support digital health product reviews across agency and particularly across medical devices. We built on that in 2020 with launch of the Digital Health Center of Excellence.

In addition, we streamlined and aligned FDA review processes with software lifecycles for digital health products and explored new regulatory frameworks that are better suited to the rapid innovation cycle in this space. And we participated in international harmonization efforts through the International Medical Device Regulators Forum and other multi-lateral efforts, such as issuance of machine learning practices and principles.

Under MDUFA V we're going to continue to build on all of this progress. And in addition, we're going to finalize device software functions guidance and publish new draft guidance on modification control plans, particularly for artificial intelligence and machine learning medical devices. We're excited to continue to build on this progress in MDUFA V. And with that, I'll turn it over to Lauren Roth.

MS. ROTH: Thank you, Brendan. And thank you, everyone. If we can go to the next slide, please. As I mentioned at the outset the MDUFA agreement includes a focus on hiring and retention. In particular, it funds strategic hiring and retention pay.

Enhancements to the medical device review

program require that FDA recruit and retain highly qualified and diverse workforce. And we're really pleased that our industry partners are recognized the need for FDA to increase its hiring efforts as part of MDUFA V through additional funding for this expertise.

Moreover one of the things we faced coming into MDUFA V negotiations was a challenge related to our payroll costs. What we saw during MDUFA IV was that rising payroll costs in CDRH and in CEBR were contributing to a shortfall in the center's ability to fund the existing staff ceiling we had in the program.

And so we're very pleased that MDUFA V incorporates funding mechanisms to help make up for that shortfall as well as to ensure the stability of the program for the MDUFA V cycle. Related to that it was really important both to FDA and to industry that the program established annual hiring targets as an accountability mechanism to ensure FDA was keeping up with its -- with the expectations that had been built into the MDUFA agreement around hiring for the program.

So what you'll see in the agreement is that there are annual hiring targets, minimum annual

hiring targets, built into the agreement. As well as an agreement by FDA's part that if those targets are missed, the FDA would provide an offset to registration fees in a future year. Essentially ensuring that either the money is used to help support hiring in the program or it's used to provide an offset in a form of registration fee offsets in a future year. Next slide.

There was also a focus on performance accountability and that came through in two ways. As I mentioned at the outset, first, continuing to resource CDRH'S Quality Management and Organizational Excellence Program. This is a program within CDRH that helps facilitate process improvements through audits of CDRH processes and through the sharing of best practices that are learned from those audits.

In addition, we will have not one but two independent assessments as part of MDUFA V. The first assessment will look at the methodologies as well as the data and other metrics available to represent the MDUFA workforce. This is tied in with the hiring goals that I mentioned earlier. It's very important to industry as well as to the FDA to have a good 360 view of the MDUFA workforce and right now we

were unable to answer some of the questions that industry and others have had about the number of people in the center who do MDUFA related work. Because of the historic wait agency has tracked MDUFA effort is through a calculation of a full-time equivalent and not individual staff members.

So as part of MDUFA V, we'll be doing an independent assessment to look at the methodologies and metrics available to assess the MDUFA workforce including an assessment of positions that are filled or vacant, MDUFA process, FTEs or full-time equivalents, and a determination of the staff and the FTEs that are funded by user fees and budget appropriations.

In addition, we will continue to do the programmatic assessment that we've had in years past and this year -- in this cycle, it will be a targeted assessment for the process of review device applications. Next slide.

This sort of collects all in one place the various forms of financial transparency that are included in the MDUFA V agreement. There is of course the financial framework itself with the guaranteed base funding as well as the opportunity for the add-on fees to improve review performance

goals if the initial goals are met.

As part of financial transparency, the agency will annually -- will publish a five-year financial plan and it will update that plan on an annual basis. That plan will include additional information in addition to what is already provided as part of annual financial reports that are posted publicly on our website.

In particular, what the financial plan will include among other things is more information about the payroll costs for our staff and FTE. Related to the carryover balance for those who aren't familiar with the carryover balance, essentially this is the fund that is available if user fees are not spent in any given year. They are stored in a fund called the carryover balance fund and available for future years' use by the program.

There's right now a substantial amount of investment in the carryover balance, and through the negotiations, FDA and industry have agreed to utilize 118 million of those funds in support of MDUFA v programs.

In particular, the 118 million will help support during MDUFA V the launch of the TAP pilot as well as continuation of the Third-Party Review

Program from MDUFA four.

We've also agreed to establish a ceiling on the carryover balance. So bringing the MDUFA program in mind with other user fee programs like PDUFA, MDUFA, and PSUFA.

The ceiling we've proposed will be 13 weeks after which point if the carryover balance were to grow beyond the 13-week ceiling those funds would be returned to industry through offsets to registration fees. Hiring goals as I mentioned, the MDUFA agreement includes annual hiring goals as well as hiring fee adjustment and finally the independent assessment of workforce metrics. Next slide. This slide provides an overview of the financial framework for the MDUFA agreement.

As I mentioned there's the guaranteed funding as well as the potential for add-on payment funding. The guaranteed fund under MDUFA V across the five years totals approximately 1.784 billion. Then there's an opportunity for up to 116 million in add-on payment funding if goals are met, bringing the maximum potential funding during the five years of MDUFA to 1.9 billion. That will enable the program to bring on between 273 and 387 new hires at a minimum. And of course, that range reflects the fact

that we may be able to bring on more individuals if we are able to secure the additional add-on payment funding.

And also helps provide, as I mentioned, for operating costs to support the hiring and retention. As well as operating costs associated with some of the programmatic improvements that will be undertaken as part of MDUFA V.

Finally, additional spending from the carryover balance of 118 million as I mentioned will support new hires for the TAP Pilot as well as continuation of the Third-Party Review Program. Next slide. This shows an overview of anticipated MDUFA V fees. As you all may know, the fees are keyed off the fee for premarket applications so that's what we have listed here.

So the proposed fee for premarket applications across each year of MDUFA V as well as the proposed registration fee for each year of MDUFA V. These amounts do not reflect the adjustments for inflation or due to the potential operation of performance improvement, hiring, and operating reserve provisions, but they give you a sense of what the base fees will be going into MDUFA V. Next slide.

So here we are with the MDUFA V reauthorization

timeline to give you a sense of where we are in the process. We are in the midst of the 30-day public comment period on the rule. So looking to the right of this screen, we are in the phase now for public review of the draft agreement and finalization of the recommendations to Congress.

We, as I mentioned, the timeline for submitting comments the docket closes on Thursday, April 21st, this coming Thursday. We really look forward to feedback from the public. And hope that you will take an opportunity to submit comments. And we of course are here today for the public meeting, April 19th, and we will be considering comments that are received during the meeting today as well as to the public docket as we work to finalize the recommendations to Congress with our industry colleagues.

Now, I'm happy to report that we appear to be running just a bit ahead of schedule, so if we can go to the next slide, please. I want to take a few minutes to talk about stakeholder consultation during the reauthorization process and then we will turn the discussion over to our colleagues from the industry team who help negotiate the MDUFA V draft commitment letter.

As I mentioned and as you heard from our initial public speakers, it really is essential to FDA and to this process that we have multiple opportunities to take feedback from the public as we set out in this process, as we engage in the journey, and now as we come to conclusion and finalization of the recommendations to Congress.

And so we've done over the course of the MDUFA V negotiations is we started out with a public meeting on October 27th, we had the comments that were submitted to the docket as part of the 30-day public comment period and helped inform the perspective that FDA brought to the table as well as we shared that information with our industry colleagues as well.

We had monthly consultation meetings with stakeholders over the course of the past 12 months from March of 2021 through last month while the negotiations were ongoing. There's of course the current 30-day comment period and today's public meeting.

If we go to the next slide, I wanted to give folks a sense of what those consultation meetings entailed of course we had much to discuss over the past 12 months. We started with the kickoff meeting

and a survey of topics of interest to the stakeholders who participated in those consultation meetings. We talked about device safety, innovation, and engagement.

We had stakeholders present on their topics of interest during the June 9th meeting. We talked about innovation in a way that FDA does business, of course at each one of these meetings we provided updates on the negotiations and solicited feedback from our stakeholders. But in some of the meetings such as the August meeting during the hot summer months, we provided a negotiation update only at that meeting.

We also talked about patient diversity, inclusion, and health equity, the results of the MDUFA independent assessment, MDUFA workload, highlights from the CDRH annual report. And of course, the MDUFA draft recommendations. And I just want to take a moment to thank all of the stakeholders, some of whom you'll hear from today, who participated in that process. I really want to express my appreciation for all of the valuable insights and input that you provided throughout the process to help shape our thinking as we engaged in these negotiations.

And really to think about what the next five years of the medical device process could and should look like. So really an expression of gratitude to all of you for sticking with us over the course of the past 12 months to continue to provide your very valuable perspectives and feedback. So with that, if we can go to the next slide, please.

I'd like to turn it over to my industry colleagues to provide their perspectives on the MDUFA reauthorization. We're joined today by Janet Trunzo, Senior Executive Vice President for Technology and Regulatory Affairs at the Advanced Medical Technology Association.

Mark Leahey President and Chief Executive Officer of the Medical Device Manufacturers Association, MDMA. Peter Weems, the Director of Policy and Strategy at the Medical Imaging and Technology Alliance, MIDA. And Thomas Sparkman Senior Vice President for Government Affairs and Policy at the American Clinical Laboratory Association.

If you can all unmute your lines now, that would be great. And I want to say I know again a note of appreciation for all that you all did throughout the past 15 months as we prepared for the

public meeting, had the public meeting, and engaged in these negotiations. So with that, why don't I turn it over to you all and thank you.

MS. TRUNZO: Thank you, Lauren, and thank you, FDA for the opportunity here to express the views of AdvaMed on the Medical Device User Fee Agreement. I also would like to thank all the FDA colleagues and my industry colleagues for all the tireless work in reaching the MDUFA agreement that was described here today.

AdvaMed believes because of the collective efforts of industry and FDA we finalized an agreement that will further strengthen our common goal of patients having timely access to safe and effective medical technology.

From the very first user fee program, from 2002, our common goal for the user fee program has not changed. That common goal of timely patient access to safe and effective medical technologies. While user fees support of the overall timeliness and predictability of the review program, user fees do not guarantee that any submission will be approved. Rather, user fees provide the resources needed to the agency for the review process so that it will be predictable and transparent. FDA always retains the

authority to make a final decision on any submission.

