

**UNITED STATES DEPARTMENT OF HEALTH AND  
HUMAN SERVICES FOOD AND DRUG ADMINISTRATION**

**PATIENT ENGAGEMENT & REGENERATIVE MEDICINE:  
AN FDA CBER WORKSHOP FOR PATIENT ADVOCATES**

**Via Adobe Link  
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## **PARTICIPANTS**

Welcome

- ANNE ROWZEE, PhD  
Associate Director for Policy  
Office of Tissues and Advanced Therapies (OTAT)  
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FDA

How FDA Is Advancing Regenerative Medicine

- RACHAEL ANATOL, PhD  
Deputy Director  
OTAT  
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The Value of the Patient Perspective in Regenerative Medicine Therapy Development: Case Studies and Panel Discussion

- ANNE ROWZEE, PhD  
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- ROB ADLER  
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#### How to Engage with FDA When Opportunities Arise: FDA Panel Discussion

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#### Closing Remarks/Q&A

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## PROCEEDINGS

(11:00 a.m.) DR. ROWZEE: Patient Engagement & Regenerative Medicine: An FDA CBER Workshop for Patient Advocates. Today's workshop is hosted by OTAT, otherwise known as the Office of Tissues and Advanced Therapies, within the Center for Biologics Evaluation and Research, also known as CBER, at the U.S. Food and Drug Administration. We truly appreciate your attendance at today's workshop and the opportunity to talk with patients, their advocates, caregivers, and others to discuss the ways in which we can work together to help advance regenerative medicine therapies.

I'm Dr. Anne Rowzee, Associate Director for Policy at OTAT, and my focus is on stakeholder outreach and engagement. I'll also be your moderator for today's event. And just a few notes about today's exciting agenda: We have panel discussions that include patients and advocates as well as FDA staff. We're going to kick off today's meeting with a brief introduction to OTAT and our work as it relates to regenerative medicine. Then we'll move into a panel discussion featuring patients, patient advocates, and representatives from patient organizations. We're going to hear about their experiences working directly with OTAT. We'll host a discussion about how we can enhance future interactions to ensure that the patient voice is included in discussions about gene and cell therapy research and treatment.

After a break, we'll move into a second panel discussion which will feature patient engagement leaders from across the FDA. And we'll talk about various opportunities available for patients and advocates to work with the 17 agencies. And lastly, we'll end the day with a Q&A with some of OTAT's patient engagement leads to address any additional questions or comments. Before we get started, I'd like to share a few notes about today's workshop. The workshop is being recorded and the recording will be posted on the website — on FDA's website. We'll have a couple of different panel discussions today and opportunities for questions.

Please use the Q&A function if you'd like to share a question. We also have a chat box that you can use to share your general comments. We appreciate questions and comments, and we'll do our best to address as many as we can. For any technical difficulties, please use the chat function and someone will assist you.

So let's go ahead and get started. It's my pleasure to introduce our first speaker, Dr. Rachael Anatol. She's OTAT's Deputy Director. Rachael will be providing an overview of OTAT's ongoing work to regulate and help advance regenerative medicine therapy and also cover some of the ways that OTAT works with patients and their advocates. Rachael, thank you so much for joining us today. I'm going to turn it over to you.

DR. ANATOL: Hi. Good morning, everybody. And thank you, Anne. I'm delighted to begin our scheduled presentations today, with an introduction to CBER and how we are working to advance the development of regenerative medicine products. So, in this session, I'll give you a brief overview on what FDA does as an agency, share some information about FDA — about — sorry — OTAT's mission, provide some background information about regenerative medicine, including what these therapies entail and the types of therapies that have been approved to date, as well as FDA's role in helping to advance these products. And finally, I'll describe patient engagement activities at CBER OTAT which, of course, is the focus of today's workshop.

I'm sorry. I turned on my webcam but did not hit "Start Sharing." So I apologize for not being on video for that first introduction.

So, as one of the leading regulatory agencies in the United States, FDA has many responsibilities. Our mission is to ensure that drugs, biological products, and medical devices are safe, effective, and secure for the public's health. Beyond approving prescription and over-the-counter drugs and therapies, FDA is also responsible for monitoring drugs and therapies that are already on the market in the event that there are any adverse reactions, ensuring that clinical trials are safe and effective for patients to participate in those trials, inspecting facilities to ensure they meet our robust manufacturing standards, and working with patients, stakeholder groups, and other important audiences to understand patient needs and find innovative approaches in treatments for patients. There's more, but in the interest of time, I'm going to move on.

So FDA has nine center-level organizations and 13 headquarter offices. Within that organization, the Center for Biologics Evaluation and Research, known as CBER, regulates a wide range of biological products and conducts renowned scientific research. So biologics represent a diverse category of products that includes vaccines, blood and blood components, allergenics, cellular therapies, gene therapies, and human tissues. Biological products are generally large and complex molecules made from living entities, such as cells and tissues, which make them different from conventional drugs — something like aspirin, for example — which are typically small molecules made from chemicals. CBER's role is to ensure the safety, purity, potency, and effectiveness of biological products used to treat, diagnose, or prevent human diseases and conditions.

CBER is comprised of the Office of the Director and seven program offices. The Office of the Director provides leadership and policy direction to the program offices and coordinates center activities and resource management. And the director of CBER is Dr. Peter Marks. Four offices in CBER provide crosscutting support in areas of compliance; surveillance; epidemiology and biostatistics; communication, outreach, and development; and management budget and administrative services. The Office of Tissues and Advanced Therapies, which you see in red, or OTAT, is one of three product offices. OTAT's mission is to promote the public health through a collaborative and data-driven process to ensure that medical products are safe and effective.

In doing so, OTAT strives to lead all regulatory decisions based on data and with impartiality and compassion. OTAT regulates a wide variety of products, and those will be shown on this slide and the next. So cell and gene therapy-related research and development in the United States is growing and continues to grow at a fast rate, with numerous types of products advancing in clinical development. In just a minute I'll describe some of these products in more detail. OTAT also regulates xenotransplantation products, therapeutic vaccines, blood- and plasma-derived products, antivenin, human tissues intended for transplantation, and certain devices. So I think, as you can see, we really run the gamut of products in this office.

So OTAT regulates regenerative medicine therapies, sometimes referred to as RMTs, and this class of products encompasses some of the different product types you saw in the previous two slides. In 2016, the 21st Century Cures Act was signed into law, and four sections of this law focused on regenerative medicine therapies. In one of the sections, the law described that these products include cell therapies, therapeutic tissue engineering products, and human cell and tissue products and combinations of such products. FDA took a look at the law and developed guidance where we explained that we also consider gene therapies that lead to a sustained effect on cells or tissues, and some products for xenotransplantation, to also meet the definition of regenerative medicine therapy. So this slide shows a few examples of products that would meet the definition of regenerative medicine therapies, and I will go into them in more detail in the next couple of slides. FDA describes cell therapy products as autologous cells, meaning

cells that are your own, allogeneic cells, meaning cells that come from someone else, or xenogeneic cells that are intended to treat or prevent a disease or condition.

Examples of cell therapy products include stem cells and stem cell-derived products, pancreatic islets, and other types of mature or differentiated cells, such as chondrocytes and keratinocytes. FDA does not have a formal definition of “tissue-engineered products,” but we can think of these products as those products that treat a disease or condition and are, you know, something like a scaffold with cells on it that are combined, and that product elicits its effect. Human gene therapy products have been defined by FDA as products that mediate their effects by transcription or translation of transferred genetic material or by specifically altering host genetic sequences. Some common types of gene therapy products include vectored products, genetically modified cells, and products incorporating genome editing. So gene therapy products are sometimes distinguished from each other by using terms such as “directly administered” versus “ex vivo modified gene therapies.” So directly administered gene therapies, sometimes referred to as “in vivo gene therapies” — in this type of product, the gene therapy is administered directly to a patient to elicit its effect.

A real-life example of this type of gene therapy is Luxturna, which is used to treat children and adult patients with an inherited form of vision loss that results from a mutation in a specific gene called *RPE65*. Luxturna consists of a vector which is really a vehicle that carries a transgene which then delivers a normal copy of the *RPE65* gene directly to retinal cells. The cells then produce the normal protein to help restore their vision loss. This is different from ex vivo modified gene therapies. In this type of product, a patient’s cells are removed from the body and then genetically modified outside of the body before they are readministered to the patient.

One example of this type of gene therapy are CAR-T products. In CAR-T gene therapy, a patient’s own T cells are removed from the body and then genetically modified to express a protein, known as a CAR, on the surface of those T cells. This CAR protein recognizes surface markers on cancer cells and directs the T cells to target and kill those cancer cells.

So this slide includes a list of our approved cell and gene therapy products. In the interest of time, I’m not going to go through this slide. When we get to the end of this presentation, there will be a slide for resources where you can find links to each of these products. But I wanted to point out that Abecma, Breyanzi, Kymriah, Tecartus, and Yescarta are ex vivo genetically modified products; Luxturna and Zolgensma are directly administered gene therapies; HPC–cord blood, laViv, and Provenge are cell therapies; and Gintuit and Maci, which consist of cells on scaffolds, fall under the umbrella of tissue-engineered products.

So I would now like to turn to a brief but high-level overview of product development. So this figure provides an overview of the product development process and the major stages of bringing medical products to market. What is important to know is that FDA and OTAT are involved at each stage, from early on in the development through the clinical trial phases and after products are approved and in the marketplace.

So this slide illustrates the standard approach to clinical trials for development of conventional drugs. So you’ll see in this model that Phase 1 and Phase 2 are Exploratory. Phase 1 studies of conventional drugs typically are performed in a small number of healthy adults to assess safety, and that data guides dosing and monitoring for the Phase 2 trial. Phase 2 studies involve more patients and primarily assess the magnitude of the effects on particular endpoints.

This data will guide the design of the Phase 3 trials, which are sometimes called Confirmatory trials. The Phase 3 trials involve many more patients and gather evidence on safety and effectiveness on a much larger scale to support marketing applications. Cell and gene therapy products and other regenerative medicine therapies are not conventional drugs and are often used to treat rare and even ultra-rare diseases. Because of this, the early-phase studies often looked at effectiveness which can support licensure as well as safety and typically include patients affected with a disease or condition rather than healthy subjects. OTAT encourages manufacturers of these products to come to talk to us at the beginning of their development programs, because these early-phase trials are so important.

So FDA has several roles in regulating regenerative medicine therapies, and I'll describe just a few of them here. As CBER-regulated products and technologies become increasingly diverse, complex, and sophisticated, we must continue to develop and adapt our approaches to evaluate them. We do this through various means, including our scientific research and through regulatory staff attending scientific meetings. And we promote development by working closely and interactively with product manufacturers, we issue guidance to reach a large number of stakeholders at once, and we develop flexible regulatory frameworks and policies and adapt our approaches as needed. Finally, stakeholder engagement is an equally important aspect of CBER's work. We collaborate and communicate with patients and caregivers and with product and technology developers to solicit input and offer support.

Over the next few slides, I will talk more about what we have done to engage the patient community. So activities that involve patient stakeholders sharing their experiences, perspectives, needs, and priorities help inform FDA's public health mission. This is because patients provide an important and unique perspective that is critical for consideration as part of the regulatory process. Our goal for patient engagement is to learn directly from patients and the patient advocacy community. We have heard from patients regarding the impact of disease and treatment on their daily lives, we've heard about what might be burdensome during participation in a clinical trial, and we have heard what types of risks patients are willing to tolerate and which they are not.

I'm going to spend some time on this slide, which covers some of the activities OTAT conducts to gather input from patients. So advisory committee meetings are most often open to the public to discuss either a license application or regulatory or policy questions. In this type of meeting, a group of experts are posed questions about the meeting topic, they listen to the information, and make a recommendation to the FDA. Special government employees, or SGEs, serve as voting members, and the committee can also include a patient representative. Patients and other members of the public can also provide input at these meetings during the public comment period. So SGEs can also provide input at the request of FDA review staff, and this can include patients and caregivers, which is referred to as the Patient Representative Program. Patient SGEs may be asked to provide their perspective on drug development, such as how meaningful a proposed endpoint might be. Public meetings and workshops are open to the public. Patients and representatives from patient organizations are often speakers and panelists at these meetings. Some recent examples include FDA's Rare Disease Day, FDA's public meeting on advancing health equity for diabetes and chronic kidney disease, and CBER's workshop on facilitating end-to-end development of individualized therapeutics.

So patient-focused drug development, or PFDD meetings, are exactly what they sound like: meetings to engage patients and elicit their perspective on two topic areas — first, the most significant symptoms of their condition and the impact of the condition on daily life; and second, their current approaches to treatment. Each PFDD meeting is tailored to the needs of the



specific disease area, taking into consideration the current state of drug development for the disease and the needs of the patient population and the FDA. In these meetings, panels are comprised entirely of patients or caregivers. There are patients in the audience, on webcasts, and there is live polling of meeting participants. FDA and other stakeholders, such as health care providers, drug developers, and researchers are there to listen. After the meetings, there is a comment period and a “Voice of the Patient” summary, and the meeting is recorded and a transcript is shared. This effort began in earnest at FDA in 2012, and there have been 30 FDA-led Patient-Focused Drug Development meetings. In addition, there have been more than 30 externally led Patient-Focused Drug Development meetings, which means they are led by patient organizations.

So the FDA/NORD Rare Disease Listening Sessions are another type of meeting, and there have been 29 since 2018. Through these meetings, rare disease patients, caregivers, and advocates convene to enhance FDA’s clinical and regulatory understanding of diseases and conditions and provide a common understanding of the most urgent needs of patients, caregivers, and advocates. Patients or FDA staff can request these meetings. FDA uses these meetings to hear directly from patients about what outcomes are important, learn more about how patients weigh the benefits or risks of potential treatments, motivations, and barriers to participating in a clinical trial.

Sometimes patient groups are looking for specific input, and patient organizations develop surveys, natural history studies, or conduct research to help move drug development forward. And FDA is more than happy to talk to them about their work. We are interested in providing patient organizations with information for them to consider that will help set up a drug development program and help set it up for success and provide these groups with information to help them be engaged through the drug development process.

One of the best ways to align regulatory needs, patient expectations, and sponsor responsibilities is to be transparent and enhance collaboration. To help foster transparency, sponsors can invite patients and their advocates to formal meetings with FDA. OTAT generally encourages this, as it often gives us the opportunity to hear the patient voice directly and gives patients a clearer view of OTAT’s concerns. Also, many patients may not have a good understanding of FDA processes, and participation in these meetings can make the process more approachable.