And also, as we pursued the MDUFA V agreement, we've taken the opportunity to try to refine and improve the goals. And you've heard all about the improvements in the review program and other program improvements. We approach this reauthorization with the same overarching goal of benefitting patients and trying to refine and improve the program. We have done that in every user fee program. The first MDUFA contained goals that turned out to be too complex. And those were adjusted in MDUFA two. And MDUFA three we introduced the concept of total time to decision goals, and those goals represent the total elapsed time, which is different than the FDA review day goals that are still part of the program.

In MDUFA IV, we focused among other things, on the pre-submission process in recognition that the quality and timing of an application in the review depends on the ability to obtain feedback from FDA prior to the submission. Each MDUFA cycle included significant resources and investments to support the improvements in the device review program.

We believe that the package today that we heard today is well crafted, the total package including the financial aspects to provide significant

resources and capacity to FDA. We believe there's greater predictability for the industry and, of course, the package is in the best interest of patients.

FDA described in detail today all of the key components of the program, the improvements in the review process, other program improvements, which are very essential to the complete package.

On behalf of AdvaMed, we support the MDUFA V reauthorization package because it will provide the agency resources to deliver not only on the commitments in the MDUFA commitment letter but, more importantly, to continue to ensure that patients have access to safe and effective medical devices. Thank you very much.

MR. LEAHEY: Thanks, Janet. I'll jump in. Again, Mark Leahey with the Medical Device Manufacturers Association. I want to thank FDA for the opportunity to share MDMA's perspective on the draft agreement and like Janet want to echo our thanks to my industry colleagues and to the other stakeholders who had input into the process and also to the folks at FDA.

This was a unique negotiation given COVID, I know some talked about the timing here, but I think

collectively both industry and FDA made the decision to delay the decision by about six months because we are at the forefront of dealing with COVID and so this required an all-hands on deck from both the FDA professionals and within our member companies.

So that delayed the process a bit, but I think when looking back it was the right decision, we all made. We also had the challenges of having a virtual negotiation. As parties know when you're not in the room together, that can sometimes have a lag as well. But as Janet said, we reached the place where we're pleased and support the agreement that has been reached by FDA and by industry.

I think FDA and Lauren did a great job of laying out and the team, the number of the key provisions so I'm not belabor that I'll add a few. But before I do that I do, think it's important to take a moment and thank the extraordinary work that has been done by the FDA from top to bottom from Dr. Shuren all the way down to front line reviewers and everybody in between who put in extraordinary effort as I said, during extraordinary times.

The reviewers were working 24/7 for many, many months and again, I think the extraordinary work ultimately all collectively industry and FDA to

ensure that patients had timely access to safe and effective products is critical and it's also important to reiterate the U.S. is the gold standard when it comes to ensuring products are safe and effective.

I think the sizable investment historic investment over $2 billion of resources that FDA will have I think really validates the commitment that industry has to the professionals at FDA, the commitment to accelerating patient care. And I think it's important to note as Janet said, we pulled our members at the beginning of each group and last few MDUFAs and again have had the pleasure, this is the fifth time I've been involved in the reauthorization of the initial authorization and four reorganizations, the last couple times the quality of the journey was actually much more important than the time of the journey.

Meaning consistency, predictability, transparency, these are the things that our members prioritized, and I think we have learned from each iteration of this program to try to enhance that. And, for example, under MDUFA IV for the first time, there was a goal multiple times referenced in the commitment letter that deficiency letters request for

additional information with cite, with specificity the statute, the guidance, the reg that was the justification for the deficiency to provide some clarity and a rationale for the sponsor to understand why that information is being requested.

And that resulted in a guidance being published in MDUFA IV we've built upon that in MDUFA V and now there's actually performance goals associated with how frequently in an audit that the reviewers are following the guidance.

So again, this goes into the theme of more consistency. Our hope is that more consistent predictable transparent a process, the net output will be in a more efficient review but they're all building upon that. I think a couple of the other areas of importance to note is the De Novo goals.

He's are new goals we put in place under MDUFA IV starting at 50 percent of submissions in the first year MDUFA IV, meeting the MDUFA performance goals, ultimately culminating in 70 percent meeting that goal in FY22.

And again, COVID has had an impact with resources and meeting some goals. But if we're able to hit these targets and make the initial investments which the end of MDUFA V 90 percent of De Novos will

be able -- are supposed to be hitting that goal within the -- by the end of the program. So again, this drives at the point of more consistency the more submissions we have meeting goals, the more predictable it is for patients for innovators, and for regulators.

Also, with the pre-submissions it was noted by Lauren and Dr. Shuren that there's been an increase in pre-submissions throughout the course of MDUFA IV, significant resources were made to build up that capacity to enhance that program. And whether it's pre-submissions or in the regular review, what we heard from member companies is interactive review is critical.

Back in MDUFA two quite frankly that was one of the foundational elements of MDUFA two was more resources for nor interactive review. When we're still as Janet said, learning from each MDUFA and trying to find ways to enhance it and our hope is that given this historic investment, as I said earlier, typically MDUFA cycle will provide funding for an additional approximately 200 new people.

Under this MDUFA V structure, as a minimum this resources for an additional 273 FTEs, and if FDA hits certain performance metrics additional investments

will be made to total 300 -- close to 400 new staff. And again, that's just from the authorized user fees, some carryover funding will be used to help fund the TAP Pilot.

So this is a monumental increase in additional FTEs to build that capacity to allow for better higher quality interaction which again, is something that I think, our members highlighted is of great importance.

Dr. Califf, Dr. Shuren, Dr. Tarver also talked about the significant investments to enhance patient engagement. Again, this is something that's been in place, very important for FDA, very important for the patient community, very important for innovators and we're thrilled again, Dr. Tarver and her team have done an extraordinary job to provide additional resources to build out that capacity. Because we have seen a number of instances where that patient input and either design of the clinical trial, or what the right appropriate endpoints are, helps ensure that technologies and that the evidence generated helps meet the market.

Again, that risk-benefit calculation and analysis that's done oftentimes patients who are in need of these technologies have not a uniform risk-

benefit profile and so understanding those perspectives I think is critical to the review process.

Standards, harmonization, these are all things as we look to have an efficient and effective regulatory process not only in the United States but across the globe that we're leveraging this data, standards in a way that's efficient. Again, while not compromising gold standard but these are all things that I think industry, FDA have worked judiciously on for many, many months throughout the negotiations. And have landed in a place that satisfies the needs first and foremost of the patient for the innovator and for the regulator.

So again, I don't want to the belabor everything said earlier just to reiterate our thanks to our industry colleagues, patient consumer physician groups who had input throughout the way, to the professionals at FDA both on the negotiating team and on the front lines of the reviews, to work through these extraordinary times. I know there's a lot of lessons learned here and I'm sure I will get back in the negotiation table in MDUFA six there will be additional lessons learned. But again, this is a significant investment and last, I'll close by saying

having been involved since day one where I think MDUFA one was a $150 million over the five years and then it got to 300 million, and 595 and 985. I think we're reaching that critical mass here too where I know people will indicate well the MDUFA is a little bit behind PDUFA, and there kind of a more mature program. But again, this type of investment will hopefully get us to a place where foundation is very firm and solid and going forward obviously always be areas for targeted investments.

But that this will really put us on solid footing, capture some of the best practices through COVID, refine the previous MDUFAs, and again really serve American patients and the public well. So thanks very much. And now I think, if I'm looking at the agenda, I will turn it over to Peter Weems at MITA.

MR. WEEMS: Thanks, Mark, and thanks, everyone.

Good afternoon, my name is Peter Weems Senior Director of Policy and Strategy with the Medical Imaging and Technology Alliance also known as MITA. MITA is the Primary Trade Association and Standards Development Organization representing the manufacturers of medical imaging devices and digital health technologies, radio pharmaceuticals, contrast

media, and focused ultrasound devices. Thank you for the opportunity to discuss the MDUFA V agreement reached between industry and FDA.

I would like to start by commending the agency for the work that it has done over the last two years ensuring patients and healthcare providers receive the safe and effective medical products necessary to combat the COVID-19 pandemic.

During the pandemic, medical imaging technologies such as mobile x-ray, chest CT and point of care ultrasound have been available to healthcare facilities serving COVID-19 patients in an expedited manner and with greater regulatory flexibility thanks to actions taken by the FDA.

We look forward to continuing to work with the agency as we transition beyond the COVID-19 pandemic and prepare for the future.

In recent years MITA member companies introduced numerous innovative technologies to the market including low dose CT, high tesla MRIs, ultrasound elastography, and advance AI algorithms. These technologies play an essential role in our nation's healthcare infrastructure and the care pathways of screening, staging, evaluating, managing and effectively treating patients with cancer, heart

disease, neurological degeneration, COVID-19, and numerous other medical conditions.

By testing disease early reducing need for invasive in-patient procedures and facilitating shorter recovery times medical imaging saves lives, reduces costs, and improves the efficiency and healthcare systems. Medical imaging technologies have revolutionized healthcare delivery in America and around the world.

Our member companies' ability to bring innovative technologies safely and effectively to patients and healthcare providers has been supported by the efficient, predictable, and transparent premarket review pathway create by the Medical Device User Fee program. Under the user fee program, medical imaging technology manufacturers have been able to deliver and iterate on innovations for patients in a timely manner.

The next generation of imaging technologies will further advance healthcare in the practice of medicine. A consistent and timely FDA review process is essential to patient access to these technologies.

The goals of the medical device industry and FDA commit to -- and FDA subsequent performance are critical to timely patient access, to safe and

effective medical advancements. Without the user fee program, the FDA review process will not be able to function as intended. And patient access to new medical imaging technologies will be delayed. And industry's ability to deliver technological advancements will be compromised. MITA continues our strong support for effective well-resourced FDA capable of fulfilling its mission to protect and promote public health.

A year and a half of highly technical negotiations resulted in agreement that if enacted will able CDRH, excuse me if enacted will provide CDRH with ample resources, bring new accountability measures to the program, and allow for exploration of new review paradigms through the total product lifecycle advisory program also known as TAP.

The last several years created significant resource challenges for FDA and it seeks to recover its operations and get back on track, it will need to be sufficiently resourced to meet its obligations and continue the review products for safety and effectiveness.

The MDUFA V agreement will raise CDRH'S funding significantly. Allowing the center to meet its premarket review commitments. These new investments

will enable the center to increase head count, expand current programs, and test new review models such as TAP.

Under the MDUFA V agreement, CDRH will be able to hire several hundred new FTEs and meet rising payroll costs. The agency will also continue to invest in successful programs that support a number of shared priorities, including use of standards and real-world evidence in regulatory premarket decisions, advancement of digital health technologies, harmonization of international medical device regulatory activities, and promotion of ongoing U.S. FDA leadership around the world, expanded patient engagement opportunities to inform the development and evaluation of innovative technologies. And continued collaboration with accredited third-party reviewers to support a voluntary alternate review pathway.

FDA will also be able to launch a limited pilot for its TAP program to enhance early communication, collaboration, and engagement between the agency and medical device stakeholders for breakthrough and other eligible devices with review and assessment of milestones along the way. MDUFA V will also bring to bear new accountability measures and their user fee

dollars are appropriately invested in the premarket program.

Review staff are critical component of the center's work so FDA will be expected to meet certain hiring targets. There will also be a new cap on accrual of carryover balances. If the carryover balance exceeds the specified cap, then those excess funds will be used to offset future fees, or otherwise, be invested in a mutually agreed-upon way.

Industry and FDA will also support multiple independent assessments to generate recommendations on how the center can continue to improve its operations. The proposed MDUFA V agreement builds upon what has been achieved over the last 20 years of the user fee program.

It is the right agreement for FDA, industry, but most importantly for patients. We support the FDA in proposing this agreement to Congress. And will continue to partner with the agency and other stakeholders asking Congress to reauthorize this important program that supports patient access to medical imaging innovations. Thank you, and I'll turn it over to Tom Sparkman.

MR. SPARKMAN: Thank you, Peter. I'm Thomas Sparkman with the American Clinical Laboratory

Association where I'm Senior Vice President for Government Affairs and Policy.

I too would like to accident my thanks to Lauren and the FDA team in addition to my industry colleagues for reaching this consensus agreement which we've proposed to Congress.

In addition, I want to thank FDA and the various memberships of the organizations represented on this panel today for the extraordinary effort and continuing response to the pandemic.

Since the first week of March 2020 ACLA members have performed near 200 million PCR tests for COVID-19 in both the form of laboratory-developed tests and also IVD test kits. Particularly the (indiscernible 01:22:32) kits. It's taken extraordinary effort and I can't extend my thanks enough to the folks that are not speaking today whose effort made the response possible. So thank you for that.

ACLA has been the reading voice for solutions that is expand access to the vital clinical laboratory tests millions of patients depend on for health. America's clinical laboratories play a fundamental role in expanding value-based healthcare system by advancing the next generation of precision medicine and care delivery.

Providing accurate and reliable data to informed diagnoses for acute infectious and chronic disease, supporting providers, hospitals, and patients in developing personalized treatment plans. And preventing serious costly complications that burden patients and the health system.

As an advocacy organization, ACLA and its members advocate for reforms that improve patient care by first providing broad access to accurate and reliable clinical laboratory tests.

Second, supporting a clear and appropriate regulatory framework and market pathway for new innovative laboratory diagnostics including laboratory-developed tests and in vitro diagnostics and finally improving care coordination among providers, hospitals, and clinical laboratories.

FDA first invited ACLA to participate in the medical device user fee negotiations in the MDUFA three, cycle. Along with my colleagues here AdvaMed, MDMA, and MITA as representatives of "regulated industry."

ACLA has subsequently participated in MDUFA IV negotiations on these MDUFA V negotiations. As ACLA has stated in these past negotiations and the MDUFA V public meeting earlier, participation in the user fee

negotiations by ACLA and its members is not intended to and does not constitute a waiver of any potential argument or legal relief to which ACLA and/or its members may be entitled with respect to potential regulatory oversight of LDTs or clinical laboratories by FDA.

Participation by ACLA and its members in these negotiations has been intended to allow laboratories to address MDUFA issues that would arise if LDTs are regulated as medical devices and if laboratories are required to register as device manufacturers. At various points, over the years FDA has asserted that LDTs are medical devices and that laboratories, which perform LDTs are device manufacturers.

ACLA has consistently communicated its disagreement with these assertions. The agency over time has articulated a number of LDTs regulatory proposals under which FDA proposed regulating LDTs as medical devices through draft guidance documents. FDA has also sought in certain instances to bring public and non-public enforcement actions upon certain lab developers tests or categories of tests.

In addition to these agency action, various FDA officials have affirmed the need for a new statutory authority for diagnostic regulation.

For example, FDA officials in New England Journal of Medicine article cited, "The need for common legislative framework to ensure that all clinical tests are accurate and reliable."

In parallel to the user fee negotiations and administration engagement, ACLA has been actively engaged in discussions with Congress and diverse stakeholders to design and negotiate a modernized statutory framework specific to clinical laboratory tests whether LDTs or IVD kits.

Originally, these discussions focused on the legislative discussion draft of the Diagnostic Accuracy and Innovation Act or DAIA and a subsequently involved to the current bipartisan and bicameral verifying accurate leading edge IVCT Development or Valid Act introduced by Senator Richard Burr ranking member of the Senate Health Education Labor and Pensions Committee. Senator Michael Bennet and Representatives Diana DeGette and Larry Bucshon.

The Senate Committee on Health Education Labor and Pensions has spent the last several months in detailed bipartisan committee discussions on the Valid Act in an effort to reach consensus on the legislation for potential congressional action this

year.

While there's much work to be done it has been a serious effort and the most significant bipartisan effort to date. And I would encourage stakeholders to take this effort seriously. All ACLA members perform laboratory-developed tests and therefore ACLA has prioritized both the administrative and legislative discussions that I've mentioned here.

Some ACLA members have voluntarily submitted or may voluntarily submit applications to FDA for medical device clearance or approval for certain LDTs. Within the COVID-19 pandemic response, some ACLA members have also submitted or may submit applications for Emergency Use Authorization of LDTs with the agency.

Against this background, we see a number of scenarios where use fees may be imposed or proposed for clinical laboratories develops and offering LDTs. The various scenarios including a potential new statutory framework make it necessary to outline various uncertainties impacting any proposed user fee allocation.

These uncertainties include the number and size of laboratories that develop and offer LDTs, the number of LDTs offered by given laboratories,

complexity and burden of any oversight for LDTs and LDT developers, criteria for and/or exemption from any premarket review or other application type. And finally, timeframe for any oversight framework.

In the draft MDUFA V commitment letter, I'd like to call attention to paginated page 31 in the commitments explicitly related to LDTs. The commitments outlined on page 31 are essential elements for transparency and accountability to understanding extent to which LDTs are or are not being reviewed by the agency.

During MDUFA V negotiations and discussions therein the FDA disclosed that resource predictions for MDUFA V and the resulting necessary fees do not include a significant expansion of regulation of LDTs. Though our projection of increase in LDT submissions to the agency.

This assumption within the projections is important as a starting point to understand the potential impacts if a dramatic change to the device regulation of LDTs were proposed and/or implemented in the future.

Critically, if thousands of clinical laboratories were suddenly required to submit thousands upon thousands of LDTs applications under

the device framework, the workload vastly exceeded the agency capacity plan for in the drafted MDUFA V commitment letter. During the pandemic, we've observed the significant backlogs and application reviews and delays in patient access which can occur when FDA's workload exceeds the agency's resources. And this all while again, I want to applaud the FDA and its workers for their incredible work during the pandemic.

We've also observed that review backlogs not only delay patient access but can also lead to further confusion as the agency attempts to apply new prioritization schemes as to the applications to get through the backlog often leaving developers and applicants in the dark.

This last point is why ACLA sought to revise language in the drafted MDUFA V commitment letter "To the extent that laboratories make submissions regarding LDTs that are covered by the MDUFA V agreement, FDA will treat such LDTs submissions no less favorably than other submissions to which MDUFA V performance goals apply."

LDTs are absolutely essential to tools for clinical care and for patients. And therefore any agency actions which impact LDTs must be made with

transparency, equity, and accountability through appropriate due process.

To reiterate, ACLA continues to assert the medical device authority does not apply to clinical laboratories nor to laboratory-developed tests. And ACLA and its members do not waive any potential argument or relief to which they may be entitled.

ACLA will continue to seek transparency on policies, proposals, or activities that may impact clinical laboratories and LDTs. And will continue to advocate against policies or activities that would inappropriately impinge upon laboratory innovation and harm patient access to accurate and reliable clinical laboratory services.

Whether in the context of the MDUFA negotiations or the legislative discussions such as on the Valid Act, ACLA and members are committed to pursuing clear and appropriate regulatory oversight and market pathways for laboratory diagnostics and innovation. Thereby ensuring that clinical laboratories remain a strong component of the nation's public health infrastructure and also ensuring broad patient access to accurate and reliable clinical laboratories tests. Again, thank you for this opportunity to speak at this public

meeting. Thank you again to the mutual negotiation teams and all the stakeholders who put input into the process to date.

MS. ROTH: Thank you to my industry colleagues. Thank you, Janet, Mark, Peter, and Tom for your support of this agreement and again, as I said for all of your collaboration over the past several months to bring us to the place, that we're in today.

The place that we happen to be in at this moment is significantly ahead of schedule. Which is a rare opportunity and I want to let everyone know that in order to make sure that we stay on track four our speakers for the next panel, representing patient consumer, academic, and physician perspectives, we plan to take a slightly longer than anticipated break. We will resume the public meeting at 2:15 where we will be joined by our colleagues on that panel as well. So until then, we hope you know we do hope all of you will return and we will see you then thank you.

(Off the record at 1:28 p.m.)

(On the record at 2:15 p.m.)

MS. ROTH: Welcome back, everyone. I want to thank you for rejoining us this afternoon to continue the discussion of the Medical Device User Fee

Amendment or MDUFA reauthorization. I'm pleased that we are able to assemble such an esteemed group of speakers for the second half of our discussion today. And we're joined first by Elizabeth Richardson from the Pew Charitable Trusts. So Liz if I could turn it over to you.

MS. RICHARDSON: Sure. And thank you so much for the opportunity to speak today. I know that the user fee process is an enormous undertaking for the agency and for other stakeholders in the negation. And I appreciate the chance to adopt a thoughts that's emerged from this process. I'm Elizabeth Richardson, I direct the Healthcare Product Project at the Pew Charitable Trusts.

We are a nonpartisan 501(c)(3) dedicated to research and advocacy and we work in a wide range of areas. We have a pretty broad health portfolio but the project I direct is focus on strengthening FDA oversight of a range of product categories including dietary supplements, invitro diagnostics, and emerging technologies like 3D printing and AI. And as you may have marked from that list a lot of the types of products, we're interested in are overseen by CDRH. So we were very interested to see what would emerge from the user fee negotiation process

and what potential implications it would have for our work and from there think about what we'd like to see the agency focusing its efforts over the next five years.