Patient groups might also work together to take on activities that advance the science in their particular disease area. For example, patient groups have approached the agency via the Critical Path Innovation Meeting mechanism to discuss their protocols for natural history study designs. Patient groups could also consider working with sponsors and academics to design drug development qualification tools which could help support acceptance of new clinical outcome assessments, such as patient-reported outcomes.

As I mentioned earlier, many of OTAT’s INDs are for rare diseases, sometimes very rare or ultrarare. Often, we are approached by several different patient groups, all representing the same small patient population. And when we can, we encourage these groups not only to work together but also to meet with us together as another means to support transparency and alignment among our stakeholders. Making sure that everyone is hearing the same messaging at the same time is critically important.

And finally, we recommend that you begin collaboration early. We encourage sponsors, academics, and all of our stakeholders to bring patients into the fold early in the process, whatever the project is. The patient perspective is unique, and it is valuable.

So today's workshop is another opportunity to learn from the patient community. Our hope is that you walk away feeling confident in FDA's commitment to understanding the patient perspective and learn about the opportunities available to engage with us. The advances made in regenerative medicine thus far point to a promising future. As the field evolves, FDA will continue to rely on stakeholders across the patient community, academia, and industry to ensure that medical products are innovative and beneficial to those affected by genetic and rare diseases. So I and everyone here from FDA is very grateful for your participation in today's workshop.

Here are a few ways on this slide you can keep in touch with us. Because of the pandemic, of course, I recommend email over phone, and some of these resources are valuable and really contain a lot of information. And you can go to our CBER website to find out more about the approved cell and gene therapy products I mentioned earlier. So thank you very much.

DR. ROWZEE: Rachael, thank you so much. Your presentation has really helped set the stage for our workshop today, and it's made us ready to hear more about OTAT's patient engagement activities. So we're going to take a short break now so we can get our panelists on board for our first panel discussion at 11:35. So we'll see everybody back here in a few minutes. Thanks again.

(Recess)

DR. ROWZEE: All right. So welcome back, everyone. We're going to now move into our first panel discussion: the value of the patient perspective in regenerative medicine therapy development. During this panel, you're going to hear from patients, patient groups, and OTAT staff about their experiences working together and how patients and their advocates can participate in the regulatory process to help advance gene and cell therapies, research, and treatments.

We're going to feature two case studies today. The first is a recent meeting between OTAT clinical staff and the Loulou Foundation. This meeting was held to discuss a question from the foundation about the design of an observational study under development and how the data from this study might be used in the design of future clinical trials of gene therapy for CDKL5 deficiency disorder. The second case study is on an FDA-requested patient listening session. You heard a little bit about patient listening sessions from Rachael earlier, and you're going to hear a little bit more about them during our Session No. 3. But I wanted to point out, in this case study, it was FDA who had the questions for the patients, as the listening session was arranged to ask patients with glycogen storage disease, type I, about their tolerance for risk in participating in clinical trials for regenerative medicine therapies and what types of benefits from therapies patients would consider to be meaningful.

I am delighted to introduce our panelists for the first case study. I'd like to introduce Dr. Xavier Liogier — he's Chief of Translational Science at the Loulou Foundation — and Dr. Lei Xu. She's Chief of General Medicine, Branch 2, from the Division of Clinical Evaluation and Pharmacology/Toxicology within OTAT. For the second case study, we have three participants: Dr. Elizabeth Hart, Chief of General Medicine, Branch 1, also in OTAT; Debbie Drell, Director of Membership for the National Organization for Rare Disorders, or NORD, as most of you know

them; and Rob Adler, a patient who participated in the GSD1 listening session. Thank you all so much for taking time to join us today. We look forward to hearing your thoughts.

Let's get started. Xavier, we didn't get a chance to hear your voice during our check. So I just want to make sure that everybody can hear you.

DR. LIOGIER D'ARDHUY: Okay. Can you hear me?

DR. ROWZEE: I can hear and see you. So, I'm going to let you —

DR. LIOGIER D'ARDHUY: Okay.

DR. ROWZEE: — go ahead and take over. I'm going to get you your first slide, and go ahead.

DR. LIOGIER D'ARDHUY: Thank you very much. Okay. So I'm Xavier Liogier, Chief of Translational Science at the Loulou Foundation. I'm really honored to be able to share my experience. So I thank you, the organizer. And also, I'm really proud to represent the Loulou Foundation today.

So let's start with Loulou. Loulou is a very cute little girl who just turned 7. Loulou suffers from CDKL5 deficiency disorder, or CDD, and her parents, Majid and Lynn Jafar, created the Loulou Foundation to support research. So of course, the first year, several years ago — this was mainly to support the technical research and so building the network of researchers able to identify the best way to develop a potential treatment in future. But now, today, we are approaching the clinical phase, and potentially in a year or two — and that's why I have prepared several slides to set the scene and explain why we really needed the first introduction with the FDA in the context of the critical path innovation meeting.

A few words on the disease: CDD, CDKL5 deficiency disorder, is a rare condition, estimated to 1 in 42,000 births, but it's probably underestimated, because it has been recently diagnosed or characterized. And if you will, for instance, the ICD-10 nomenclature has been issued in October last year, so very recently. CDD is a unique phenotype, showing two main devastating problems: severe epilepsy, which is very hard to treat and to control with typical antiepileptic drugs, but also global and profound developmental delays. Besides these core symptoms, if I may describe it this way, we have also characterized and described multiple cooperating conditions, such as gastrointestinal disorders, low muscle tones, and visual impairments. The visual impairments are really related — mostly related to cortical visual impairments and less on the vision itself. So that's the difficulties and issues in evaluating the severity of these cortical visual impairments and also autonomic dysfunctions.

So, today, what are the treatments? Well, there is a huge, unmet medical need here, because the anti-seizure drugs are of limited efficacy. If they are (Inaudible) that the defect doesn't last too long and we need to change and switch to another antiepileptic drug and sometimes to play and find the right dosage to be used. And this is — without speaking about the toxic aspects — many impacting the cognitive aspects — processing speed, attention level, of course language, and communications. Other strategies — diets, such as the ketogenic diet, but also more invasive strategies, such as vagus nerve stimulation, or even corpus callosotomy. Okay.

Now that we know a bit more on the disease itself, the whole purpose was to find and identify the best strategy to reach the clinical phase as soon as possible. And basically, it means to develop the field before developing a therapy. And as most of the rare diseases, everything

starts from the parents, and the patient community needs to be built. And once it's done, of course, you need to liaise and build a network for academic research and funding and to be able to create the momentum and to make sure that, you know, everything is done to find the right strategy, the right molecule, or the right — here in this context — genetic strategies on the practical level. But now that we are approaching the clinical level, we need also to identify the right tools to evaluate potential effective gene therapies.

Tools means scales, questionnaires that you may have been, you know, involved in or are testing yourself. And we need to show and demonstrate that they are sensitive enough that they are validated for the condition, and all these aspects are key for development. And that's why the foundation decided to try building a consortium of different companies but also academic experts and starting with a non-direct study, what we call an observational study, for a follow-up over maybe 2 or 3 years and try to identify the trajectories of some domains impaired in the conditions and to test scales that are already known which has been validated in other conditions and similar conditions and therefore, at the end of these types of studies, being able to say, yes, we could use these scales or this questionnaire for this condition and clinical trial settings.

So, in this context, the critical path innovation meeting was identified as the best way to first start an interaction with the FDA officers to identify maybe improvement in our study design but also having the opportunity to discuss the scales itself, the choice of the scales, and for that study specifically. And other questions that will display in subsequent slides are the topics that we wanted to tackle with the officers.

Prior to that, I have to mention that one key — first interaction with the health authorities and the FDA was the externally led patient-focused drug development meeting, PFDD, which is basically the first time that maybe the rare condition, the rare disease is shared and displayed — explained to the FDA officers, but not only because it's about educating the FDA on the disease itself but also potentially the industry and the pharma industry. This is a big meeting where the families are invited and patient advocacy groups who share their daily experience and challenges with the disease itself and their kids. It's also a way to tackle or identify the most important domains that — or potential threat. It needs to focus as a priority, for instance, but here, maybe developing a bit more on the critical path innovation meeting.

In my recent experience, we wanted to discuss the clinical outcome assessment choice and whether, you know, these scales made sense to be implemented in future clinical trials and first in this observational study, but also one question specifically on the safety aspects and maybe the poor occurring conditions, because in future clinical trials involving gene therapies, we thought that maybe we want to minimize the number of kids exposed to the placebo. So, in this context, we wanted to discuss with the FDA officers whether this type of observational study and study data collected through that study could help minimize the exposure of these potential participants in future clinical trials involving gene therapies, for instance, with invasive administrations. And that's why that was really critical to have these — I have to stop here, I guess — these critical path innovation meetings and these first interactions. And I will probably expand a bit more on the — I don't know why it's moving on its own. But, anyway, in the Q&A section I would be more than willing to share my experience — how we interacted with the FDA officers in this context and also the difference I experienced myself, because I come from the pharma industry after, let's say, 20 years, and our development for neurodevelopmental disorders. And the interactions with the FDA was slightly different, and, yeah, I can explain how we, you know, are representing the foundation, how we interacted with the FDA in this context.

With this, I will stop. I think I have my 10 minutes over. Thank you very much, and I'll hand it over to the next speaker.

DR. ROWZEE: Yeah, hi, Xavier, this is Anne. I'm so sorry. I'm sorry about that.

DR. LIOGIER D'ARDHUY: No worries.

DR. ROWZEE: Yeah, I don't know what happened, either. No one was trying to rush you along. But, like you said, it's really important to know that you have experience sort of in various roles, and how that's different and how sometimes those interactions can be different. So we'll look forward to talking to you a little bit more in the Q&A section.

And now, I'm going to hand it over to Dr. Lei Xu who was participating in this meeting with the Loulou Foundation and let her provide some opening remarks. Thanks, Lei.

DR. XU: Thank you, and good morning everybody. Thank you, Anne, and thank you, Xavier, for the very nice introduction and overview. So, from my perspective, I just feel the interaction we have had so far with the Loulou Foundation has been very helpful, and I'm grateful to be part of the team.

So I think part of FDA's mission is to advance the public health by helping to speed the innovation to make the medical products safer and more effective. And here we are faced with a disease which is very serious with very limited treatment options. So I think, as Xavier pointed out, the patient-focused drug development meeting that was held, I think, almost 2 or 3 years ago was really an educational process for us as the reviewers, because we really heard something from caregivers' perspectives about how the worse symptoms are most significant to those patients and their loved ones and to the caregivers and how the condition has impacted their day-to-day life. And we also have a much better understanding of currently available treatment for those patients. And so, I think this is really important when we review all the files when we make the benefit risk assessment even from the first inhuman study and to have a more, I think, thorough view about this assessment. And then later last year, I also attended this critical path meeting with the focus on the clinical outcome assessment and also on the natural history of the observational study and in this patient population. And again, I think that's important from the drug development perspective, because I feel that for a product, especially for those rare conditions to have a more efficient development process — I think the patients and the patient advocacy groups and the sponsors, the scientists, and the regulatory bodies — we really need to work closely so that the development process can be more efficient and that the collaborative type of work should start from during the preparation stage. That's when the early collaboration between the basic scientists and the clinicians will start, and that also means we should think about a natural history study or observational study, especially for the conditions we have a really limited understanding of the disease and the disease progression. And so that will help for later, a study design and also for the potential outcomes which can be used for those later-phase — not later-phase but for the later study to evaluate both the safety and efficacy of the investigational product.

So I think that the critical path meeting served that purpose. Then, in the follow-up discussion between the Loulou Foundation and OTAT, I think it's more specific to the product that we regulate. And I think we, as a review team, after hearing the Loulou Foundation's perspective and their plans — I think we just feel it is helpful to provide more detailed feedback regarding the observational study and also what we think the most appropriate views of the findings from these studies. And also, we think it is helpful to provide some general comments regarding the

design of the trials or the studies to evaluate the safety and efficacy of the investigational product.

So, in summary, I mean, I really appreciate the opportunity to work with the Loulou Foundation, and I really appreciate the knowledge I have obtained from the patients and their families. And I am pretty sure the collaboration will continue. Thank you.

DR. ROWZEE: Thank you, Lei, Lei and Xavier, both of you for sharing your thoughts and perspectives on this important meeting. I think what everybody could hear is, start early, you know, begin that collaboration — begin those conversations early.

So I'm going to move over now to our next case study, but I just want everybody to remember: Please enter your questions into the Q&A box. And you know, we'll have questions for both of our case studies. We'll follow at the end — a Q&A session at the end there.

So I'm going to now hand it over to Dr. Elizabeth Hart, and she's going to share her remarks on her perspective on the recent GSD1 listening session. So, Elizabeth, I'll hand it over to you now.

DR. HART: Good morning and thank you. As you've already heard, the patient voice is critical to what we do. One key aspect of our mission is to advance the public health by helping to seed innovations and make medical products more effective and safer and by helping the public get the accurate science-based information that they need to use medical products to maintain and improve their health. When we look at approving new therapies, we want to make sure that they are safe and effective. Specifically, our regulations require that for traditional approval, adequate and well-controlled studies demonstrate effectiveness on a clinically meaningful endpoint. From a regulatory perspective, we consider clinically meaningful endpoints to be one set of facts the way patients feel, function, or survive. Obviously, patients are best positioned to tell us about this. Therefore, to accomplish this, it is very important that we partner —

(Audio skip)

DR. HART: Sorry, we seem to be having some technical issues, but maybe they'll be able to fix it on their end, and I will continue. Can you hear me? Thumbs up? Something?

DR. ROWZEE: Hi, Elizabeth. This is Anne. I can hear you. I can't see you. So if you have a chance to set your webcam, go ahead and do that.

DR. HART: It started on my end. So if —

DR. ROWZEE: Okay.

DR. HART: — whoever's doing technical could help fix that, that would be great.

DR. ROWZEE: Perfect. I'll hand it back over to you. Thanks.

DR. HART: Great. Sorry about that. So as I was saying, patients are best positioned to tell us about their perspectives. So it's very important that we partner with these stakeholders. People living with a condition and their caregivers are uniquely positioned to inform our understanding of the therapeutic context for drug development and evaluation. This is especially important in the rare disease phase, where less may be known and understood about the condition and what is important to patients. And by the nature of these being rare diseases, there are fewer

patients. So each study must be carefully designed to ensure interpretability of each patient's data.