Today I just want to take the opportunity to reemphasize our general support for the user fee agreement process given how central it is to funding core FDA activities.

The fees FDA collects under the user fee agreements provide the agency with very badly needed resources to review applications and better facilitate the introduction of a wide variety of new medical technologies. We were interested to see that the total fees collected by the agency could reach 1.9 billion.

Though I think roughly double the amount of fees authorized under MDUFA IV and I think these funds will help FDA to delivery more efficient and comprehensive oversight process that is better resourced to protect consumer safety and adapt to a rapidly evolving device market, where emerging technologies are opposing challenges to traditional FDA oversight.

CDRH has for many years done a lot with very limited resources and so we look forward to seeing it

can do with more. Of particular interest to Pew though is how the agency will handle oversight for the growing market of AI-enabled digital health products. The agency has already approved or cleared nearly; I think 350 AI-enabled devices for a broad range of applications. And nearly 140 have been granted since the start of 2020. And the pace of submissions that include an AI component is only expected to grow over the next five years.

And these tools offer unique opportunities to lower costs, improve patient care, and health outcomes. But the volumes applications and the pace at which these products evolve pose unique challenges to the FDA's traditional approach to oversight. And these products will need, not only to be reviewed to ensure they are safe and effective at time of approval they also need to be adequately monitored over time to ensure they continue to be safe and effective in the real world. And when used on diverse patient population.

And accomplishing that will require the right mix of expertise among FDA staff as well as ongoing commitment to developing regulatory policies that can facilitate ongoing innovation while still providing adequate public health safeguards for these rapidly

changing products.

We were encouraged to see that the agency will be focusing on developing that expertise as part of its commitment. Over the next five years we also hope that the agency will continue to focus on improving transparency around these products, the public meeting in October was a great start. But we're eager to see what's next in that process.

We were also encouraged to see that health equity was a core part of the CDRH'S strategic action plan over the next three years. Again, an encouraging start and we're interested to hear from the folks at FDA about what's next. And we hope that the agency will continue to invest in that work even beyond 2025 to ensure that equity considerations are embedded throughout its review and oversight process.

Similarly, 3D printing is increasingly being used at the point of care to manufacture a range of products including anatomical models used to guide surgery re-planning. And medical devices like surgical cutting guides. This technology allows for decentralized manufacturing of highly customized products, which could one include implants, pharmaceuticals even biological products. That are manufactured directly within healthcare facilities.

But to date, most of the clinical applications for including manufacturing techniques have been in medical devices. And like any medical product, 3D printed devices also carry risks. And existing laws and guidance meant to ensure the safety of devices don't clearly map to this technology only in part to how much customization is possible. And also the way the technology allows for decentralization.

It's a lot of potential new facilities for the FDA to oversee. So as more healthcare facilities adopt 3D printing, they'll need clear guidance from FDA on how they can deploy this technology in compliance with existing regulations that ensure the safety, the quality, and the effectiveness of the medical devices that they're producing.

We were encouraged to see that user fee revenues would go towards supporting timely development of FDA guidance documents and hope that this will include the draft guidance for products that are 3D printed at the point of care. The agency is in the process of developing such a document. We're encouraged by the work that has already been done to gather feedback from a range of stakeholders who are either developing or deploying this technology.

And look forward to the guidance when it's published and hope it's published soon. And with that, I just want to wrap up by concluding with how important it is for CDRH to be adequately resourced to meet the challenges that it faces. And we look forward to seeing how the agency leverages the additional funding it receives through user fees over the next five years. Thanks again for the time to speak

MS. ROTH: Thank you, Liz. I have to say one of the privileges of this role is being able to hear directly from stakeholders, such as the ones we'll hear from this afternoon, Liz, and others, as in terms of feedback to the agency.

So I again want to just acknowledge how important it is to me personally and to the agency to hear your perspectives and say thank you for that. And with that, I would like to turn to our next speaker Dr. Raymond Golish, who is joining us on behalf of the American Academy of Orthopedic Surgeons. Dr. Golish?

DR. GOLISH: Good afternoon. I'm Raymond Golish I'm an orthopedic surgeon, a spinal surgeon, a medical device scientist, and a member of the Devices Biologics and Technology Committee of the American

Academy of Orthopedic Surgeons.

On behalf of over 30,000 members of the leading surgical professionals' society devoted to musculoskeletal care one of the largest drivers of patient well-being, we applaud the agency for embracing the public dialogue and our opportunity to comment on the critical program known as MDUFA. Thank you to Doctors Califf, Shuren, and Marks and for the earlier introductory remarks.

And our comments encompass three main points. First, the American Academy of Orthopedic Surgeons recognizes that safe medical devices, implants, and technology are really now central to high-quality patient care. As clinicians, the core of our profession is the spirit of caring. And the use of non-surgical and surgical care to assist patients in living their best lives.

But in addition to that, some things do improve over time. And improvements in medical device technology have dramatically consistently, and positively impacted the quality and quantity of life of countless patients throughout the United States and worldwide.

Second, the development of safe and effective medical devices and technology can only result from a

multistakeholder interaction among patients and their advocacy groups, among physicians caring for patients, among the regulated device industry, and regulators, and regulatory scientists. And the primary physician advocates for musculoskeletal patients we recognize that our many colleagues both FDA and in industry represented today by AdvaMed and MDNA and others are committed professionals trying to do their best work on behalf of patients. Far from being zero-sum. These multiple stakeholder interactions are synergistic. They require vigorous debate as well as collaboration to achieve mutual understanding we need to underpin patients' best interest.

Finally, we support and applaud the extensive work that has and is being done at the agency to make MDUFA fee stewardship efficient and purposeful as MDUFA iterates over time. And the investment on behalf of patient care ought to have a return. Over its evolution, MDUFA has embraced the goals of timeliness, quality, transparency with evolving foresight over its lifecycle.

All of three of these interacting goals yield a safer more effective and more innovative cycle all in the interest of patients and the interest of

physicians' ability to care for them.

Thank you for the opportunity to comment today as physician advocates for our patients in this critical substantive program at FDA and its most recent developments on behalf of patients.

MS. ROTH: Thank you too, Dr. Golish. And next, I would like to turn to Jennifer Dexter from the National Health Council.

MS. DEXTER: Thank you so much for the opportunity to be here today. The National Health Council really appreciates the opportunity to provide feedback on the Food and Drug Administration's performance goals for MDUFA for 2023-2027. This process and its outcome are incredibly important to patients. And this opportunity along with the stakeholders' meetings held throughout this process will help make sure that the final outcome meets the needs of patients.

Created by and for patient organizations over 100 years ago the NHC brings diverse organizations together to forge consensus and drive patient-centered health policy. Made up of more than 140 national health-related organizations and businesses, the NHC's core membership includes the nation's leading patient organizations. Other members include

health-related associations and nonprofit organizations, including the provider, research, and family caregiver communities. And businesses representing biopharmaceutical, device, diagnostic generic, and payer organizations.

The MDUFA V draft goal level demonstrates FDA's commitments to modernizing its regulatory framework and activities to meet the demands of the 21st Century technologies. The NHC appreciates the hard work that went into the aspects of the agreement but will ensure that the FDA and its industry work together to make sure that the process of approving or clearing new medical device innovations is efficient, properly resourced, and focus on getting innovative products out to patients quickly and safely.

Importantly, we are pleased to see that there are several aspects of the agreement that are focused on infusing the voices of patients into the process and making sure that the real needs and goals of patients are considered by FDA and manufacturers. I'd like to specifically comment on some of those today. The first is the section on enhancing the patient's voice in device development and decision-making. The NHC applauds the FDA for continuing its

crucial work to involve the sides of patient engagement for MDUFA V and the other agreements.

Specifically, we applaud the inclusion of the following provisions. Expanded clinical statistical and other scientific expertise and staff capacity that respond to submissions, containing applicant proposed use of voluntary patient preference information, voluntary patient-reported outcomes, and/or patient generated health data. Issuing guidance on best practices on incorporating clinical outcome assessments and premarket studies. Updating existing guidance on patient preference information. Support for the use of innovative technologies to capture patient input and reduce patient burden to inform clinical study design. And conduct with the goal of reducing barriers to patient participation and facilitating recruitment and retention.

Public meeting to explore ways to use patient-generated health data to help advance remote clinical data trial collection and support critical outcome assessments. Activities to improve the regulatory predictability and impact of patient science. And finally patient-friendly educational modules on device trials, real-world data, device development tools, and regulatory frameworks.

Together these initiatives will help assure us that the medical device approval process better incorporates the input of patients and drives equity. The multitiered approach of making sure the FDA has the expertise and capacity to utilize many types of data, including patient-generated data. Identifying high-impact opportunities to incorporate patient perspectives and creating tools and resources to educate the general public on the device development process, is effective and will lead to better end results.

Now, the aspects of the agreement that address patient engagement are very positive steps. It will be critical that the details of these processes are developed correctly from the beginning. It's imperative that every step of the way patients and patient organizations are engaged in implementation. The NHC has a long history of helping manufacturers and regulators create quality patient engagement processes. We stand ready to assist this agreement is modified and implemented.

The second topic is advancing real-world evidence and use in decision-making. The NHC is supportive of leveraging real-world evidence to inform FDA's decision-making. The proposed

provisions for MDUFA V are a commonsense approach to furthering the signs of real-world evidence. We support updating the use of real-world evidence to support regulatory decision-making for medical devices guidance. We believe if appropriately crafted the proposed updated guidance can lead to a better and more consistent collection of data and best practices.

Finally, digital health. The NHC understands the importance of increasing timely access to existing and innovative digital health tools. To best help, patients access care and to assist in the effective and equitable development of new treatments. We appreciate the attention and the agreement to growing FDA's ability to quickly evaluate digital health tools and provide needed clarity on the process of evaluating digital health tools.

I note that PDUFA VII agreement also has significant commitment in the area of using digital health technologies in drug development and I encourage FDA to work across the centers to ensure these work streams are coordinated. Thank you so much for this opportunity today. And we look forward to working with you as we proceed to implementation.

MS. ROTH: Thank you very much. And with that, I'll turn to Cynthia Bens from Personalized Medicine Coalition. Cynthia?

MS. BENS: Hi, good afternoon, everyone. My thanks to the FDA for the opportunity to share some thoughts on why the Medical Device User Fee Program is important for precise medicine. And also to reflect on the MDUFA V commitment letter. As Lauren mentioned, I'm Cynthia Bens and I serve as Senior Vice President of Public Policy at The Personalized Medicine Coalition or PMC.