One way that we have an opportunity to hear the patient voice is through listening sessions. These are small, informal, non-public opportunities for FDA staff to hear firsthand from patients and their caregivers. Our office recently held two sessions focused on glycogen storage disease 1A, which I will subsequently refer to as "GSD1A." One included adults and their caretakers, and one included caretakers (a.k.a. parents) of children with GSD1A. We collaborated with NORD and the Reagan-Udall Foundation. As you've heard, our office regulates gene therapy amongst other products. And we have recently seen increased product development in this disease phase. Therefore, the focus of the sessions were to gain a better understanding of GSD1A and specifically the patient caretaker perspective on the burden of disease and its various symptoms and how this has changed over time. We wanted to hear about how patients and their caretakers managed and prevented the low blood sugar or hyperglycemia associated with the disease and the burden associated with the current available therapies. We also wanted to discuss risk tolerance and their consideration with respect to participating in clinical trials, especially for gene therapy, and the considerations of risks and benefits that would make this worthwhile. Prior to the session, members of the GSD community, who might be interested in participating in the listening session, answered many questions about their experience. We had an unprecedented number of responses. This data was exceptionally informative, and there will be an opportunity for follow-up with these patients and caretakers who were unable to participate in the listening session.

For the listening sessions themselves, as I mentioned, one focused on adults and one focused on children. For the adult session, we had six participants, five patients, and one caretaker, with the patients ranging from 18 to 47 years, all with moderately severe to very severe GSD1A. For the listening session focused on children, there were seven caretakers, some of whom had multiple affected children. The children ranged in age from 4 years to 19 years. Most described the severity of the GSD1A as moderate or moderately severe and one as mild.

Each participant had a unique perspective, but there were certain themes that emerged. One theme was how managing GSD1A was a constant responsibility that impacted every aspect of their life. There was fear about low blood sugars and hospitalization. There were comments about how current therapies for prevention of hypoglycemia, cornstarch, and glycoside required constant attention and dramatically impacted quality of life. Participants described how this impacted their social life, including school, attending college, dating. The list goes on and on. They also described issues related to the risk that they were willing to take based on their own experiences. This was all very, very helpful information for us, as it allows us to better understand patient preferences and advise sponsors on clinical trial designs.

For those of you interested in learning more details about the session after today's session and case study, you can go to the website and see a summary. So you can click on the link "Patient Listening Session Summary" under "Learn About FDA Patient Engagement Site," and the full link will be provided as well. Listening sessions represent one way that we can hear from the patient community, but as you have already heard and will continue to hear, there are many opportunities for collaboration with the patient community.

We recognize that patients with the same condition often have different perspectives, and we heard this during this listening session. And hearing these different perspectives better informs us. The patient voice is essential to what we do. It is more efficient to work together, and we are

so appreciative to have the opportunity to partner with patients and their caretakers and advocates.

Before I hand this over to Debbie, I just want to thank everyone from the GSD community who participated in these listening sessions and everyone in the GSD community who has taken the time to provide us with written feedback. Thank you.

DR. ROWZEE: Thanks, Elizabeth. I think I'm going to pass it right over to Debbie now to present the perspective from NORD, and then she'll hand it over to Rob to hear directly from patients. Thanks, Debbie.

MS. DRELL: Thanks so much for having me. Can I be heard?

DR. ROWZEE: I can hear you, not quite see you yet.

MS. DRELL: Okay. How about now?

DR. ROWZEE: Not quite yet.

MS. DRELL: I'm not sure — okay. Do I need to click on "Start Sharing"?

DR. ROWZEE: Yes, that's correct.

MS. DRELL: Okay. All right.

DR. ROWZEE: Perfect. We can see you. Thanks so much.

MS. DRELL: Excellent. Thank you so much for having me, and it's a pleasure to be here speaking on behalf of the National Organization for Rare Disorders. I'm Debbie Drell, the Director of Membership at NORD, which is a nearly 40-year-old nonprofit umbrella organization who serves 25 million Americans living with rare diseases across all 7,000 rare diseases. We're a little busy. I specifically work to support NORD's network of 330 member organizations as well as a total of 1,200 nonprofits involved in rare diseases. My team and membership even help start nonprofits through a program called RareLaunch if there are no nonprofits in your rare disease.

I see so many people in this meeting. There's 257 people right now, and I just want to say hello to the audience for being here. I see choroideremia advocates, SynGAP advocates, hemophilia, leukodystrophy, GSD (glycogen storage disorder) groups, FARA, pemphigus, pemphigoid. Just saying your name brings a smile and knowledge that, as leaders, we're taking time to learn and to network and connect. And while this is a virtual meeting and we miss seeing each other face to face, I just want to give shout-outs to some of the diseases that I recognize, just quickly scanning — just to help with networking and also to share your rare disease name on this webinar. So thank you for being here and everyone else and anyone that I missed.

This workshop is specifically intended for rare advocates, and so, with this in mind, my talk is really focused on how rare advocates get involved in engaging FDA. And as an advocate — let me advance this slide — a little clumsy in Adobe, but please forgive me. As an advocate, you know that there are many in the community with varying knowledge and understanding of research in their rare disease field. Many patients and caregivers don't even know how to get involved in research and may not be aware of all the treatment options. Patients and caregivers



who do know may have questions about the therapies that are available. They may not know all the therapies that are available, or they may only have their limited experience. And then there are many that have strong opinions on their treatment. Perhaps they're open to gene therapy. Maybe they're open to it but hesitant unless it's administered in a certain way. And they want to know more about the benefits. So, as a patient advocate, you know, the first thing you can do in a nonprofit leadership is to educate, inform, and help patients understand what all the options are in participating in research of your natural history studies or clinical trials. And so, that's just sort of foundational.

Your community has diverse experiences, and engaging FDA allows FDA to understand what their perspectives are. And as previous speakers have mentioned, the GSD1 summary notes are listed on the FDA's webpage, per the listening session. But we've done 13 listening sessions with FDA that are FDA-initiated through our memorandum of understanding — NORD and FDA working together. So those summaries are online, and I encourage you to take a look at them, because they ask questions like this one: "As you may know, some clinical trials involve different types of medical products. For example, some involve an experimental drug and others involve gene therapy. If you were thinking about taking part in a clinical trial, would it make a difference to you if the trial involved an experimental drug or a gene therapy?" This is literally a question that was asked in a listening session. And I mention this question because, if you had all of the same types of people, maybe city dwellers who are of a particular gender and age, all patients, you would only get — you might get the same over and over again. But getting diversity of demographic information in the participants and looking at diverse experiences and disease severities, adult caregivers, patients, young patients who grew up with the disease and are now adults — there are so many different experiences and those experiences allow FDA to understand the spectrum of opinions on risk-benefit for new treatments and research.

And so, listening sessions are private. They're 90 minutes long. They're non-recorded. They're all high-level notes, but you don't know which individual patient, you know, said one way or the other on their opinion. So it's a very safe space to share. And we really want to find the right combination and diversity of perspective invaluable.

And so, I'll talk just a little bit about how FDA finds those participants and prepares them and, like with — previously, the GSD listening session received the largest number of speaker interest, 75. So, elephant in the room — we're living in an era — if you can see the slide — a little bit small — the pandemic drags on, and the trust in government decreases, for a number of reasons — political, populism in politics, around the world. But if I had a way of looking — and I'm sure this exists — FDA's being in the news 2019 versus being in the news in 2020 versus 2021 — that would be incrementally increasing, because FDA's had a huge role in the pandemic as well as just government in general in managing this crisis. And as a result, in the public eye, there has been some criticism, and so, when we think about trusting government, you're looking for patients. And if they're going to get a call from FDA or an email, one, they might not believe that it truly is FDA knocking on their door, and two, they might not trust FDA. Many people do, and patient advocates do, but our community is so diverse. And we don't want to exclude anybody regardless of how they feel about the current sentiment that we can't control. We just want to be able to extend our hand and our heart and bring them in as equal partners, everyone. Diversity means inclusion of all experiences and all perspectives. What's more is that, during the pandemic, we've been bombarded with messages about being careful when you get an email from the government, because there are scammers, people talking about the COVID relief package, trying to get your bank account information, or scammers on vaccines and testing. So, you know, people just don't know who to trust when they get communications over email or phone.

So, that said, we're in a difficult situation. On top of that, there are 7,000 rare diseases, and 6,000 of them — a little bit less than 6,000 of them do not have a nonprofit, and so it's hard to find patients when there isn't a home that's already been created, when there isn't a tight-knit community. There may be lots of Facebook groups, or there may not even be that. And so, how do you find patients who, one, may be skeptical, hesitant on interacting with FDA, and two, are just hard to find? And that's where NORD comes in.

Also, even when there are nonprofit organizations — this might be shocking to some of you but not to some patient advocates — there are splinters. I know of one rare disease that has 13 different nonprofits. That happens for any number of good reasons. Some may want to focus on research as their mission. Others may just want to focus on support. Sometimes it's personalities or personal politics and histories at play. Whatever the case is, it's a challenge to find patients, to find diverse patients in the community. It can be.

But I want to talk a little bit about NORD. So I have these two pictures of the founder of NORD, Abbey Meyers, on the left along with Congressman Henry Waxman, and on the right it's them 40 years later. And they look good for 40 years. But NORD had just been doing this work. I just thought it was a funny picture to put in there. And you know, I talk about — or, on the slide, you'll see Switzerland — we're neutral; we're a third party. Sherlock Holmes — we are detectives; we work to find patients. But it's not a detective work. It's really a relationship. It's shepherding the relationships that we have built for 40 years. NORD is a convener. We have 330 organizations. We work with 1,200 of them. It doesn't necessarily need to be detective work, but we have to be careful. We have to be sensitive about how we engage with the patient community. And when a patient community sees NORD approaching, they know that we have this 40-year history.

And so, that Memorandum of Understanding working with the vision-initiated listening sessions — it really is to have NORD as the convener. So there may be a group that has two major nonprofit organizations in this space, and we want them to be treated fairly. NORD will work together to help them find patients to speak on these listening sessions. We understand that FDA has a message, which is that they're really, truly, genuinely interested in learning more and engaging. And we understand that patients want to be heard.

And so, we work to help — we've created social media tool kits. We have, when there are no organizations, created the messages and posted it on our Facebook, which has over 75,000 fans, and our Twitter and LinkedIn have tens of thousands more. We have the networks and we have the channels to find patients. And so, working with all 7,000 rare diseases, we also have a rich history of relationships with the medical community. And so, we will work with medical professionals and outreach to them to help identify patients, too.

And I think that's why the glycogen storage disorder listening session was so well received. There are organizations around the world who helped identify patients in the U.S. There were physicians who actually wanted to participate as well, and we had to tell them, "Sorry, it's only for patients." And so, navigating all of the stakeholders, across splinters, across personal politics, NORD is able to do that. And there are even global politics to consider in terms of engagement for the patient community.

So we love what we do. It is our pride. We are a dot-org because that is our mission, and everyone who works at NORD works like we have a brother or sister. And I actually often forget I do have a sister living with a rare disease, pulmonary hypertension. So I personally call the patients that are selected who fill out a survey. So, for glycogen storage, we had 76 people —

FDA staff, division staff, because they initiate it; they have specific questions. So they're looking for, you know, particular types of patients in terms of their experiences, whether they're in trial or not, or, you know, age and severity. But once they're selected, it's my job to help them feel comfortable coming into the listening session. And with that, I talk with them, see if they have any questions, give them some guidelines and guidance. I'm available if anybody is really unsure. We look for those people who aren't champions or cheerleaders, people who are on the speaker circuit. Those people know how to engage, and their experiences are valuable.

But we're really looking for people who may not necessarily have been involved. And for the shy patients, we want them to really participate. It's 90 minutes, it's less than 10 people, sometimes it's 6 people, and we really want them to feel like they have a voice and that they're not overpowered. And of course, FDA has an excellent staff. The patient affairs staff does an excellent job in facilitating and allowing, making sure everybody's engaged.

On this slide, you'll see NORD and our connections. We have many members in a space. We help them activate their communities — excuse me — and work with physicians to find patients. So, finally — I think this is really important — there are a range of rare-disease communities, and our survey gathers great information about the individuals who are interested in participating. And it's so hard to decide, you know, of the 76 people. You know, how would we do this? And there were so many we did, too, and there's opportunities to do more, because we go back to the 76 and say, "You know, if there's future opportunities, would you like to learn more?" So they know they can continue to engage, even if they didn't speak with the listening session — during the listening session. But we collect all sorts of information — diagnosis states or age, delays, whether they're urban or rural, past treatments, and trial experiences — device experiences. But what we don't get in the survey necessarily are storytelling abilities, and we don't necessarily gain understanding, you know, how they can talk about their experience.

And so, we really do work with them one-to-one, whatever their emotional intelligence is. Sometimes an individual may be hesitant to share. So the coaching that we do in advance of a listening session — it's not mandatory. If a patient engaged says, "I can do this; I'm good," you know, we leave it at that and say, "Well, we're so glad that you're good to go." But if there are people who are really hesitant, it's really important to talk to them. So these are 13 sessions that we have talked and coached through many patients and caregivers to ensure the best information. It's so important. It's an incredible opportunity.

And I will stop talking, because I definitely want to have the next speaker share directly from their experience as a listening session participant. But thank you, again, so much for having NORD speak and share our work with FDA on listening sessions. It's a great honor, and we do not take it for granted. Thank you.

DR. ROWZEE: Thanks so much, Debbie. I'm going to double-check and make sure that we have Rob on the line and see if he's able to share — there he is. Rob, thanks so much for joining us today. I'm going to give you a few more minutes if you want to share your perspective. That would be great. Thank you.

MR. ADLER: Well, thank you very much. I appreciate it. Thank you to the FDA and NORD for having me on.

My name is Rob Adler. I have GSD1A. And while most of us take cornstarch to help maintain our blood sugar — hypoglycemia — I no longer do. So it makes me kind of unique in the GSD1A community. And I think that regimen of being a little bit unique is probably part of the

reason the FDA may have wanted to hear my perspective, because, compared to especially people who are younger and having to take cornstarch, I'd like to say that I'm kind of the outlier on the bell curve, and getting a different perspective, I think, was something that perhaps the FDA was looking for.

In terms of actually hearing about the listening session, I heard about it through a Facebook post that Debbie posted on one of the glycogen storage disease webpages. And so, I said, "Hey, you know what? Let me take a look at the survey. Let me go fill it out. I'm pretty open anyways in terms of — I speak to, like, grad school, medical school classes, so I'm pretty comfortable in sharing information." And you know, I saw the survey, and I'm like, "Wow, this is really detailed. This could be really good. I'm not sure if they want — you know, if I'd be able to participate in the session, but at least they could get my feedback, and if nothing else, somebody could always reach out and ask more questions." So, for me, it was nice to hear — I didn't realize that the FDA had, like, a social media presence, which I now know they do based off Twitter. But it was a nice way for me to find out.

As far as being like a panelist goes, it's pretty simple. It's an hour of your time. It's scheduled, you know, a couple weeks in advance, so you can put it on your calendar.

For me, because GSD1A, especially for someone who's 45 — there are not that many people who are older than me who have it. So always being able to hear somebody's different perspective is very helpful. And there was somebody a couple years older who — once I heard the voice, I actually knew who she was, but I didn't know if she was going to be on it. So it was nice, and we kind of chatted a little bit afterwards about how we go through seeing a hard day. And our regimens, even though we have GSD1A, are actually very different. And part of this participating, for me, is also being able to maybe help a parent or caretakers who are parents or patients who are much younger, to help them through the experiences that I've had, to maybe make things a little bit easier, a little bit more comfortable, things like that, because when you have a rare condition and it's very hard sometimes to find that feedback to find that support — that you need somebody to listen, to understand what you go through, because some doctors and physicians don't always understand what you're actually going through. And listening sessions like this help build — help the FDA get all these different perspectives, but it also helps people in a small group, especially if you don't realize that someone else has it. It's like, "Hey, I'm not the only one dealing with this, and I'm not the only one dealing with, you know, whether it's having trouble at work, having trouble at school, hypoglycemia, whatever it is." It really helps you as a patient to hear others' stories. So, from my perspective, that was a huge thing for me in participating in the listening session — was to understand that, "Hey, you know, even though I know I'm not alone, it's always nice to hear that."

And in terms of, you know, for me, whether my story makes a difference, you know, in terms of the FDA, I don't know whether it'll make a difference as a whole, but you never know when somebody at the FDA goes, "Ooh, I'd like to follow up more; I'd like to ask them a couple of questions," or in this situation, "I'd like to invite him to another panel to give some feedback." You know, whatever it is, you never know how you can help. And it may not just be now; it might be somebody listening right now going, "You know what? I'd like to help. I'd like to, you know, reach out to him, or you meet somebody down the road." There are a multitude of ways that, you know, things start going, like — you participate in one thing, and all of a sudden, everything grows: the FDA benefits, the patient's benefit, the caretaker's benefit, and the doctor's benefit.

So, for me, I think it's, you know, a really good thing to do, and regardless of whether how somebody feels about the FDA and government, this might be your one chance to actually get

heard. And I think the importance of getting heard far outweighs how might one feel about the climate of the time.

So, again, I want to thank the FDA, I want to thank NORD for their time, allowing me to participate. And if anyone has any questions here or afterwards that you'd like to reach out to me for, I'd be happy to answer them. Thanks.

DR. ROWZEE: Well, Rob, well done. There's nothing else I could say but "Thank you." I mean, it was really great to hear that from you and hear your perspective. And you know, especially if you hear that these sessions — although they're for — you know, FDA's requesting that we have specific questions, you know, to hear that it does help benefit the patient community, too, for you to hear each other's stories and find other ways to connect. It's really interesting to hear, and it's great that the benefits from these sessions are multifaceted.

So I think we're going to go to our Q&A portion of the panel now, and I want to thank all the panelists and see if we could get folks back on the webcam. We're going to kick off with a couple questions. I'm actually going to start with Debbie, if you don't mind, Debbie. I'm going to ask you a question about what patients and patient groups can do to help advocate for research and development of regenerative medicine therapies.

MS. DRELL: Of course. Thank you so much. See what I did there. Thank you so much for the question. There are so many things that a patient advocacy group can do for research and development of regenerative medicine therapies. Funding natural history studies is immediately what comes to mind. In a rare-disease space, there may not be any kind of natural history study, but if one exists helping to support and fuel the work of the researchers.

NORD has a program, IAMRARE Registry. It's a proprietary platform that has been informed by FDA with questions vetted for maximum drug development. And that IAMRARE Registry is a program that we have a full team to provide white-glove service to rare disease nonprofits who want to be the researchers. So I'm a firm believer that patients are not just participants but patients are also researchers. Whether or not they have the degree, they should be at the table and involved in the trial designs as well as in initiating natural history studies, so for those who do not have registries or natural history studies, just starting that. And how do you start that process? This information to learn how to do it is something that NORD can help you with.

So we have a program called Research Ready which helps nonprofit organizations get involved in fueling and conducting research, not just funding but actually conducting the research. And that is a huge value to the nonprofit organizations. For anybody in the room that doesn't have a nonprofit, we actually can help you start one.

So I'll leave it at that, but thank you for that question.

DR. ROWZEE: You know, it would help if I unmute it. (Laughs) So, I was just going to say, Lei, I was wondering if anything that you'd like to add onto what Debbie had shared there.

DR. XU: Yeah, I think I totally agree with Debbie about the importance of the natural history studies, especially for those — a lot of the rare diseases and actually some of which — we may not even know that until more recently, when we have the genetic deficits being identified. So I think the understanding of the disease and also the understanding of the progression of the disease is really limited. So, without really a relatively good understanding, it is very challenging to design a clinical study to investigate whether a potential investigational product was likely to

work for that particular condition. So I think that natural history data is critical to help the design of the future interventional studies. And also, the conduct of the natural history studies also are very important. I think, for us, we're glad to see a longitudinal perspective kind of natural history study and to get the data that potentially could be interpretable and be helpful to guide the development of the treatments.

DR. ROWZEE: Thank you. I just wanted to remind folks that if you have questions, please enter them in the Q&A box. We should be able to see it there. I know there may be some folks — have been entering in their questions in the chat. But if you have questions for our panelists, please don't hesitate.

I'm going to actually take a question from our audience over to Xavier. You know, the Loulou Foundation has really been engaged in sort of the gamut of our patient engagement activities at FDA. And perhaps you could comment on how or when a patient organization — how should they define whether they should approach the agency for a CPIM meeting or a PFDD meeting or, you know, one of these one-on-one patient organization FDA meetings? So, if you wouldn't mind, that would be great.

DR.LIOGIER D'ARDHUY: Yes, of course. So, I joined the foundation roughly a year ago, but before I joined, they organized this — or tried to organize this PFDD meeting with the patient community together with another foundation for CDD. So what I can share is that it's extremely important to do that very early on, you know, as soon as possible, even before the development of any compounds, because that's — especially in neurodevelopmental disorders and then a new indication or rare disease, because, as has been mentioned already, we have very limited knowledge about the disease, and we need to discover — we mentioned already the trajectory in the scales, but not only — so the PFDD — we need to get the input from the families, from the patients, even sometimes directly from the patients.

Now, my role when I joined the foundation, to set up this observational study and also this CPIM with the FDA, was really to find a way to interact with the FDA officers directly in discussing this observational study. Personally, I didn't know that CPIM was one of the key possibilities or opportunities, and when you go to the website, you'll have, like, a (inaudible). And I saw a question from someone in the chat about the defenses between the PFDD meeting and the CPIM and how — whether it was really designed for a patient advocacy group or for industry. And actually, if I understood it correctly — I don't want to, of course, speak on behalf of the FDA officers, but as far as I understood, it's open to everyone: patient advocacy groups, industry, academics, foundations, and industry. Here we had the opportunity also to invite to our CPIM — to invite an industry representative, and I think that was extremely helpful, not only to help with designing the package but also for them to understand how to best design and prepare the clinical trial landscape on this condition. Does that answer the question?

DR. ROWZEE: I think so, yeah. I mean, I think that folks can be aware that there's a menu, really, of opportunities to engage with the FDA. And that was going to be covered a little bit further in the session after our break. So, folks, stay tuned. You're going to hear the differences between the different types of meetings and who hosts them and how to get in touch to get those underway. So, yes, thanks. That was great.

Elizabeth, I know you've been involved in quite a few patient listening sessions, both the FDA-requested and the patient-requested, and I was wondering if you would like to provide some thoughts about maybe, you know, what we would call best practices for organizations who —

ones that hold a listening session with FDA or folks that maybe hear from Debbie about their potential to participate in an FDA-requested session.

DR. HART: Thank you. Yes, as you've heard repeatedly, there are multiple opportunities to participate, and hearing that patient perspective is so important, and that's one of the reasons that there are so many different ways to get involved. And so, the listening sessions that are instigated by the FDA are usually instigated because there's a specific need, a specific question, something that's particularly timely. But there are so many areas of development. So even if it's not something that maybe we're currently contemplating a particular issue — again, as you've heard, setting that foundation early to go forward so that we hear the patient perspective, so that we can include your perspective and share that with sponsors, so that that can inform us on best practices, because, again, in rare disease, as we hear time and time again, every patient matters, and it really takes a lot of thought to really preplanning. And so, whether that's doing a natural history and you've heard about, whether that is doing a patient preference study, whether that's looking at developing endpoints, whether you're maybe an individual or a smaller organization and don't have the resources to do all of that, even just sharing your individual perspective and input is so important. Does that answer the question?

DR. ROWZEE: Yeah, I think that was really very helpful, and hopefully our folks online think so, too. And when I actually take a second, I see — check in with Rob, and I see — Rob, I know you covered a little bit of this in your remarks, but, you know, if you have any advice, something in particular that you'd like to share with patients who are either considering approaching FDA with the listening session or if they're approached by Debbie and NORD about participating, if you'd like to share just sort of, you know, some of those thoughts on what people should think about when they're considering joining...

MR. ADLER: Well, I will say this. Debbie made it very easy for me to want to do it. She made me feel very comfortable. And so, that level of comfort when you're a patient always makes you more willing to share, whether it's in a listening session, in a doctor's office, wherever it is. It makes life a lot easier.

So, like, for me, I would say, you know, you have to — if you're a patient and you want to find a way to either reach out to the FDA or if the FDA reaches out to you, it's really on the patient to feel comfortable and — wanting to basically give feedback to try to help. It's not just about helping yourself. It's about helping everyone else. So, in terms of the — you know, when the FDA reaches out, you know, I mean, like, you know, I think a lot of people will — you know, you'll Google somebody if somebody reaches out to you. I have no shame in admitting that. So, when you see that and you go, "Okay, well, that person is legitimate; I need to go help," and once that happens, like, you know, I'm — for me, I'm totally okay again. I worked in the media for a long time, so I understand the whole feedback, and sometimes you just have to trust people even if you don't really know who they are. You just have to kind of go in and go, "Okay, and if there's a — you know, if there's an issue, I'll figure it out later." But I went in going, "You know what, I think I could help people, I could help younger people. I think my information can help the FDA." And I said, "You know what? It's totally worth doing this." And I think a lot of patients that considered that aspect: that it's not just about them but it's about helping everybody else. I'm not sure if that answers your question.

DR. ROWZEE: It does, because I think I remember a colleague saying, you know — or, to paraphrase it, it sounds like these listening sessions — they open up doors to other avenues of support that folks — they didn't know about and hadn't heard of before, within even their own communities. So, I think that —

MR. ADLER: Can I give a quick example, like 30 seconds?

DR. ROWZEE: Please. Go ahead.

MR. ADLER: There was a caregiver, and her son was getting ready to go off to university, and they were worried about what's going to happen. And so, I reached out and kind of went through my experience. Now, granted, my experience, you know, was 20 years ago, but it's still having to deal with everything: "Take your cornstarch; manage your blood sugar; manage your schedule." All that stuff still stays the same. And I lived in a dorm like their son wants to. So at least I can say, "Here's what worked for me. It worked out well. Here's what you as a caregiver need to tell your son how to — you know, what he needs to do now in order to prepare himself to go off to college."

DR. ROWZEE: That was excellent. Thanks for sharing that.

I know that that listening session — like Debbie had mentioned, it enjoyed such enthusiasm from the community. And I think that it's a really nice, you know, poster child, I guess, for other folks to hear about and see what could come out of that.

And that actually leads to a question that we have from our audience. Debbie and Elizabeth, maybe you guys could just sort of take a stab at this. And it's just, what happens to the information provided to the FDA after a listening session has been held? Maybe, Elizabeth, if you wouldn't mind taking that one...

DR. HART: So, first, as you heard, there is a place where the sort of — there's a summary of the information so that others can go back and in the future — and can reference. And so that is public, but that is relatively brief, and it's de-identified. But internally, we have more detailed information, and that's the information I was talking about that gets used to really help inform the clinical studies and the reason that we started having the listening sessions in the first place and requested it.

DR. ROWZEE: Debbie, if you want to tell a little bit about sort of the experience after the CPIM meetings in particular, I think that, you know, that might be helpful, because this is certainly a different type of meeting that — generally, they're more scientific in nature but would provide a nice contrast for the listening sessions.

MS. DRELL: So, was the question "What happens after the listening session from the patient organization side"?

DR. ROWZEE: Oh, I'm sorry. Yeah, go ahead.

MS. DRELL: Okay. I just wanted to make sure I understood your question. So one of the listening sessions we had — the Patient Organization No. 2 that were involved — the patient organization wrote an article about it. They used it to help continue to fuel patient and caregiver understanding about research and about how pivotal this moment was with the listening session. So they just built on top of this wonderful opportunity the ability to share with their community how they're being heard at FDA. And so, that was something that one organization did. And what happens right after — what typically happens is, you know, we thank the participants; we actually survey the participants to see if they have feedback to help us improve listening sessions. And then, we'll let the organizations know who were involved, all of them, really, when the notes are posted. And I wasn't surprised about a lot of people who wanted to



speakers who were unable to. They are very interested in the notes, too. I think people truly — and they'll ask us, you know, "Are they up? Are they up?" And we'll get an email in a couple weeks. So we love the enthusiasm, and people do understand; many people understand how important these meetings are.

DR. ROWZEE: And like Elizabeth said, GSD went up today. So it's live. It's exactly the listening session that we're talking about. You guys (inaudible), you know, sort of what we're talking about, real time. You can search for "FDA" and "patient listening session," and you'll be able to find the page.