PMC is a nonprofit education and advocacy organization that has more than 220 members from across the healthcare spectrum. We're working together to advance personalized medicine in ways that benefit patients. As you probably heard throughout the day the MDUFA program is a critical source of funding, having a well-resourced focus and flexible FDA, is essential to our ability to achieve our mission of bringing forth the best treatment and prevention strategies for each patient. And ensuring they're delivered based on that person's biology, medical history, circumstances, and values.

PMC's analysis have shown that initiatives advanced by the FDA in recent years have fostered

many notable regulatory milestones including in 2021 CDRH'S recognition of the first tumor mutation database to allowing test developers to use real-world data to support the clinic validity of new diagnostic tests as well as approval of several new diagnostic indications that will allow for targeted treatment decisions for various health conditions.

The new technologies and policies will help innovators and physicians develop safer and more efficacious treatment regimens that are based on principles of patient-centered care. It's difficult to summarize all the reasons why MDUFA V will make ample changes to the field of personalized medicine, but I'd like to emphasize that PMC is pleased to see increased staffing to support CDRH review activities, additional considerations for advancing the use of real-world evidence in real-world data. And increasing patient engagement, and the use of digital health tools in the MDUFA V commitment letter.

In comments provided at the beginning of the MDUFA V process, we highlighted that progress made in previous reauthorizations have enabled CDRH to reduce the total time it takes return a decision on a product submission while maintaining high standards for ensuring safety and effectiveness.

This has been possible because of additional opportunities for engagement between industry and CDRH during the device repeat process. These interactions allow product sponsors to better understand FDA's data needs and we understand that this level of engagement is only possible if CDRH is properly resourced.

PMC supports resource levels in MDUFA V that provide adequate staffing to complete timely evaluation of personalized medicine products that are submitted for CDRH for review. To help CDRH to continue fulfilling its mission, the 21st Century Cares Act included provisions supporting CDRH'S efforts to maintain capable and well-trained staff. FDA has made progress in addressing staffing needs, but we understand that there are still challenges that remain. And our previous comments on the MDUFA V reauthorization we encouraged CDRH to continue using hiring authorities provided by the Cares Act and to pursue enhancements that assist the center in meeting hiring needs. It's reassuring us that MDUFA V resources will be devoted to expanding use of hiring authorities under the Cares Act.

Regarding real-world evidence and real-world data we at PMC believe that data collected about an

individual's lifestyle, disease biology, and treatment outcomes, can be harnessed to transform the future of personalized medicine.

The inclusion of guidance development under MDUFA IV on the use of real-world evidence along with resources to allow CDRH'S participation in the coordinating committee for the National Valuation System for Health Technologies or NEST and the Medical Device Innovation Consortium, MDIC, provided a foundation for understanding how and when our RWE and RWD may be used to support medical device regulatory decisions. To further FDA's success in this area PMC called for exclusion of resources of MDUFA V to continue CDRH'S involvement in NEST and MDIC.

We're pleased to see that the agreements support these activities in addition to the issuance of updated guidance, providing more clarity on CDRH'S RWE activities. And at least two public meetings to update stakeholders on the RWE program.

It's also promising what CDRH plans to convene experts to advance innovative methodological approaches on the development and analysis of RWE. As well as best practices. Because the impact of CDRH'S work in this area extends beyond regulated

industry, we'd asked for additional transparency on evidence available for researchers, health data organization, and other non-industry stakeholders like patient groups to interact with the agency on these two important issues.

And my third and final point identifying the benefits and risks of medical products that matter to patients is really essential for the effective delivery of personalized medicine.

We generally support continued work and understanding patients' preferences and we believe that it can potentially advance activities to positively impact the design and conduct of premarket clinical studies, benefit-risk assessments, and post-market evaluation of medical devices.

We appreciate that CDRH'S commitment for patient engagement under MDUFA V include an update to FDA's existing patient preference information guidance and will address common questions for those interested in voluntarily using patient preferences information in their regulatory submissions. PMC also called on the FDA at the start of the MDUFA V process to take steps that would accelerate the acceptance of digital health technologies. We believe that advances in sensing technologies and

self-management platforms are important tools for personalized medicine that could be leveraged in ways to allow for diverse populations and patients in difficult geographical locations to be included in clinical trials.

MDUFA V includes commitments to reduce barriers to patient participation and facilitate recruitment and retention in clinical studies by utilizing in data technologies to capture patient input and reduce patient burden. We also look forward to the planned public meeting in FY24, which will explore ways to use patient-generated health data to help advance promote clinical trial data collection.

Finally, we believe that digital health tools can enhance trial efficiency parallel to the delivery of real-world care and may even provide personalized insights at the point of care. So we really applaud commitments in MDUFA V that are intended to foster the adoption of digital health technologies. As evidence generation continues to evolve and to advanced understandings of how to measure and monitor software quality.

The FDA and external stakeholders will really benefit from CDRH'S continued work under MDUFA V to

develop guidances and provide expertise for premarket submissions that include software, interoperable devices, wearables, AI, and machine learning, and digital health technologies.

So thanks again for the opportunity to speak on the panel. My colleagues and I look forward to working with you and Congress as MDUFA V reauthorization process moves forward in the coming months.

MS. ROTH: Thank you very much. We look forward to working with you as well. Now, I'd like to turn it over to Michael Abrams from Public Citizen.

DR. ABRAMS: Thank you, Laura. Can you hear me, okay?

MS. ROTH: Yes, sir.

DR. ABRAMS: Good. Thank you. So I'm Michael Abrams, Senior Health Researcher with Public Citizen's Health Research Group. And I have no financial conflicts of interest to disclose today.

During this most recent user fee reauthorization cycle, I presented Public Citizens' views at the FDA convened Consumer Patient Stakeholder meetings for both prescription drugs and for medical devices. And that expanse has left my colleagues and I deeply concerned that existing

regulatory review programs for these critical components of healthcare are increasingly compromised by substantial regulatory capture by industry and by concomitant undue pressure.

The recently released MDUFA draft commitment letter is no exception. We feel that it expands a program that gives device makers too much influence over their federal regulators. The draft commitment language may well please industry but certainly does not advance the best interests of consumers, especially for patients who rely on the FDA to protect them from ineffective or unsafe medical devices. Accordingly, my brief testimony today offers a list of reforms that should be appended to the goals and procedures of the medical device program.

And these are reforms that I have suggested throughout the MDUFA reauthorization process. So they will be familiar to many staffers that are on the webinar today. As two examples that evoke the need for such reforms, consider, number one, implant the spinal cord stimulation for pain. And secondly, transvaginal meshes for surgical repair. Past studies by our group Public Citizen had revealed that the FDA's lacks approval. And subsequently,

inadequate oversight of these high-risk implantable devices resulted in serious harms including fatalities for large numbers of patients.

There are several reforms that can reduce such morbidity and mortality. I strongly encourage the FDA to carefully consider each of them and to include these proposals in the commitment letter packet that is eventually submitted to Congress. To advance FDA's key mission of efficiently managing a safe and effective medical device pipeline, the FDA should, number one, require high-risk, permanently implanted medical device sponsors to submit PMAS with data from well-designed randomized trials. This is not real-world evidence. Well-designed trials is what should be emphasized.

The FDA should make publicly available the following, reveal summaries for all PMAS supplements for Class III medical devices. They should make publicly available comprehensive assessments of adverse events for all approved PMAS and supplements and MOD and the agency's MOD Database. They should make publicly available a list of Class III devices for which approval is granted based solely on the literature reviews of studies assessing devices other than the ones that PMA was actually -- the original

PMA approval was actually sought.

And finally, the FDA should make publicly available PMA annual report information that reveals the number of devices sold, shipped and deployed, or implanted.

Another point, the FDA should designate other staff who are not involved in providing advice and guidance to sponsors prior to the submission of a PMA or 510(k) premarket submission for a medical device to review and make decisions on any subsequent premarket submission related to that device. To ensure the integrity of these reviews and decisions, a firewall should be created between the FDA staff involved and any pre-submission interactions and those involved in the formal evaluation of the PMA or 510(k) premarket submissions, a firewall should be created.

And FDA should create a 510(k)-predicate database that is used to issue annual performance reports examining the quality and appropriateness of legacy devices as a foundation from premarket clearance decisions.

And finally, the FDA should establish annual public health performance measures that quantify the benefits of harms and the harms of medical devices

that have been cleared, approved, recalled, or even rejected. These proposals all aim to strengthen FDA's independence from the industry it regulates. And its commitment to safeguard the medical device pipeline by careful transparent monitoring of regulatory decisions especially as they pertain to the safety and effectiveness of high-risk devices like implantable stimulators, meshes, stints, valves, catheters, and countless other invasive technologies.

Remarkably the draft commitment letter currently contains none of these commonsense patient-centered suggestions. Instead yielding to industry's obvious desire to bring devices to market as quickly and as cheaply as possible. Accordingly, we strongly encourage the FDA to revise the commitment letter by aiming assertively to the following two justifiable goals.

First, establish and expand efforts that explicitly promote a regulatory pipeline that yields safe and effective devices. First and foremost. And second, perhaps most importantly, the FDA should press Congress to provide the agency with taxpayer-funded resources to achieve that first goal independent of what we know to be pernicious financial interest from the industry that it

regulates.

This last point is essential if congress truly desires a sound regulatory system, it must give the FDA direct financial appropriations and authorities to maintain and adapt that system for its critical part the FDA must not be shy in asking for those monies and mandates.

And finally, I want to point out a recent scholarly analysis published about MDUFA concluded that the program, "Raises serious questions about PDUFA's on US regulatory policy. That policymakers should reconsider perpetuating this UFA system and reallocate the necessary funds to relieve FDA of its financial reliance on industry." This is similarly applicable to MDUFA. Thank you.

MS. ROTH: Thank you, Michael. We appreciate yours and others' participation throughout this process. Including participation by our next speaker, Paul Conway, from the American Association of Kidney Patients. Can I turn it to you?

MR. CONWAY: Sure. Thank you very much, Lauren. My name is Paul Conway, I serve as the Chair of Policy of Global Affairs of The American Association of Kidney Patients. Next slide, please. Right off the top, we'd like to say as the American

Association of Kidney Patients understands the unique role that FDA plays in the regulatory environment, the duties it has been charged with by citizens, and by the congress through legislative authority.