So I wanted to get back. Xavier, I'm not sure if you want to talk a little bit about the CPIM meeting and just sort of, you know, what happens afterwards. In this case, I know that what happened was a follow-up meeting for a specific question for OTAT, but — how you use the information that you get from those meetings to keep moving forward.

DR. LIOGIER D'ARDHUY: Yeah, well, the first thing is that I was extremely surprised that we were willing to ask for an additional informal call with the FDA officer. So we were told after the CPIM that if we wanted to have additional clarification on one specific topic, we could liaise with the project manager and maybe expand on one specific question, so ask for clarifications. Again, coming from the industry — the FDA, for me, it's the Army, and we were almost told that, you know, "You should be afraid." And so, to me, that was no good, and I couldn't envisage that we could go further in asking for clarification. So that was a very nice surprise, I would say, very straightforward. And we had this additional follow-up call with the OTAT people on one specific question related to safety. And I have mentioned that already. But besides that, of course we took notes and, again, an additional surprise that the officer attending — who attended the follow-up call — they were willing to commence and give us some feedback on our notes. So, altogether, I have to say that it was extremely helpful in many, many aspects.

The CPIM, of course, itself — the follow-up call, and we had, you know, a very thorough discussion on these topics. So, I mean, that was shared by Dr. Xu as well, of course, who was part of the discussion.

DR. ROWZEE: Thank you. And Lei, I see you're back. We've got you back on camera here. So do you want to wrap this up with your comments?

DR. XU: No, I don't think I have much to add. I think I just want to emphasize, I think, where I really see — or try to help, to expedite the development of the treatments, especially for this rare disease is, most of which have really limited treatment options available. So I think our advice, really, it's getting towards — that I don't think we are here to — really want to be a road blocker to prevent any effective treatment from being developed. But on the other hand, we really need good clinical studies to provide data to really demonstrate that there is a benefit-risk profile for those vulnerable patients.

DR. ROWZEE: Wonderful. Thanks. I see that we're at time. And if we were in person, I would ask for a round of applause for our panelists, I guess. Thank you for hearing their experiences. So I'm just going to have to settle for saying how grateful we are to have heard your perspectives. And thank you for your time today. It was great to see you.

We're going to take a break now. Get up and stretch. Do what you've got to do. And we'll be back here at 1:15 p.m. Thanks, everyone.

(Recess)

DR. ROWZEE: Welcome back everyone. Thanks for joining for the second half of today's workshop. We've had some great discussion and questions so far. So let's settle in — get cozy. We'll jump right back in.

We'll start off Session 3, which is a panel discussion. This panel features our stakeholder engagement leaders from across FDA who are going to provide an overview of the different ways that patients and patient groups can reach out to FDA to take advantage of our engagement opportunities, and I'm going to pass it over to my colleague, Karen Jackler, and she's going to moderate this session. Karen, thank you.

MS. JACKLER: Hi. Thank you, Anne. And welcome back, everybody. It's my pleasure to speak with you today and to help you to get to know the FDA and CBER better. And as Anne said, I'm Karen Jackler, and I'm the patient engagement program manager in the Office of Director at the Center for Biologics Evaluation and Research. Welcome to Session 3, the FDA panel discussion on how to engage with FDA when opportunities arise.

So we're going to hear from three — four different — actually five different FDA offices and centers on ways patient groups can reach out to the FDA and take advantage of patient engagement activities. So it's my pleasure to be alongside my colleagues today. A lot of these people have been my colleagues for a long time in different capacities, and I'm very happy to be able to continue to work with them in the patient engagement capacity.

So I'm just going to give a quick overview of who you're going to be hearing from. So first up, we're going to have Andrea Furia-Helms, and Andrea's the director of the Office of Patient Affairs, the Office of Clinical Policy and Programs, and the Office of the Commissioner. And after that, we'll hear from Sadhna Khatri, who is a regulatory officer in the Professional Affairs and Stakeholder Engagement Office, which is in the Office of the Center Director at the Center for Drug Evaluation and Research. That will be followed by Michelle Tarver, who is deputy director of the Office of Strategic Partnerships and Technology Innovation and program director for patient science, the Digital Health Center of Excellence, at the Center for Devices and Radiological Health. Then we will hear from Rea Blakey, who is the associate director of external outreach and engagement at the Oncology Center of Excellence at the FDA. And then it'll come back to me, and I'll tell you a little bit about what happens at CBER in terms of patient engagement. And I'm also going to try and put a wrap — wrap it up a little bit, to sort of zoom out a little bit and put some things into context before we move on to our Q&A.

All right. So I'm going to go on mute now, and Andrea will give you some information about her office.

MS. FURIA-HELMS: Thank you, Karen. Just to check, can you hear me okay?

MS. JACKLER: Yes, Andrea; you sound great.

MS. FURIA-HELMS: Okay. Just wanted to make sure. You never know.

So hello, everyone. Good afternoon. My name is Andrea Furia-Helms. I am the director of the Office of Patient Affairs in the Office of the Commissioner. And thank you for attending this session today, and thank you for the Center for Biologics for providing us an opportunity to

share how we involve patients and advocates and their perspectives in the agency's work. Next slide, please.

So, I'll start with an overview about some ways that FDA includes patient perspectives in our regulatory activities, and then I want to share some resources with you which might be helpful. Next slide, please.

So, first, I think it's important to start with why we feel it's really important to hear patient perspectives and incorporate those perspectives into the medical product development and review process. Patients and caregivers and their advocates can provide insights on specific issues, their needs, and their priorities and really talk about what's important to them and their family members. Their perspectives can provide really diverse opinions and experiences and shed light on issues like risk tolerance and potential benefits. And their perspectives also can share things about participating in clinical trials. And of course, I think it's really important to remember that they provide real, everyday experiences in real-world settings, which always reminds us of the human element. So, ultimately, patients are really at the heart of FDA's work and activities. Next slide, please.

So this busy slide is not meant for you to read every little detail, but it's only meant to demonstrate that there's been ongoing patient engagement activities at FDA for many years. It may seem that it's been something pretty recent, but really, incorporating patient perspectives in FDA's work has been something that has been ongoing for over 30 years. And while we might not be able to specifically pinpoint when patient engagement at FDA began, we do know there was an increase in engagement at the height of the HIV/AIDS crisis in the late 1980s. And since that time, involving patient stakeholders in our work has certainly increased and evolved in so many ways. And it has provided patients and advocates to get involved in FDA activities in a variety of manners which help FDA staff hear about various patient experiences and opinions. Next slide, please.

So I want to talk a little bit about Patient Affairs, like who we are and what we do. But, before I get to that, I think it's always important to share a little history. And as I mentioned, there was an increase of engagement at the height of the HIV/AIDS crisis in the 1980s. In response to this, at that time, an office was established to interact and build relationships with patients and advocates specifically at that time with the HIV/AIDS community, but then that office expanded to include oncology and eventually all serious and life-threatening diseases and conditions. Now, over the years, center-specific patient engagement offices and staff have been established, because there's really unique issues specific to each medical product center for the products they regulate. Patient Affairs was established in late 2017, and there was — and that was from a trans-FDA assessment on patient engagement activities. And that determined a need for a cross-center effort at the commissioner level and with the focus on crosscutting issues. And really, we also are, like, the central entry point for patients who do not know where to begin in contacting the agency.

So, because we are the central entry point, we work closely with the medical product centers as well as other offices in the Office of the Commissioner, such as the Office of Minority Health and Health Equity. And we all collaborate with patient communities. We're all looking to enhance patient engagement efforts, and ultimately we're looking to include ways which patient perspectives can inform FDA's work. Next slide, please.

Now I'm going to talk about some of the Patient Affairs activities, including patient perspectives in our work. Next slide, please.

Some of our programs and activities for incorporating these perspectives are patient listening sessions — are patient engagement collaboratives, and we're also continually enhancing communications to keep in contact with patient communities. Next slide, please.

So, I'll start with the patient listening sessions. In the last session, you heard about a recent patient listening session we held on the glycogen storage disease. And if you're interested in accessing that summary — those summaries and other patient listening session summaries, I have put the link into the chat, so you can access that at another time. So, just to mention a little bit more about the sessions and what they are and what we're trying to accomplish — is that — just remember that these are just one of many ways that FDA is expanding patient involvement and to encourage communication between patient communities and FDA staff. Currently, our focus on patient listening sessions are on rare diseases, and we conduct these in collaboration with our partners, the National Organization for Rare Disorders and Reagan Udall Foundation for FDA.

So patient listening sessions allow FDA staff to engage with and hear experiences directly from patients, caregivers, and their advocates to help better inform medical product development and other regulatory issues within the agency. So, just for example, patient listening sessions provide an understanding of things like disease and treatment burdens, functionality, and impact on daily activities and what are the priorities to consider when developing new products. But patient listening sessions also educate review staff about rare diseases and other conditions, help patients and their advocates understand FDA's work just by participating, and it also provides a starting point to inform early stage research and development. Next slide, please.

So, just to dive a little bit deeper into what we hear — what we're trying to better understand in our listening sessions, I'm going to share some examples of the topics that are discussed. So, for patient experiences, some examples include symptoms and aspects of the disease that patients and caregivers consider are important to address: as I mentioned earlier, disease burden and symptom progression, activities and functions that are most important to preserve or restore, and things like their experiences on how current treatment regimens are working to manage their symptoms. Next slide, please.

Things we discuss and hear about around treatment options are priorities for potential treatments for a disease; expectations that are of a potential treatment that might be being developed; what might be considered, such as meaningful outcomes for new therapies and what could be the decrease — potential therapies that could decrease the severity of symptoms rather than completely remove it or resolve it and is that acceptable; and perspectives on the level of willingness to continue in an investigational medical product before feeling relief from symptoms. Next slide, please.

Then, as I mentioned, we also have discussions about participation in clinical trials, so things like considerations about participating in a clinical trial, what the barriers might be in participating, understanding the benefit-risk trade-offs in participating, exploring potential study designs that might encourage or discourage participation, and differences in perceptions between investigational drugs and other therapies such as gene therapies, and then also discussing things that would give us some insights into meaningful clinical endpoints. Next slide, please.

So I'm going to switch gears now to the Patient Engagement Collaborative, which is a collaboration between FDA and the Clinical Trials Transformation Initiative, which you may know as CTTI, through our public-private partnership. It's comprised of an external group of

patient organization and individual representatives from various communities — took four topics about enhancing patient engagement and medical product development and other regulatory discussions at FDA. So the PEC members decided they wanted to focus on communications for this time around, and so they're helping us improve our For Patients webpage, which we're implementing improvements right now. And they are also working with us to develop educational resources patient communities about FDA, what we do, and how patients can get involved. Next slide, please.

So, as I mentioned, we're also trying to enhance communications and keep in contact with our patient stakeholders. And so, we heard from them loud and clear that they were having some trouble finding information. So we have been working on ways to enhance these communications. As I mentioned, we are implementing improvements to the FDA's For Patients webpage with the help of the Patient Engagement Collaborative. We are in to keep connected with patient communities through our Twitter handle, so we share things like FDA important safety announcements and other updates for patients, such this particular workshop, so that you keep informed and you can participate. We also have an educational video series, and that's called Patients Matter, and this helps patients and caregivers better understand FDA, our programs, and, of course, how to get involved. And we also send targeted emails out on FDA-related communications that may be relevant to specific patient communities. Next slide, please.

Well, I'm going to wrap up with the following slides for — to share some resources that may be helpful to you. Next slide, please.

So this slide is a resource of contacts and links. And if you're looking to learn more about all the Patient Engagement program and initiatives across the agency that you might have already — that you will be hearing about today from my other colleagues on the panel, this slide will give you all those points and how to contact those programs. So, just to be aware, each medical product center — they have their own staff and their own Patient Engagement initiatives. So it's important to know who to contact for what specific initiative you may be interested in getting involved with. Next slide, please.

And again, from our patient stakeholder feedback, which we so appreciate, it wasn't so easy to know where to begin in contacting FDA. So we worked closely with our colleagues in the medical product centers in creating this web form for patients and advocates to submit their inquiries or possibly even after a meeting to discuss something. So please visit the patient portal. It's called Patients Ask FDA, and you can find that off the FDA.gov website. Next slide, please.

And finally, this is the Office of Patient Affairs team and how to contact us. So feel free to reach out to us any time. We're always here to help and we're happy to help you in any way we can. Thank you for your attention, and now I will turn it over to Sadhna Khatri from the Center for Drug Evaluation and Research.

DR. KHATRI: Thank you, Andrea. And, Karen, thank you for the introduction. Good afternoon, everyone. I am Commander Sadhna Khatri from the Professional Affairs and Stakeholder Engagement Staff in the Office of Center Director in the Center for Drug Evaluation and Research. So welcome to each and every one of you. Can I have the next slide, please?

CDER's mission is to promote and protect the public health by ensuring that safe and effective drugs are available to Americans. So this is a very succinct mission statement, but it encompasses a lot of activities. CDER routinely consults with American people in making its

decisions about the drugs that they use. It holds public think tanks to incorporate expert and consumer input into its decisions. The center also announces many of its decisions in advance so that members of the public, academia, industry, consumer groups, and professional societies can comment and make suggestions before decisions become final. In addition, CDER holds annual public meetings and consumers — with consumers and patient groups, professional societies, and pharmaceutical trade associations to obtain and ask public input into its planning and priority-setting practices. Over the years, policies have changed and laws have gotten stronger, but the center's present and future missions remain constant to ensure that the benefits of drug products made available to the public outweigh all known risks. Ultimately, patients are the focus of all CDER activities, and we need to engage with them. Next 16 slide, please.

So, first, let's start with "Where are the opportunities for engagement?" This has changed over the last decade since I have been involved in drug development. Patient input has played an important and increasing role in development and regulation of medical products. A large number of patient engagement activities are in progress at CDER. You can see on the slide the multiple different opportunities for patients to engage with FDA. Patient-Focused Drug Development — we refer to them as PFDD meetings, which is turning out to be perhaps the most effective and best way to bring to us patients understanding and experience of the disease. I will be talking more about them in a later slide.

Next, we have Advisory Committee meetings, and most of these advisory committees do have a patient representative assigned to their advisory committee to represent that point of view. Patient representatives are selected to participate in an AC meeting. This is an opportunity for public dialogue. Patient representatives are considered government employees for the duration of the time they are serving on the advisory committee. We also have public speaking sessions where many patients ask to take advantage and come and speak, but they often get about 5 minutes to make their point of view to that advisory committee. That's 5 minutes each. We often encounter patient advocacy organizations at external meetings, and my colleague, Andrea — she mentioned the NORD, which is the National Organization of Rare Diseases. In those meetings, they often have very lively engaging conversations with patients and patient communities, and we do invite them to talk to us here at CDER. Then the patients at the advocacy organizations — they also request to speak with us, and yet they will actually comment and even talk to them and engage with them and listen to their point of view. They are typically scheduled with the Review Division at CDER. Next slide, please.

Then there is the Citizen Petitions. Many patient advocacy organizations have the specification to submit to us a Citizen Petition which outlines a desired action that they would like us to consider or point of view for us to consider. So we carefully review those. We often have a (inaudible) legalistic aspect to them.

Then, finally, we do put out notices in *Federal Register* so that the public can be aware of some of the things we are doing, such as guidances. We do carefully review all the comments, sometimes thousands of comments that come to us from those *Federal Register* notices, often from patients and patient advocacy organizations. We often receive a lot of emails and letters, and sometimes certain advocacy organizations seem to think that the most effective way is to bombard us with thousands of emails. And while certainly it does get our attention, I can tell you it's probably not the most effective way to be able to get your point across to us. Patient opinions are also expressed publicly, including through social media — for example, the FDA's Facebook page. The activities listed on these slides are common across all FDA centers. Next slide, please.

Patient-Focused Drug Development is a systematic approach to help ensure that patients' experiences, perspectives, needs, and priorities are captured and meaningfully incorporated into drug development and evaluation. Now, keeping the definition of PFDD in mind, I would like to talk about FDA's patient focus with drug development initiative. FDA recognized a need for more systematic way of gathering patient perspective on their condition and treatment option. So, in the Prescription Drug User Act 5 — and we conducted about 24 disease-specific patient focus drug development meetings, giving patients and caregivers a platform to contribute input that can inform drug development and evaluation. Next slide, please.

Patients are experts on what it's like to live with their condition. They are able to articulate specific disease facts, symptoms, loss of function in concrete terms. They can identify and articulate what is important to them regarding treatment benefit. For progressive regenerative diseases, many patients, their parents see an ideal treatment without minimum stop regression of their child's loss of function.

The chief complaint will not be factored explicitly into drug development plan. Example at endpoint — and measures of drug benefit in clinical trial. Patients want to be as active as possible in the work to develop and evaluate new treatment. They aren't expecting for FDA to address all the gaps in the current treatment or current approaches to drug development but do want FDA to help identify the most effective pathway for them to play a major contributing role. Next slide, please.

Under PDUFA, FDA began developing a more systematic way of gathering patient perspective on their condition and available treatment options. Here you can see some PFDD meetings today. Next slide, please.

There are also going external interest in expanding efforts to gather patient input in support of drug development and evaluation. So, in 2015, we started welcoming patient organizations to identify and organize their own patient focus collaborations. These externally led patient-focused drug development meetings provided opportunities to hear directly from patients. And it is also encouraging to the patient population that advocacy groups are collaborating with the agency.

Patients are able to express what matters most to them and take charge of their health, while FDA's open to attending an externally led meeting and any results products, like reports or surveys will not be considered FDA sponsored or endorsed. Patient organizations identify and organize patient focus collaboration to generate public input on specific disease areas. The FDA meetings provide an important opportunity to hear directly from patients, patient advocates, and caregivers about the symptoms that matter most to them, the effect the disease has on the patient's daily life, the patient's experiences with currently available treatment. While FDA will be open to participating in a well-designed and well-conducted meeting, an externally led patient focus or development meeting and any developing product — example, surveys or reports — will not be considered FDA sponsored or FDA endorsed. Next slide, please.

Patient input from meetings can support FDA staff as they conduct benefit assessment for products under review or advise their sponsors on their development programs. We also believe that patient input collected from these meetings can have value to drug development more broadly, for example, by helping to identify areas of unmet need, such as aspects of a patient's condition that is not currently being addressed in current therapies. This input may also help developers as they identify or create tools used to measure the benefit of prevention therapies.

The deliverables are, as I mentioned before, a summary and a survey from an externally led patient focused drug development. Next slide, please.

If you would like to request a meeting with the Center for Drug Evaluation and Research, please reach out to professional affairs and stakeholder engagement staff. We are located in the Office of the Center Director in CDER. And our mission is to enhance the experience of our external stakeholders which influence patients, their caregivers, and patient advocacy organizations, and engaging with our center. I will share a contact email at the end of my presentation. Patient work is important to CDER, because patients look inside to a disease. Patients provide insight on issues, problems, and/or questions that are important to patients and family members. We also recognize not just one patient represents the whole patient community of a particular disease. Patients have invested in drug and diversity (inaudible) perspective, both in terms of risk tolerance and potential benefit. So it's important to identify what matters, what is important to patients. We highly value patient engagement and its contribution to the development of drug products. (Inaudible) for patient engagement is used in integrating patient voice into the regulatory process to better enable patient perspective to shape product development and approval. Next slide, please.

Collaborative strategy is a key to successful campaign by patients to identify the intersection between the needs of the agency and the community. If you think about it, FDA language has homogenous dimensions and related scales. Patients' language is what is working and what's not working in my life — also examples of hydrogenous-like circumstances. There are more similarities than we might think. Here I have shared an example from the Depression and Bipolar Support Alliance Group. They were able to bridge that gap. They identified the unmet need. They contacted our group, which is the Professional Affairs and Stakeholder Engagement. We conducted a meeting with the review division, which resulted in a meaningful output, a scientific workshop, and an externally led patient-focused drug development. Next slide, please.

Here are a few examples of how PASE has engaged with the patient community. We have hosted several workshops, which help patient advocacy groups gain understanding of how to effectively engage with CDER. Next slide, please.

In this slide, you can see the variety of external stakeholders our group engages with. We're trained to instruct professional organizations through academia, through also a lot of patient advocacy groups. Next slide, please.

And the last — I would like to wrap up by sharing some resources on how to contact us. And these slides will be available later on, and you can — please feel free to contact us, and we are here to help in any way we could. And that concludes my presentation. Next slide please.

Thank you everyone for attending, and also thank you to CBER for organizing this event. Now I will pass it on to my colleague, Dr. Michelle Tarver.

DR. TARVER: Good afternoon, and thank you all very much for the opportunity to share with you the work we're doing to engage with patients in the Center for Devices and Radiological Health. As you know, patient input and engagement is useful across the total product life cycle of medical devices. Patients have unique insights into the unmet needs that they have in their community that can inform innovative medical devices and also can be useful in design of the devices so that they are user friendly and have a good user experience. Patients have wonderful insights that help design clinical trials so that they are more patient friendly,



measuring the outcomes that matter to patients, and ensure that patients are more likely to be in the trial, from the beginning to the final endpoint of the study. Patient preference information can be quite useful, because how patients do the trade-off between the benefits and risks of various medical intervention can be informative to regulators who are also looking at the risk and benefit profile of medical therapy. Patients have insights in the best way to communicate about benefit risk to their colleagues. And patients can be part of our active surveillance effort, giving us insights in how a medical device performs once it's in general use.

We've seen the impact of patient perspective on the regulatory process itself. In fact, right now, we have 24 industry-sponsored, regulatory patient preference studies that have been either completed or in the pipeline. And these studies have led to expanded (inaudible) for medical devices, as well as informing the designing of a clinical trial. To date, we have over 50 percent of our pre-market authorized devices that have clinical data have a patient reported outcome as part of them. Clearly, it's impacting the work we do at FDA. Our Patient Science and Engagement Program is inspired by patients and driven by science. And we've been working very hard to cultivate a culture of patient engagement when we make it part of our daily business at the center.

I want to show you some examples that demonstrate how that happens across the total product life cycle. Patient engagement cannot happen by chance. It happens by intentional activities. And we've put in place some mechanisms that can foster those engagement opportunities for our staff. One such mechanism is the patient and caregiver connection. This is an opportunity for our staff to hear directly from patients about what it's like to live with their condition as well as innovative medical devices. We also get the opportunity to hear challenges that may be facing that patient community.

On this slide, you can see the number of organizations that are currently part of this connection. We have 18, and we are always open to new partners. What I will say is that these partners are much more than partners. They are active partners in the process. And I want to share with you some of the work that they've done with our center. They have come and shared their experiences with their condition as well as with their devices. In fact, the temporomandibular joint association worked with the review office that looks at devices that are used to treat and manage this condition — shared with them their experiences, and that conversation actually led to a research effort that we are doing collaboratively with them to look at the natural history of the disease in mild and moderate conditions.

We've also invited patients and their patient and caregiver connection to be part of our scientific workshop. Last February, right before COVID shut down most of our in-person events, we had a workshop on the evolving role of artificial intelligence in radiological imaging. And we asked members from two of our patient and caregiver connections partners to have patients come speak to the audience. And it was really informative, because from their experiences, their input, the industry, and the reviewers that look at these types of devices — realized there were things they had not considered and (inaudible) to consider in the development and evaluation of medical devices.

We've also heard from patients about the challenges they experienced during COVID-19. In fact, we were concerned that certain patient communities may be experiencing shortages in their medical devices that they need for the management of their underlying medical condition. So we reached out to the American Association of Kidney Patients, who is one of our partners, and asked them to survey their membership to see if any of them were having challenges

accessing particular devices. And what we learned, we took, and it informed some of our shortage response efforts.

We also want to hear when we get things right, when things are going very well. So we had a patient engagement town hall, where we invited patients who were using digital health technology to help manage and monitor their health condition. What we learned from the conversation with various patient groups is that these technologies really could help them in the management of their condition and improve the quality of their life. So our staff needed to hear that their work was having a meaningful impact on real people.

We also wanted to make sure that it was known that our doors are open. We created a video that talked about ways in which we engage with varied patient group and also that encouraged patients to come speak with us, if they're interested in finding ways to improve the population that they work with, in terms of their health and their outcome.

And the last mechanism I want to share with you is our Patient Engagement Advisory Committee. This committee is the only one working at the agency. It is comprised solely of patients, caregivers, and patient advocates. And they provide us with formal recommendations regarding general matters related to medical devices. We often have one to two of these meetings per year and we do many agency-directed appointments to that committee.

These are some of the topics that we've talked about in the past, but what I'd like to focus on — not necessarily just the conversation but — that these conversations turn into impact, into actionable efforts. One such effort is the video that you see on this slide, which was designed to help encourage underrepresented populations to participate in clinical trials, which is a thing we heard during that Advisory Committee meeting in 2017. We put together this video in conjunction with the Office of Minority Health and Health Equity.

We also heard that there's a formal step that FDA needed to take. And so, we drafted guidance documents on patient engagement in the design and conduct of medical device clinical investigation. This is still draft guidance, but it will go to reflect some of those themes that we heard during the Advisory Committee meeting.

We also had a meeting on cybersecurity, where we talked about the threat that medical devices face, especially since they are increasingly interconnected, and talked about what are ways we can communicate about these cybersecurity vulnerabilities to the public. We put forth a framework or a consideration for a framework document in 2019 — excuse me, 2020. And we also announced in 2020 that there was a video we're putting together on cybersecurity hygiene.

So I've talked about ways that we've engaged, but we don't just stop that engagement at the medical device stage, but also, we incorporate patient and research efforts, where they sit at the table and help design the studies with us. Part of empowering patients to contribute is to educate them, as well as other stakeholders, in some of the important concepts that can be brought to the table when designing these research studies. We've held virtual boot camps at various centers of excellence and regulatory science innovation across the country. And we've invited small, private companies; patient groups; as well as academic centers and other interested parties to attend. We've had 70 to 90 attendees at each of these interactive sessions and really walk through the consideration for digital health technologies that are measuring meaningful patient outcomes. We've also got a number of patient preference studies that are illustrated on this slide, where patients are involved in the design and the conduct of those studies.

But we know that it's also important to include all voices, not just one voice. And so, we have undertaken studies that examine the voices of diverse patient populations, whether it be our pediatric population, different gender, patients from various racial and ethnic groups, as well as those who may not have English as their primary language and those who have other function limitations. So we've done projects on all these areas, wanting to make sure that our devices are being designed with all people in mind. We've worked collaboratively with the Medical Device Innovation Consortium, which is one of our partners in advancing the science of patient input.

And that leads me to the last thing that I'd like to leave you all with today: the importance of collaboration. One of our strategic priorities at the Center for Devices and Radiological Health is collaborative communities. Collaborative communities are continuing forums for public- and private-sector members who actively work together to achieve common objectives and outcomes, share challenges, and leverage collective opportunities in an environment of trust, respect, empathy, and openness. We are currently participating in 10 collaborative communities that have been started through grassroots movements in various topic areas. But what's important to note is many of these communities have patients sitting at the table with another solving these shared challenges.

And in fact, that is one of our Center's priorities in deciding to participate in the collaborative community. We invite you all to consider this approach as you're trying to tackle some of the challenges facing your community. We've talked about the importance of engaging patients in many different ways across the total product life cycle, from measuring that input with a structured, well-defined measurement tool, as well as involving patients in the construction of those tools.

If you are interested in learning more about the work we're doing at the Center for Devices and Radiological Health, I encourage you to reach out, visit our website. And we do send newsletters quarterly about the work we're doing. If you're interested, please don't hesitate to reach out. We're happy to send a newsletter to you as well.

Thank you. And with that, I'd like to introduce Rea Blakey from the Oncology Center of Excellence.

MS. BLAKEY: Thank you, Michelle. I have to say all of the presenters, my colleagues, have given really great information for those of you in the audience. And I insist that you avail yourselves of the opportunity. They're great colleagues, and there's great information available to you. We're very much interested as an agency in patient engagement.

I will let you know that our center, the Oncology Center of Excellence, is one of the newer centers to be involved at FDA. In fact, just as a matter of historical perspective, in December of 2016, the 21st Century Cures Act was signed into law and then the following month, that January, the Oncology Center of Excellence was created in response to the national Cancer Moonshot initiative. OCE is the first interactive, inter-center institute offering a unified interaction between three FDA centers, all focused on oncology information. And the mission of the OCE is to achieve patient-centered regulatory decision-making through innovation and collaboration. So, well, we're glad to be a part of the FDA family.

OCE marked its third anniversary, actually, a year ago in January of 2020, just as the COVID-19 pandemic was beginning, and our attention has been devoted in the meanwhile to patients with cancer to make sure that they were not forgotten during the pandemic. OCE's staff shifted to

working from home in mid-March, and the work has not stopped at all. In fact, we've probably been more productive. Many approvals were aimed at limiting potential COVID exposure by reducing interactions patients might have with health care providers.