We also understand the pressures that are faced by industry to get products into the marketplace and most importantly we live the experience of understanding what it's like to be a patient consumer with a chronic disease or a disease that has very high mortality, waiting for devices that help keep us alive. As a kidney patient of over 42 years I've lived on FDA-approved safe medical devices and we respect the role of the FDA and the fine balance that you've had.

Essentially, what the FDA has been able to do through the MDUFA program is the democratization of the federal regulatory process and the product development lifecycle process for medical devices, especially for kidney patients. For nearly 60 years the kidney space was stagnant in terms of medical innovation. But things changed once the FDA began to weigh in heavily on the science of patient engagement and patient insight data inclusion. This has made definitive changes in the views of kidney patients. Practically speaking, you can see some of them right

now.

Patients can now speak for themselves at the FDA table and other tables right next to those who purport to speak for patients, be they doctors or be the industry. Our voice is equal. Special interests are less effective now at saying that they speak for patients. Why? Because patients are in front of the FDA closely involved with the FDA and working with industry and medical professionals who are trying to bring devices to the market.

Our voice is clear, and it is heard by FDA. FDA efforts have changed patient self-perception because now you're not living life in isolation with a chronic disease or a fatal disease. You're able to give your insights and your lived experiences to researchers and regulators so that they better understand, and the industry better understands what it's actually like to bear the burden of disease.

Patients are also much more aware of how valuable their insights are. This has empowered patients and advocacy organizations in a way that has not been done in the past 20 or 30 years. It has been revolutionary to advocacy organizations in individual patients. And what we have seen across the federal landscape is by the leadership of FDA in

the mainstreaming of patient insight data and patient engagement, it has definitive impacts on PCORI. The National Institutes of Health, CMS, the CDC, DOD, and VA, and other programs. Because they're now managing the expectations of demands of patients to be more heavily involved and have their insights appropriately elevated and listened to.

FDA's efforts have worked hand in glove from our perception with PCORI in terms of bringing patients to the research table and to the regulatory table to make certain that those insights and that engagement is shared both on the research side and across the product development lifecycle side. And increasingly you're seeing references to this type of evidence of patient science in all of the peer-reviewed journals, especially in Kidney World and beyond. Next slide.

Of particular interest to AAKP has been the priority areas that FDA within MDUFA for patient science and engagement and real-world evidence. Our board and our advisory board of patient ambassadors across the United States have very heavy experiences as professionals in government, in industry, in the media, and on capitol hill. They happen to also have kidney disease. So our focus was very much on the

capacity building of the FDA to meet what we are seeing as a fine age of innovation in kidney diseases.

But it's very important that the agency has focused on capacity building, training, and the provision of best practice guidance for the industry. We believe this is one of the most fundamentally important things that the agency has done and clearly articulated in the agreement.

We were also extremely pleased with the focus on new technologies to harness patient insights. We believe that will work very nicely along with advocacy organizations in this space. We're very, very pleased to see the effort to increase guidance on best practices for clinical outcome assessments in the premarket studies and the potential application for primary or coprimary clinical endpoints.

We're also very glad to see the commitment to transparency. We think this is very, very important across the board to all stakeholders, but especially to the American taxpayer. We will look forward to working closely with the FDA on transparency initiatives and other initiatives that are spelled out very clearly. Next slide.

The reason why we focused on capacity of the

FDA is based on the practical impacts that our patient advocacy organization as the oldest kidney patient organization in the United States has seen in terms of what the volume has been coming to us. Just in the past three years and we monitor these facts and statistics very closely, we've had over 200 entrepreneurs, private investors, and companies come to us asking very specific questions, years in advance of going forward with new products to understand patient insights and patient lived experience. This has been new in the past 10 years. Our organization has not seen this in our 50 years of existence.

And we think it's very important that folks understand this on capitol hill and in the agency. We see it a bow wave of interest coming in the battle of kidney disease and other diseases for innovations in medical devices. In that same period of time, we had over 350 contacts with government, with media, with private sector, research institutions, federal researchers, all trying to understand what our capacities are, what insight data we can bring, and how we can collaborate with them to better elevate the voice of kidney patients in their lived experiences within the product development lifecycle.

For our patients right now, this across the United States, this is a golden era of having their voice and their lived experiences respected, their dignity respected, and most importantly their insights applied across the product development lifecycle.

The last thing that I would say is that the practical impact on us has been a restructuring within AAKP. We've had to add additional national contractors for our social media capacities, for our capacity as capture insight data. Most importantly, it led to the retitling of one of our top staffers as a director of patient insights data analytics, and advocacy. So we have had to adjust as FDA has adjusted as well. Next slide.

And to have you understand perhaps you spoke to the international program earlier today, the FDA did, and I just want to be very clear what the practical impacts of the FDA's leadership role has been globally. It has not gone without notice. So the WHO, and the Pan American Health Organization through their engagement program with people living with noncommunicable diseases, is working with patient and patient advocates across the world to better understand what avenues they have to have their life

experiences captured and their insights captured and example provided in the United States by the FDA, and with your collaboration with other organizations, given patients and patient advocacy organizations, great lease on life and great optimism that their voices can be captured.

And this is very important because many companies that are in this space are international companies. Not simply on the drug side but also on the device side. We've seen great support of the FDA efforts and efforts to capture patient insights with numbers of the European Union and also with of course the United States Congress. Tremendous interest in whether or not our voices are being included, and we are proud to say they are included through the FDA and through the MDUFA process.

The other thing that we're seeing is great interest among patient advocacy organization or how to access and give their insights in a growing international collaborative that is focused on artificial kidneys, implantable kidneys, and xenotransplantation. We believe that the FDA's example has provided a very good pathway of patients around the world in many different countries to have their voices heard on this great age of innovation

and artificial organs.

And then lastly, some of the key events in the kidney space. We do a global summit in partnership with the George Washington University. That has had over 80 countries, over 20,000 patients and researchers, and medical professionals engaged. Some of the top programming is when the FDA officials participate in that program. And lay out what the avenues are that FDA has created for engagement. And again that reinforces the expectation of patients and advocates and the understanding of regulators around the world.

The IDEAS Summit at the University of Washington is another international event focused on artificial implantable kidneys and devices very high interest with participation of FDA and what FDA has to say about capturing patient insights and patient engagement. And finally, of course, the International Society of Nephrology And American Society of Nephrology, both conducting global events where FDA officials, particularly speaking to patient engagement have been extremely well-received and each of those organizations as well patients have been elevated to have key roles not simply providing a qualitative perspective but giving their quantitative

insights from their advocacy organizations and from their life experiences.

And on a final note, on the last slide, this is a survey that AAKP does every year. It captures a very simple question. And I think a compelling one, which is this. If you ask kidney patients whether or not their insights would be valued in the development of new clinical trials and devices if they have that opportunity, how many of them would participate in it? You can see the number is actually 94 percent. Simply having a disease and managing the burden is one thing. But there's a certain level of degree of idealism that goes with being a chronic disease patient. And what that idealism is, is quite simple. You don't want other people to suffer the same thing that you've gone through and the luck that you've had in living another day. Your experience has to matter. The program that FDA manages from our perspective, with MDUFA, is one of the most important outlets for patients and patient advocates to make certain that their life matters, and that when they are gone, the insights they will have shared will have improved the lives of others.

We believe it's a fundamentally strong program. It has great talent associated with it. And we feel

like our voice is equal at the table and our concerns are equal too, for safety, accountability to the taxpayers, and our concerns are heard. So we appreciate the opportunity to participate. And Lauren, thank you very much.

MS. ROTH: Thank you, Paul. Those are important insights for us and for all of the folks participating in this meeting today. And I just want to say thank you again for taking the time to share those. And I also wanted to turn it over to our next speaker, Amy Ohmer, from the International Children's Advisory Network. Amy?

MS. OHMER: Thank you, Lauren. I'm so glad to be here today. And before I start off, I just want to say, I'm very thankful to the agency and all of you at the MDUFA V team for the opportunity to share some thoughts today. I represent the International Children's Advisory Network, as you just shared. And our mission is simple. It's to foster the greater global understanding about the importance of the pediatric patient and caregiver voice in healthcare, and clinical trials, and research.

And our organization, our membership is our kids. So this is a really important thing to us. To be a part of. And at iCAN we support the work that

MDUFA is doing to support the best interests in the healthcare of patients of all ages. Right down to the youngest patient.

We believe the goals that MDUFA has set forth help us to address the need to get patients' medical devices quickly and safely with an emphasis on ensuring patient voice, especially through pediatrics. This is an important need for us because pediatrics is a short time. And our kids deserve the best quality healthcare that they can receive at the very beginning of life. So that they have a longer, happier, and more fulfilled life as they go forward. Next slide, please.

And through our partnership with the FDA, we've been very fortunate to be able to share in providing that patient perspective through different projects that we've worked with the FDA. And we also, on a continuous basis, provide young people to share their voices forward focus groups, speaking opportunities, surveys, and any kind of creative communication, like art and stories, and their own ideas. And this is an important piece that we hope is included within the MDUFA plan as we go forward that we should meet kids where they are today.

And having them share their voice in unique

ways that are appropriate for children to communicate through. That will be a support that will ensure that we're understanding the needs of patients. They can communicate them. But it may be just different than what you're currently seeing through the adult population. And next slide, please.

So what we recommend is to include the patient voice early on within all projects and all reviews. And we want to support the feedback loop of patient review changes and then another patient review. This is incredibly important as kids like to know how their voices are being utilized, what they're sharing, the content, how important it is, and we think that should also be included as another suggestion. We also think that -- as we had spoke earlier about meeting children and young people right where they are by offering a variety of ways to participate in supplying that patient perspective is very important. And we wanted to make sure that the FDA recognizes that the culture and community of pediatric is one that needs to support multiple language and multiple reading abilities.

This is critically important for our kiddos as they go forward and as they grow so they can understand and work in partnership. We also

recognize that we have work on our end to do so we'd like to incorporate things to help support your work with curriculums that we're designing to share insights of our young people and to help train and teach and ensure that the kids as they grow up are understanding more of what they can accomplish and do.

To that end, we support the use of patient science engagement and the use of real-world evidence. We work in collaboration with many different groups, and we want to ensure that community of support is included within pediatric medical development. That it's not settle driven and that it incorporates everyone that can support children in a way that brings about the best possible health.

And then we would also like to provide support for showing the draft guidance practices and specifically having our kids review that material and that content right directly from the FDA and showing where they can also support and help.

Incredibly important and very critical in ensuring that our kids feel as though they're stakeholders and have the ability to move ahead in a way that leads them to know that they were being

thought of as a group and not as an afterthought. Next slide, please.

And then we would also like to continue to have engagement from the FDA, I think this has been a wonderful thing in that the FDA has participated in helping our kids understand and learn directly from their staff points. So we would love to continue to have that happen, too, as far as supplying educational materials directly from the FDA to help teach the kiddos on what they can do to participate as they move forward. And with that said, that's our end.

And again, I just want to thank you for allowing us to few minutes to talk about the young person's perspective and making sure that they're not forgotten as we move forward in MDUFA V.

MS. ROTH: Thank you, Amy. Thank you very much for providing that important diverse perspective from the perspective of our young people. And with that, I would like to turn it over to Dylan Simon from the EveryLife Foundation for Rare Diseases. Dylan, thank you for being here today.

MR. SIMON: Thank you, Lauren. Thank you for inviting me to present on behalf of the EveryLife Foundation for Rare Diseases in the rare disease

community. My name is Dylan Simon, and I am pleased to serve as director of policy for the EveryLife Foundation for rare diseases.

We are a rare disease policy organization that understands that no disease is too rare to deserve diagnosis and treatment, and rare disease devices should be safe and effective. To that end, we convene a robust coalition comprised of patient advocacy organizations, biopharmaceutical partners, coalition groups, and other relevant stakeholders with a shared interest in rare diagnostic and therapeutic development, and regulatory infrastructure policy. Today's remarks are a reflection of the engagement with this coalition.

First and foremost, thank you for all the work that you and your FDA colleagues do to protect the rare disease community and the nation. We are grateful for your leadership as your capacity was stretched during the pandemic to ensure that the urgency of other areas did not wane.

For the more than 30 million Americans living with rare diseases, a disproportionate percentage of whom are children, the pandemic further exacerbated the challenges they face in rare disease therapy and diagnostic development.

A joint FDA/NIH report about medical devices and rare diseases found that 77 percent of clinicians cite a need for entirely new diagnostic and/or therapeutic devices, with 64 percent dissatisfied with existing diagnostic and/or therapeutic devices. Around 95 percent of rare diseases still having no FDA-approved therapy. Innovation in diagnostics and devices is essential for filling this unmet need in the rare disease community. The continued commitment and understanding of that need is evident in the MDUFA V enhancements we are discussing today.

To start off CDRH has long been a leader in patient engagement efforts and the commitments made during MDUFA IV built upon that legacy. CDRH has been a leader in committing to robust patient engagement through the Patient Engagement Advisory Committee and the patient and caregiver connection programs.

In addition, the advancements in the conduct and use of patient preference information is directly attributable to the center's commitment to meaningful incorporation of patient experiences in the regulatory process.

The EveryLife Foundation is very pleased to see the FDA's commitment to advancing the field of

patient engagement clear in the MDUFA V commitment letter released last month, including the continued growth of expertise on how to include voluntary patient preference information, voluntary patient-reported outcomes, and/or patient-generated health data in submissions. In addition, agreeing to provide best practices on how to incorporate clinical outcome assessments identifying. As well as identifying ways to improve the impact of patient science that includes expanding staff understanding of patient science and engagement through increasing training, and exploring how to best include a diverse treatment patient perspective. In addition, we want to thank you for all the great work that is occurring at CDRH around innovations in digital health.

We are pleased by the launch of the digital health center of excellence and eager to see all the great work the center will do in setting international standards on developing software and other digital harmonization efforts. Within the rare disease community, digital health holds great promise for shortening the diagnostic odyssey for so many with rare diseases. During the COVID-19 pandemic, the use of telehealth grew tremendously within the rare disease community.

It showed that temporary policy changes allowed 46 percent of Americans to replace a canceled healthcare visit with a telehealth service during the pandemic. With so many patients accessing care virtually, expectations for the future of our healthcare system have shifted significantly and 76 percent of Americans now report having a strong interest in using telehealth moving forward.

Telemedicine and digital health will play a vital role in the diagnostic and therapeutic process moving forward. The EveryLife Foundation is pleased to see the FDA is working to stay at the forefront of digital health with the inclusion of digital health in the MDUFA V commitment letter.

The continued focus on developing expertise on software, interoperable devices, and artificial intelligence/machine learning highlights the understanding of the expected growth in the digital health space and how important it is within the device development process.

In addition, we would also encourage that as we work to further refine these commitments, we think about how to better engage with the rare disease community. While many rare disease priorities are identified broadly throughout the MDUFA V commitment

letter, challenges specific to the rare disease community are not addressed.

Understanding the natural history of a disease is foundational to a rare disease therapy development program. However, because of the small numbers of patients affected, the natural history of rare diseases is often poorly described, making it difficult to engage the agency consistently in the development of overarching standards.

In addition, the ability to detect clinically meaningful outcomes requires understanding of their rate of occurrence, rare ability, importance to patients, and the amount of changes that would be considered clinically meaningful. All of these contributes to difficulties in powering a study in an already small population. The incorporation of patient preference studies discussed multiple times throughout the MDUFA V commitment letter is a vital part of improving the regulatory process.

Due to the unique challenges of the rare disease regulatory process such as those just described, it is important for the FDA to consider these complexities and how they impact existing and future guidances.

We encourage CDRH to prioritize strong

collaboration with CDRH's Office Of Organ Product Development, identify areas of need for rare-disease specific guidance that can ensure the benefits of patient preference studies and CDRH's dedication incorporating patient experience data into the regulatory process can be fully realized by the rare disease population as well.

The EveryLife Foundation is encouraged by the provisions within the MDUFA V commitment letter. We are grateful to all of the community members who have worked so hard to ensure that the needs, priorities, opportunities of the rare disease community is so strongly reflected in these considerations. And our collective patient community organization partners are eager to continue to lean in and work with the agency, sponsors, and patient communities to ensure that the rare disease communities' patient experience continues to inform considerations and decision-making all throughout the product development life cycle.

Thank you for your tireless work on behalf of the rare disease community. And for your commitment to ensuring that the promise of today's pipelines will change health outcomes for this generation of patients. Thanks so much.

MS. ROTH: Thank you, Dylan. Thank you again for joining us today. And for our final speaker in this portion of the agenda, I would like to welcome Paul Melmeyer from the Muscular Dystrophy Association. Thank you, Paul.

MR. MELMEYER: All right. Thank you very much, Lauren. I am Paul Melmeyer the Vice President of Public Policy and Advocacy at The Muscular Dystrophy Association. Seeing we have 23 minutes left in this section maybe I'll wax about medical devices which I've long wanted to do. No, I'm kidding, I'll stay within my time allotted.

So just as a quick introduction to MDA, we serve the neuromuscular disease community through a variety of services including, advocacy, education, summer camps, family and community supports, coordinated care approaches, and funding research and scientific discovery. Individuals with neuromuscular diseases use devices throughout their experience with their stees, their diagnosed using either newborn screening, or genetic testing, or other diagnostics, oftentimes mobility assistive devices, and some often also use ventilators, respirators, or tracheas. So consequently devices are very important to our community which is why MDA puts a focus on FDA's

medical device efforts including as a member of the patient and caregiver connection, participating in patient engagement advisory committee hearings, and taking advantage of all MDUFA participation opportunities that are offered to us. So there are several important provisions within the MDUFA V commitment letter that I would like to highlight.

First and foremost a well-funded CDRH is really an existential importance to our community. And to bringing life-changing diagnostics and devices to those with neuromuscular diseases. And so we're pleased to see over 200 -- at least over 200 new FTEs that are being -- expected to be hired under this agreement with potentially many more from there.

Second, the patient science and engagement section includes several exciting new goals and initiatives. So this agreement would include new staff to expand expertise in assistance on including patient preference information and patient-reported outcomes and product submissions, we hope this staff will be able to assist not only industry partners but also patient organizations who are aiming to collect such data.

Also included within this section is guidance that will be published on using innovative clinic

outcome assessments and device trials. The neuromuscular disease community really needs better endpoints, so this guidance is welcome. Still, COAs don't just appear out of thin air. CDRH and CEBR have a COA qualification program as well as a funding mechanism for the development of COA's so we hope CDRH will work with colleagues and CDRH and CEBR on their COA efforts. But what consider additional ways to further developments of innovative COA's not just inclusion within clinical trials.

Within this section, there's a provision that supports development of new technologies to capture patient experience and input to better structure clinical trials. This could help revolutionize not only device trials but also biologic trials as well. These technologies that allow for remote collection of data are often devices themselves, so CDRH has the opportunity to not only revolutionize trial structures but also the devices necessary to do so.

In this provision, there's a public meeting expansion of staff knowledge and training as well as updating of previous guidances that will all be very welcome.

And then there's additionally a provision on using case examples of successful use of PROs which

is really fantastic, and we hope that CDRH will work with the patient community on what PROs are preferable and what the definition of success is to the community. Not just to industry.

We strongly support the provision intended to tackle lack of diversity in current approaches to patient inclusion. And again we urge the agency to partner with patient communities already working on this very issue.

And then finally under this section also support the creation of patient-facing educational materials for all of these topics. We encourage CDRH to work with all PCC members in doing so.

The next section I want to mention is on real-world evidence. As this section similarly advances device developments and exciting new ways. We support updating previous guidances on real-world evidence, particularly with case examples, and support the furtherance of the real world evidence training program, and encourage CDRH to partner with other medical centers who are also working on real-world evidence and real-world data.

We similarly encourage CDRH when evaluating these efforts in the two or more public meetings that patient focus success measures for these real-world

evidence are also included. Not just success measures under the user fee agreement or for those in industry.

The fourth section I want to mention that I want to comment on is on digital health and we're pleased to see this section within the commitment letter. But we also urge FDA to include patients in the organizations that serve them in these efforts.; As we did not see any mention of the patient community, or patient organizations within the commitment letter as being able to participate within these processes.

Finally, while outside the scope of this commitment letter we still encourage the continued development of policy approaches to resolve our current absence of inadequate regulatory approach to lab-developed tests. As well as the need for re-examination of the incentives to develop humanitarian youth devices or devices intended for knows with rare disease easy.

So in conclusion, we're excited about many of the initiatives included within this agreement and we'll be advocating for its swift enactment in congress later this year. We also see ways in which the implementation of this agreement can be pursued

in partnership with the patient community as already detailed. So thank you again for the opportunity to participate today. Lauren, back to you.

MS. ROTH: Thank you, Paul. And with Paul's comments, we are now at the end of our third portion of the public meeting today. And if we were all live at White Oak as we would have been before the pandemic, this would have been an opportunity for open public comment. The benefits of a virtual approach is that we can get a lost participation from a really wide range across a really wide spectrum of people geographically, they don't have to be in the D.C. area.