And you're certainly welcome to visit the FDA/OCE website to see a copy of this annual report or any other information that you'd like to learn about OCE. And by the way, I did drop in the webpage in the chat box for those who are interested in that as well.

Among other things, we are also interested in many diverse voices just as many of the other patient engagement aspects of FDA. We want to hear from patients and caregivers, and we want that information from the patient experts and advocates to inform our decision-making at the Oncology Center of Excellence.

So one of the many things that we did was, first and foremost, OCE conducted 11 meetings with patients and advocates and stakeholders during the very earliest and most uncertain days of COVID. And those listening sessions allowed OCE to disseminate the latest FDA clinical trial guidance during COVID to these most vulnerable groups. And we're happy to have had the opportunity to interact, and again we wanted them to know that we were still actively involved and engaged in making sure that the regulatory process continued on their behalf.

Also, in the past year, OCE began Project Equity, which is designed to provide guidance to industry to facilitate diverse representation in oncology clinical trials and promote more equitable and inclusive research in policy practice. We're also proud that during 2020, Project Silver was created. And that focuses on increased geriatric patient enrollment in clinical trials and bringing the voice of underrepresented populations to the world of drug development. And by the way, this year, we saw the very first draft guidance to industry on inclusion of older adults in cancer clinical trials. Also underway at OCE is the Project Facilitate Call Center. That's a pilot program to assist oncology health care providers or regulatory professionals in requesting access to investigational therapies for patients with cancer. And also, we're proud — among many other projects at OCE — proud of Project Patient Voice, which is an online platform for patients and caregivers, along with their health care providers, to look at patient-centered, patient-reported symptom data collection from cancer clinical trials. And again, all of this is available if you go to [FDA.gov/OCE](https://www.fda.gov/OCE) and you can find all of our projects actually listed there. I tried to be selective just to keep them focused.

Another that I have to tell you about, because it's mine, is Project Community. That's an ongoing, external outreach and engagement initiative, which started in 2018 as an on-the-ground, beyond-the-beltway, if you will, in-person outreach. The purpose of Project Community includes to facilitate access to cancer information for high-risk individuals, the underserved population, those who are underrepresented, and then the public at large as well, specifically to increasing clinical trial participation and genomic database contributions in regard to cancer research. We, obviously, are very much interested in creating mutually beneficial and enduring relationships among those communities as well as advocates and our OCE cancer product reviewers. Keep in mind we're fairly new, and we do appreciate the fact that many people across the nation are not yet familiar with the Oncology Center of Excellence. So we like to get our folks out and about so that you can learn more about what we do and our need to interact with you.

I'm going to talk a little bit more in additional slides about the National Black Family Cancer Awareness Week. But, for now, I do want to talk a little more about Project Community, because there is an effort underway to try to get more involved in actual communities in person. While we

all know that we're still dealing with COVID, we were able, right before the COVID pandemic hit, to get out to Chicago and had a very interesting activity there with in-person outreach and the NIH *All of Us* genetic database campaign. And we had Black History Month cancer-focused panel discussions that basically came about only because of the community involvement of advocacy groups in Chicago. So we're grateful for that.

And then this year, we were able to come back, and again — the enduring relationships aspect here. We had another Black History Month outreach opportunity with some of the same groups — one group in particular, Peer Plus Wellness, which we're big fans of. Their community outreach efforts — we worked with them specifically to have a conversation about straight talk about cancer, COVID-19, and long-hauler syndrome. That was this year, just in February. We look forward to more engagements like that with communities around the country.

So again, we do want to have activities that are out in the populace. Many of them are happening by Zoom these days. But those are some of the things that we've been doing with Project Community.

Also, we wanted to make you aware of our conversations on cancer series. It's a public panel discussion series that has really been groundbreaking at FDA. We focused our panel discussions often around cancer clinical trials but included very diverse groups. We're specifically proud of the fact that we were able to incorporate Native Americans, LGBTQ, Latinx, and African American communities in regards to cancer clinical trials. However, the image that you're seeing here is our last conversation on cancer, which was specific to COVID, 365 days and counting, which marked the year anniversary from when we left our offices and started working fully remotely and still got a lot of work done. And I think it's a good sign for us that we're still able to be impactful.

One of the things that I do want to share with you — because we do have very strong ties with AACR, the American Association for Cancer Research. Last year, in their virtual meeting entitled "COVID-19 and Cancer," I was part of a panel discussion, which — surprisingly, there was a question that led to a publication. This publication resulted because of a question about "What do we do to make sure that people are still keeping up with their cancer screenings?" And at the time, I suggested that perhaps a social media campaign might be in order. A publication resulted instead, and I'm happy to be a part of that.

But I'm going to tell you a little bit more about our social media campaign in a moment, which is sort of another dovetail into ways that we can engage others in the work that we're doing, because, obviously, there's no one right way to engage people. But we do want to make sure to take advantage of all opportunity. On this slide in particular, I just wanted to share that, as many of you know, the COVID pandemic has devastated many families across the nation and around the world. And we know for a fact, from NCI modeling, that at least an additional 10,000 cancer deaths in breast and colon cancer alone will result due to screenings that didn't take place due to COVID. And so — lay this modeling — and by the way, that's only one sixth of all the cancer deaths annually. So when you lay this modeling over the historic cancer inequities in the most vulnerable populations, it's pretty evident that the greatest cancer death inequity and disparity, comparable to COVID, actually, compared to the rest of the country, is among black Americans.

And so, this prompted me to think back to that social media campaign that I mentioned a slide or so ago and reflect on a conversation that I'd had with our OCE director, Dr. Richard Pazdur. And he had asked me at one point if there was a National Black Cancer Week or Month. I wasn't able to find one. And so, tying this all up in a nice little bow, we decided Project

Community would launch off and create a new initiative. So this year, we're pleased to announce that coming up next month will be the first ever National Black Family Cancer Awareness Week. It takes place June 17 through the 23rd.

The information here available for you — and also try to drop in, if I remember — in the chat, the exact web page which I am going to make evidently clear to you, even if you don't go to the chat. This initiative is one that we're happy about, regarding the timing, because also, this is the 50th anniversary this year of the signing of the National Cancer Act. So we feel like we're in line with a lot of immediacy regarding the necessity for addressing this issue, which is an ongoing issue. Cancer was here before COVID and will remain after.

Let's see. Something interesting is happening with my Cisco Connect. And please tell me if you're not seeing it, and I will go on. Anyone? I think I may have been disconnected.

SPEAKER: Rea, we can hear you, yeah.

MS. BLAKEY: Okay. Well, I don't know if you're still seeing the slides, but I'll just finish talking in case it all syncs up again. Okay, excellent. Thank you, I appreciate that.

The campaign that I was referencing, National Black Family Cancer Awareness Week — actually, if you search that term — that title, you'll find the social medial toolkit, which will show you everything you could ever want to know, including the panel discussion that takes place on June 17 from 2:00 to 3:30. And there is a registration required for this, but this is a panel discussion that is open to the public, and we just want to make sure that people are aware of it.

One other just little point of interest I was talking about — the appropriateness of the timing, and that is that the very first presidential executive order of the new administration is called Advancing Racial Equity in Support for Underserved Communities Through the Federal Government. It was signed January 20, and we're happy that we believe our campaign is actually representing that. I apologize, because I can't see this slide anymore, so I'm just going to try wrap up as quickly as I can, for fear that none of this is syncing. But basically, if you go to the National Black Family Cancer Awareness web page, you will find — now I see a slide, so I'm hoping we're all in sync — you will find a variety of graphics that are designed to be used and distributed widely through the public via Twitter, via Facebook, via Instagram.

This campaign is totally volunteer. If you choose to participate, we're thrilled. It is all-inclusive; no one is excluded. It's very much designed to be a grassroots effort. At FDA, we have a lot of opportunities to reach out to larger organizations, and that's fabulous, but we also want to make sure that we're reaching some of the people who may not otherwise engage with FDA and particularly, when it comes to cancer awareness.

So I'll try to quickly advance the next few, so that you can just get a sense of some of the other opportunities that exist, and then end here, reminding you that we're having the panel discussion, which is open to the public, on June 17 from 2:00 to 3:30 Eastern. And then the rest of the week, because I have mentioned specifically that it's a week, we're asking people to participate in the social media campaign, which is, again, voluntary, non-prescriptive. So, if you're deciding that you'd like to have a series of posts that show cancer caregivers or others in your life or community that you just want to celebrate and recognize during the inaugural National Black Family Cancer Awareness Week, we're thrilled to have you do that. Please do use the hashtag #BlackFamCan, and then that will, of course, unite all of the energy that we're all going to put into this to try to increase cancer awareness. Just wanted to make sure to

highlight this opportunity, because after all, you're a little bit of a captive audience, and I would be remiss for not mentioning it.

So I will conclude my comments with that and just let you know that these are a few of the other options that are available for patient engagement if you go to [FDA.gov/OCE](https://www.fda.gov/OCE), and we are thrilled to have your feedback on any of the work that we're doing. And any opportunities that you'd like to engage with us, please don't hesitate. We're very much interested in patient engagement, as is the rest of FDA, and it is the lifeblood of what we do. So thank you again, CBER, for allowing us to participate, OCE, in this particular session. And Karen, I will hand everything back to you. Thank you so much.

MS. JACKLER: Thank you, Rea. And thank you, Michelle, Sadhna, and Andrea. Again, it's a pleasure to have you all here with us today.

So let me advance — okay, there we go, okay. So, again, I'm Karen Jackler, and I'm with the Center for Biologics, and I'm the patient engagement program manager at CBER in the Office of the Center Director. And I am going to give you an overview of CBER's patient engagement activities. We're going to try and bring it all together. You've heard a lot of different programs, a lot of different avenues and channels for engagement. And so, I'm going to try and — just in a couple slides — try to sum up where some of these things sit — or things that are sort of more general mechanisms for you guys to consider. And then I'll have a couple of examples on how we collaborate across the centers, because I think you've heard that a program might reside in CDER or it might reside in CDRH. But there's a lot of ways that (audio skips) information isn't siloed and that it does spread across the — when you come to us, information moves around FDA.

So I will just keep on going here. So CBER's patient engagement activities are represented by these four different groups of activities. All of these are involved in patient engagement and using patient input in different ways. But we stay connected with each other inside our center.

The Patient Engagement Workgroup serves as the center's hub for sharing information about patient engagement activities. In that workgroup, we convene representatives from all of the CBER offices, in order to share information, gather ideas and input on patient engagement. We'll often invite guest speakers from inside the center and from other centers to inform us on what kind of activities they're up to, what kind of research they've been doing, so that we're constantly cross-pollinating and sharing information with one another.

The workgroup also serves as a source of communication for CBER staff on what patient engagement activities are open, how review staff and epidemiological staff — any staff, really, in CBER — can learn directly from patient advocacy groups and from patients. And then each of the offices inside the Center for Biologics — and this includes the Office of Tissues and Advanced Therapies, who has convened us today, the Office of Vaccine Research, and the Office of Biostatistics and Epidemiology — have a strong presence on the workgroup as well. And what they do is, they go back to their individual offices and further disseminate the information. And oftentimes, they're involved in identifying staff. When patient advocacy groups come to the FDA and they say, "We would like to talk to you about a certain topic," it's these people who go to their offices and say — and identify the people who — their portfolio or their expertise or whatever they're working on aligns with the things that you would like for us to hear. So you're getting to the best experts. So we're trying to get the right people around the table who are going to be able to make the most of the information and the most of your time as well.

The set here in the green box, the Rare Disease Coordinating Committee, is an important part of our patient engagement activities. They are — the mission of that group is focused on rare disease efforts, particularly around regulatory flexibility with rare diseases. But part of their mission is also outreach on CBER's rare disease efforts. And that has a nice crossover with patient engagement activities. So those two groups work very closely together to share information. We talk often. The Rare Disease Coordinator, who is Julie Vaillancourt, if anybody of you know her, will work together to talk to patients. We'll talk to patient groups together often.

Okay, in the purple box here is the Science of Patient Input Initiative. And this group resides inside our Office of Biostatistics and Epidemiology. And they provide consultation to review staff on patient-reported outcomes and similar clinical outcome assessments submitted by sponsors. Or they are open to citing feedback to groups — to patient advocacy groups who are working on clinical outcome assessments that are derived from patient input. They also conduct research on patient input. And they use that to demonstrate how patient input can be used to inform endpoints or to generate reliable data that can be used to inform FDA's decision making. Let's see. So that research that they do play an important role in bridging from a listening session or other qualitative data sources, to patient-centered tools that can be used to support things like benefit with certain terminations. So they are a very valuable part of the patient engagement program at CBER.

And then the fourth component and fourth sort of partner inside CBER is the Office of Communication, Outreach, and Development. So, they are the group that — as you can imagine, being the Office of Communication, they are a public face for the Center for Biologics, and they are the ones that will field questions from patients and patient organizations. They are also very involved in organizing meetings with patients. So, they will often be the one that is working with a patient group in order to find a time, find the people to be able to meet with you, if a meeting is something that people mutually find is going to be beneficial.

So those are sort of the four big patient engagement activities. It's sort of the program as a whole. So we don't have a staff, so to speak, but we do have a program that pulls together some very key elements from across the center to make patient engagement accessible to everybody at the center.

So you've heard from CBER; you've heard from CDER, CDRH, OCE, and the Office of Public Affairs. You've heard a lot about different mechanisms and different programs. And so, this is the part where I tell you — I wanted to zoom out a bit and help you kind of organize all this information that you've heard, and what might be the best mechanism for your — to help you kind of figure out what mechanisms might be right for your patient group and what opportunities — where to find those opportunities to be across the FDA.

So this slide was sort of in-common opportunities. I think you've heard this a couple of times. I think Sadhna mentioned this as well, and then Dr. Anatol in Session 1 also mentioned it. So these are several well-established mechanisms that people with disease and disorders and the stakeholders who represent them can use to provide input to the FDA. And they are common to all of our centers. Meetings with patient organizations are opportunities for people to provide specific input or to present to a center on — maybe you conducted a study that involved getting patient input and you'd like to provide the agency with an opportunity to listen to your results and have people ask questions; that's a mechanism that's common across all the centers. Public meetings and workshops, such as advisory committee meetings you've heard — so you know that that is way to come to the FDA that is common to all the centers and the offices. And you heard people talk about the special government employees who can sit on the advisory



committees and who also the centers can use — can ask to provide input on maybe specific issues they have when they're reviewing a drug development program.