The downside, of course, is that we do have to choreograph the open public comment period a bit more than we otherwise would in order to ensure that we have the people available and on the line. So I just want to extend thanks to everyone who registered to participate in this portion of the discussion.

We did have three individuals that we heard from and so I would like to turn it over to those folks to provide their perspectives on medical device reauthorization process and I will start with Robert Durgin. Robert, if you could introduce yourself, and provide your comments now, that would be great.

Welcome.

MR. DURGIN: Good afternoon, Lauren. Hi, everyone, I'm Bob Durgin and I serve as the Vice President for Regulatory Affairs Global Policy for Johnson and Johnson's med-tech sector. I'm here today to express support for the MDUFA V commitment letter agreement. To begin with, given the significant impact to the COVID-19 pandemic on the workload of CDRH, Johnson and Johnson would like to commend the center as well as each individual reviewer and their managers for their tremendous effort in responding to the pandemic for the benefit of patients and healthcare providers. You all did an outstanding job.

With respect to the MDUFA V commitment letter, Johnson and Johnson believes that FDA and the industry have produced an agreement that will further strengthen the medical device premarket review program by ensuring that Johnson believes that FDA and the industry have produced an agreement that will further strengthen the medical device premarket review program by ensuring that the agency has the resources it needs by enhancing review predictability and transparency, and by continuing to ensure that patients have timely access to safe and effective

medical devices.

The user fee program has changed over the years as technologies have advanced and the FDA's needs have evolved. And the performance goal as reflected in the agreement have been honed to meet those evolving needs.

In this agreement, industry has supported significant increases in investment which lead to concomitant increases in the number of personnel that support the premarket review process.

The MDUFA V user fee negotiations have resulted in funding levels that will provide the resources necessary to return to pre-pandemic review performance and continue to support the development of innovative medical devices designed to meet the unmet clinical needs of American patients.

In sum, Johnson and Johnson supports the MDUFA V agreement as reflected in the commitment letter, and thank you for allowing our participation today.

MS. ROTH: Thank you. Next, I'd like to turn to Scott Trevino. Scott, if you could introduce yourself.

MR. TREVINO: Thank you, Lauren. Good afternoon, everybody, and thank you for the opportunity to speak. My name is Scott Trevino and

I'm the Senior Vice President for cybersecurity at TRIMEDX leading independent service organization for medical devices. As well as a founding member of the Alliance for Quality Medical Device Servicing.

I want it start by saying that the alliance is a coalition of five leading independent medical device servicing groups that represent a large segment of the U.S. medical device service industry collectively employing tens of thousands of associates in all 50 states and servicing millions. And maintaining millions of medical devices. So I thank you again for this opportunity. I wanted to express our support for the Medical Device User Fee Amendments. And share that we see this as a critical component to ensuring safety and effectiveness of medical devices in our industry. I additionally, wanted to touch on a couple topics that are also critical to the continued safety and effectiveness of medical devices. Specifically related to service and maintenance of medical devices.

First, the requirement of fair access at a reasonable cost to the materials necessary for service and maintenance of medical devices is a particular importance to the safety and effectiveness of those devices. And as such it's our belief that

the FDA should align with recent FTC statements as well as present Presidential Executive Order on right to repair and do its part to ensure fair access to those materials.

Additionally, as is well known, the healthcare industry's number one targeted sector out of all critical infrastructure for cybersecurity threats and vulnerabilities. However, it's not uncommon that these vulnerabilities and threats to medical devices take a significant amount of time to be addressed and many cases are not addressed at all.

And when available such remediations and mitigating controls are often difficult if not very hard to implement further slowing down the process. We appreciate the FDA's efforts and applaud those our own cybersecurity and would encourage FDA to consider taking more steps to address these challenges.

Finally, I wanted to take few moments to highlight legislation in particular HR7253. This is a bill that redefine remanufacturing. And the bill would circumvent the outgoing FDA process to develop guidance on remanufacturing of the medical devices.

Although this legislation proposes to clarify the issue of remanufacturing, it creates more confusion, and conflicts with current guidance, and

raises following concerns. There's no apparent evidence that an inadequate definition of remanufacturing is a significant threat to patient safety. The definition provided until the legislation is vague and overly broad further confounding the issue of what is and is not remanufacturing.

The Bill is unduly burdensome and places heavy burden on industry participants to prove they are not remanufacturing. And finally fails to provide for fair access at a reasonable price, so the materials needed to comply with provisions. Given this, we encourage the FDA to continue with its existing guidance process and oppose efforts by Congress and industry trade groups to broadly redefine remanufacturing.

In conclusion, I want to say thank you very much for this opportunity to speak. We support the Medical Device User Fee Amendments and strongly encourage FDA to pursue further actions to ensure fair access to service materials, take additional actions to improve cybersecurity and continue with the ongoing guidance process for remanufacturing. Thank you.

MS. ROTH: Thank you, Scott. Maria Gmitro is

our next speaker. Maria, can I turn it over to you?

MS. GMITRO: Yes. Good afternoon. My name is Maria Gmitro and I thank you for the opportunity to speak today. I reacted negatively to a common FDA-approved medical device and my health changed significantly, which is why I choose to be involved in patient and consumer safety. I have no financial conflicts of interest. I am President and Founder of Breast Implant Safety Alliance. Also known as a BISA nonprofit.

Grassroots organization continues the work of issues raised by women doctors and patients for decades in regards to breast implants. Our mission is to raise awareness, optimize outcomes, and ensure informed consent for patients considering breast implant surgery. We empower patients and work collaboratively with all stakeholders including healthcare providers and regulators, and legislature to support system of education and accountability that promote patient safety.

Patient and advocate education is very important to us, so to part us education is to understand how medical devices are approved and regulated. I was thankful to be part of -- I was thankful to be a consumer rep for BISA during the

MDUFA V state quarter consultation meetings and have appreciated the opportunity to learn more about the MDUFA process.

Over the past year attended these meetings and have continued to express my concerns but I'm starting to feel a bit like a broken record. Advocates continue to have concerns about the transparency and accountability involved in this process. The MDUFA draft commitment letter provides the medical device industry seems more influenced than it does to hold the consumer and patients' best interest for protection under safe and effective medical devices.

We don't want to stifle innovation. However, these devices that are harming patients were once deemed safe and effective. In 2019 a class one recall was placed on Allergan Biocell breast implants due to increased risk of cancer. A class one recall is the most urgent and serious of these types of recalls and pertains to defective products that can cause serious health and problems or death.

Even though the cancer caused by textured breast implants has also been linked to all manufacturers, only the Allergan Biocell breast implants have been voluntarily recalled. And the

general public is not aware of it. Now, on October 27th, 2021, the FDA issued a black box warning on breast implants this was to alert the public and healthcare providers of the serious side effects, such as injury or death. However, this information is not reaching the general public.

Over the past two years, I've personally witnessed the impact on patients when a device is deemed no longer safe and effective. And the results have been devastating. We hear from patients that volunteer no idea which devices they have in their body. They're also very upset when they learn that they were not informed that the implants in their body were recalled. And that the cancer can develop years after the device has been removed. The information is not reaching the general public when it does patients lose faith in FDA and the current process in place.

As a key stakeholder, I urge the FDA to strengthen pre-and post-market safety performance measures and surveillance as part of the MDUFA commitment letter.

User fees should be used to strengthen post-market surveillance activities including timely monitor of post-market clinical trials. User fees

should strengthen adverse event monitoring and improve the speed of device recalls.

We will continue to share patient stories and experiences with members of Congress because patients and consumers deserve better and should be protected from devices that are unsafe and ineffective.

The tone of today's meeting seems to be one of positivity and approval and I'm usually very positive but as I speak today, I am thinking of all the patients that have been harmed by breast devices that were once deemed safe and effective. And innovation is exciting, but the inconvenient truth is there's a lacks approval looks process, and this oversight continues to be a cause for concern. Please consider the proposals put forth by Public Citizen in the National Center for Health Research.

And I thank you for the opportunity to speak today and will follow up by submitting additional written comments. Thank you so much.

MS. ROTH: Thank you, Maria. And with that, we are concluded with our presentations by outside stakeholders. And I want to just offer a few concluding thoughts. And the first is by a way of thanks. I know I've said this a few times during the meeting. But I do greatly appreciate all of the

feedback that we have received throughout this process from the inception of the process with the public meeting in October to the monthly stakeholder meetings that we've had over the course of the past year, to the feedback that we've received here today, and that we look forward to receiving in the docket.

I also want to highlight that after today FDA's engagement with the public on aspects of the MDUFA agreement does not end. And we heard that to some extent in the remarks from the National Health Council and others, implementation of the MDUFA agreement is the next phase and equally as important as the negotiation of the agreement itself. And during the implementation phase, there are a number of mechanisms in the draft commitment letter that provide for public engagement.

We've heard about them today, so I won't provide an exhaustive list but rather I want to highlight that the proposals related to pre-submissions, real-world evidence, international harmonization, digital health, and patient science and engagement all include mechanisms for public feedback either on draft guidance's, strategic plans, or other documents.

So we hope that you'll continue to provide your

perspectives, your ideas, your data, and other information through these and other channels to help inform FDA's implementation of our programs and these and other areas.

Thank you again for all of your support, and for your honest feedback, and your diverse perspectives during the meeting and we look forward to continuing to engage with you. And with that, I will say I hope you have a wonderful afternoon. Thank you.

(Whereupon, at 4:00 p.m., the meeting was adjourned.)

C E R T I F I C A T E

This is to certify that the attached proceedings in the matter of:

PUBLIC MEETING - MEDICAL DEVICE USER FEE AMENDMENTS FOR FISCAL YEARS 2023 THROUGH 2027

April 19, 2022

Via Microsoft Teams Videoconference

were held as herein appears, and that this is the original transcription thereof for the files of the Food and Drug Administration, Center for Devices and Radiological Health, Medical Devices Advisory Committee.

_____

KIMMBERLY ALLEN

Official Reporter

CERTIFICATE OF TRANSCRIPTIONIST

I, MELLONEE MCDONALD, a transcriptionist located in BLOOMINGDALE, OHIO, hereby certify:

That the foregoing is a complete and accurate transcript of the digital audio recording of the proceeding in the above-entitled matter, all to the best of my skills and ability.

I further certify that I am not related to any of the parties to this action by blood or marriage and that I am in no way interested in the outcome of this matter.

IN WITNESS THEREOF, I have hereunto set my hand this 24th day of April, 2022.

_____
Mellonee McDonald