And docket comments, which — in the docket comments, we offer the opportunity to comment on Regulations.gov. These often follow the release of the guidance, or they're opened after a public meeting to allow additional comments to be made after the meeting is over. And I want to assure you, these comments are read. And we invite you to use that mechanism. Oftentimes, groups will submit a comment on behalf of an organization they might represent. But individuals are also welcome to comment as well. And then there's always the tried and true ways to reach the agency through emails, letters, and phone calls.

And then let me move on to this next slide here. You heard a lot about programs that reside in specific centers. So Sadhna from the Center for Drugs spoke to us about patient-focused drug development and about the patient and stakeholder engagement program, PASE. So that resides in CDER. Michelle talked to us about the Patient Engagement Advisory Committee for Medical Devices and the Patient and Caregiver Connection that resides in CDRH. And we heard from Andrea, who talked to us about the NORD listening sessions and the Patient Engagement Collaborative. And then we just heard from Rea about private community and conversations on cancer, as well as a whole number of other initiatives that they have. So I think — what's important about this slide is — if you kind of see, I've tried to make connections here between everything. Because even though those programs reside in those specific centers, we work together to bring more than just one office or center to the table to hear your voices. So, if you approach Sadhna in PASE about a meeting, that meeting information will be shared with CDER and CDRH and CBER to see if there is any cross-center interest in it. And most often, there is, because patient concerns cross these program areas. And so, often, if you approach one of these — one of our centers or offices, you're going to actually find out that you're going to have reached out to all of us in some way. And as you know, there are designated staff in each center, many of whom are on this panel, whose job it is to facilitate patient engagement and to facilitate these connections that I've tried to make you see here.

And then, in addition to the people whose jobs are specific to facilitating patient engagement, when you kind of look at the spokes at staff, staff are actually talking to one another. Review staff talk to one another across parallel programs in the different centers. Specialists talk to one another across programs in the different centers. And epidemiologists also, specifically those who are involved with clinical outcome assessments and the science of patient input, the patient input studies, also talk to one another. So I just wanted to bring that home for you — that even though you're hearing from different centers about these programs, there is a strong effort to collaborate and to make sure that information is not siloed.

And then I just wanted to give you a couple of examples of how this sort of cross-center collaboration and information sharing has happened. All right, so you've heard about a couple of listening sessions, and already I was taking some notes about some that you've heard of. I know that some specifically mentioned were the GSD1A and others. So there have been some listening sessions that have also been — both CBER and CDER have planned them together, because they have similar questions. They requested — CDER and CBER requested one on Hunter syndrome, MPS II, childhood cerebral adrenal leukodystrophy, Sanfilippo syndrome, and then, as we mentioned, GSD. So those — CBER and CDER had similar questions, and so they worked together on the questions to ask the patients. And the PFDD program — even though it is a program that is originated and sort of resourced out of the Center for Drugs, CBER has planned PFDD meetings, too. We have been — we planned one on Alpha One Antitrypsin, hemophilia, heritable bleeding disorders, and hereditary angioedema. And we are also —

presently, we work with CDER to be technical advisors for externally PFDD meetings. For specific ones, when there's a very strong connection to the CBER products as well, then we will work together to provide technical advice to the patient groups that are organizing an external PFDD. And then, as you know, if you've ever had a listening session through Andrea's group in the Office of Public Affairs, those are always cross-centers, there are always multiple offices and centers at those meetings. So I just want to drive home the idea that there is an opportunity to talk to more than one center at a time, and we make a lot of effort to make sure that that happens and that that information is spread throughout the agency when you come to us.

On the last slide, I think Dr. Anatol might have mentioned this as well, but we're interested in all aspects of patient input. The listening sessions are a great way to talk to us, but we're also interested in any reports that might have come out of any scientific meetings you have. I know a lot of patient groups may have world summits or national summits once a year or every couple of years. If you have reports about the outcomes of those, share those with us. If you have reports on natural history settings or patient registries, share that with us. If you're trying to figure out how to pull one of those together, that's something we're interested in hearing about. White papers, case examples — there are any number of ways that we can help you with patient input and to try and also put patient input into the greater context of your patient-focused drug development effort.

So I am going to wrap up there. And — moving to Q&A. So — if I can ask my fellow panelists to turn their cameras on and unmute. So, Andrea, I was just wondering, can you tell us how patient listening sessions have evolved over the years? You had mentioned that there's actually a very long history of FDA patient engagement. And it might be interesting to hear how the listening session itself has evolved and how it's sort of become more part of the culture, I guess, of FDA patient engagement.

MS. FURIA-HELMS: Yeah, happy to share. Before the formal program began and my formal role as managing the FDA Patient Representative Program, it began probably over a decade ago when divisions in various centers reached out. And they really wanted to connect with patients and caregivers, because they were — within their work, they were trying to better understand the disease experience and the priorities and what was important to patients so that it could inform their work that they were working on at that moment in time. So they began as really informal one-off discussions that I would pull together through phone calls. And then, when Patient Affairs was established, we thought that it might be a good opportunity to formalize the program. So we did a formal pilot to really make sure that the process was fully developed and that it was going to be meaningful, both for FDA staff and the patient participants. So we went through a series of pilot programs to really determine what was going to be efficient. And from that point, when it was launched in October of 2018, there's been really an uptake in the number of people requesting them internally and the number of organizations requesting them from external patient organizations.

So, last year, we had 16 listening sessions. And this year, we'll probably be aiming about that by mid-year. And in total, we've done, so far, to date, about 40. And I'll say that what has happened is, they've sort of been high-level, in the beginning, really focusing on the disease and treatment burden. And the priorities now, especially the internally requested ones from FDA staff — they're really becoming more and more specific and really trying to understand real specific issues and experiences that patients and caregivers can share.

MS. JACKLER: Thanks, Andrea. Let's see; let me take a look here. I've got several questions coming in. So — and this is for everybody: How can patients and caregivers get more involved

in the regulatory process? Anybody want to — maybe Michelle? I think your career has sort of spanned several roles, so maybe that's a good place for you to start. And then others can chime in.

DR. TARVER: Well, I mean, I think, as I said in the talk, across the total product life cycle, I mean, at any point, patients can reach out to us and we are looking to involve them in more and more of our work. I will say one aspect that our center has really tried to help bring into the daily work of each reviewer is the importance of trying to include the patient's voice as much as possible, whether it's structured outcome assessment or if it's having a listening session or conversation with patient groups. We have an upcoming one, actually, Friday, with an adolescent population living with scoliosis, where they're sharing how that disease condition impacted their lives, what the surgery did, and the devices that were used to treat that condition, how that impacted their lives. So, of hearing from them directly — I think really transforms the work we do, makes it more tangible, and also reminds us that what we're all here at the FDA doing is really wanting to help improve public health. And it solidifies that mission and kind of galvanizes us even more in the work that we're undertaking.

MS. JACKLER: I think something that we tell people and that I hear a lot at the different places is, "Come early and come often. Right, talk to us early and talk to us often." So, like you said, Michelle, the total product life cycle is important. And becoming — I think what we're seeing more and what's been some of the successes we've seen are groups who have been sort of working in those very early phases, trying to figure out ways to fund research and fund screening tools and sort of providing that seed — that very seed money to kick things off. So I think that there are plenty of opportunities to get involved, and one of them is to start early and don't wait until a clinical trial has already started to start with your patient input. You can — patient input is valuable at all points in the life cycle. But I would stress: Don't make the first time you come to the FDA at the kickoff of your clinical trial.

So, let me see. Sadhna, I didn't know if you wanted to chime in.

DR. KHATRI: Sure. I agree with Michelle and Andrea, what they have mentioned before. Patient input is very valuable at any stage for the life cycle of the drug product here at CDER as well. And also, not only patients are interested in coming and talking to us and sharing their perspective, but also, I would just like to mention that our medical reviewers — they are also equally interested in understanding their unmet needs and what are the symptoms that they are trying to address. In my presentation, I shared the BDSA example, and it was really eye opening for our medical reviewers to hear from the community how the treatment — even though the treatment exists for certain conditions for that community — still there was so much unmet need that was not being met. So it's always, as Michelle said, always valuable to hear from the patient their perspective and their language. And it really, really matters to the (inaudible) agency and the medical officers who are reviewing the product.

MS. BLAKEY: Karen, can I jump in quickly?

MS. JACKLER: Yes.

MS. BLAKEY: Okay. A couple of the items that were mentioned previously in my presentation were about Project Equity, which is specifically designed to do exactly what you're talking about to encourage that early inclusion and to make sure the industry knows that it's important to us to have full representation across oncology clinical trials. And then, also, another that I mentioned was Project Silver, which — again, because cancer tends to be a little bit more age related, if

you will; certainly, the older you are, the greater your possibility of developing a cancer. And so, we wanted to make sure that that inclusive aspect was communicated in all the work that we do.

And then, obviously, I think, across the agency, if people are not aware, there are those funding mechanisms that you briefly touched on — things like broad agency announcements, which I think, internally, sometimes we even struggle with exactly what is all of that. But again, if you go to the FDA.gov website, you will find a web page specific to broad agency announcements, which can be used as mechanisms for gaining more science about the things that FDA is interested in. And we do want to have those opportunities with people across the entire cancer continuum, which is sort of the area that we focus in, but all across the agency in all therapeutic areas, there are opportunities for people to figure out if there's a way for them to help us advance the science. So that, too, can be very much patient-centered to give people a perspective of really getting that input in early and often and to be a part of the science that creates the products that we're responsible for.

MS. JACKLER: Right. Collaboration in all kinds of different forms, with the agency, across organizations — I think there's lots of opportunities for that.

So I'm actually going to thank everybody who asked a question. And also, I think there are — there was lots of activity going on in the Q&A in the chat, and some people were answering questions in there. So thank you for that. That's a very efficient use of the chatroom there. Thank you all, panelists. It's always a pleasure to talk to you all and hear from you all. And I think I am going to wrap this up and let us move on to the next, our next session. Thanks, everyone.

DR. ROWZEE: Thanks, everyone. That was fantastic. Just to reiterate what Karen said, thanks to all the panelists for their — another great discussion. Thank you for your time today. Also, thanks to the folks who've been working behind the scenes, sort of answering some questions via chat. I know there's a lot of resources that have been shared that way. Also, just to remind everybody, we'll have a recording of today's workshop available on our website, as well as slides from today's meeting. So you'll be able to capture all those resources that have been shared earlier today.

So I'm going to move into the final sessions for today's workshop. This is a question-and-answer session with two of my OTAT colleagues who have broad experience in patient engagement: Dr. Tejashri Purohit-Sheth, who is the director of the Division of Clinical Evaluation and Pharmacology/Toxicology in OTAT — it's a mouthful — and Dr. Sandra Retzky, who's a medical officer and a patient engagement lead within our DCEPT division. As a reminder, you can use the Q&A chat window. I don't think folks need that reminder; you've been doing a great job with putting your questions in there today, but just to reiterate. And we'll do our best to address as many as we can.

I'm going to hand it over to Tejashri now to give a few opening remarks, and she can then pass over to Sandy when she's finished. And Tejashri, can you hear me?

DR. PUROHIT-SHETH: Yes, I can hear you. Can you hear me?

DR. ROWZEE: I hear you, and if you want to start your webcams, if that's available, go ahead.

DR. PUROHIT-SHETH: Can you see me and hear me?

DR. ROWZEE: Yep.

DR. PUROHIT-SHETH: Okay, great. Well, good afternoon, everyone. It is a pleasure to be here today. My name, as Anne said, is Tejashri Purohit-Sheth, and I'm the division director for the Division of Clinical Evaluation and Pharmacology/Toxicology. In medical school, I think one of the first things we actually learn that the most important piece of information is the history when we talk to any patient. And so, in clinical practice, what the patients tells us, their perspective, what they're feeling, what they're sensing, what their symptoms are — all of these things — that is the most important aspect of what we do as physicians in clinical practice. And it's great to see us as an agency moving forward in gaining information and getting information from our patients, understanding the patient perspective.

And I will tell you I started in 2002 at the FDA as a medical officer, clinical reviewer, in CDER. And one of our first — you know, we oversaw at least my division, files for asthma, COPD, allergic rhinitis. And we actually utilized patient input — patient experience as one of our primary endpoints. So when we looked at files for allergic rhinitis, we looked at the total nasal symptom score. And that score, that primary endpoint was actually based on our patients' experience. It was based on their diary data and what they told us. So even back then, in the early 2000s, we were actually utilizing patient feedback, perspective, and input in our regulatory decision making. We have since, in the last several years, attempted to formalize our patient engagement activities. And what I am looking forward to is continued engagement with our patients and our stakeholders in utilizing patient perspective in our regulatory decision making.

Sandy, I'll turn it over to you now. Sandy? San, can you still hear me?

DR. ROWZEE: I can hear you, yeah. I think we're getting Sandy connected. Well, anyway, I can see her video trying to get started. Sandy, if you can hear us, we're having a little bit of trouble connecting with you.

DR. PUROHIT-SHETH: While we're waiting while Sandy is trying to connect, I can also tell you that I've lived in CDER for some years. And then, I actually went to CDRH for close to five years. And it was in CDRH that I was first exposed to one of our strategic initiatives which was patient engagement, patient experience data, and utilizing this in our regulatory decision making. And we actually did a very nice job of trying to formalize the process in that center and one of the first experiences that I had in a patient perspective study was as part of a marketing application. And this was trying to gather feedback from patients as to what is their risk tolerance. So we had a device for a condition, and there were some unique risks to that device. But then we also — the other option or standard of care was surgery. And of course, any surgical procedure and a major surgical procedure is not without risk and issues for the patient. So the patient practice study was intended to identify risk tolerance, and understanding the risk was critical to procure a perfect feedback from our patients that participated in the patient preference perspective study. So I think that we, as an agency — all of the centers are moving forward, trying to glean information for patients and to understand their perspective on a variety of issues.

In our center — now I'm in CBER — we have utilized patient perspectives in a variety of different manners. Of course, the first example I gave from CDER was utilizing patient reported outcomes as patient feedback, an important piece in our regulatory decision making. But we participated in the patient-focused drug development, both those that have been led by FDA, as well as the externally led PFDDs, and we found their input very, very valuable, especially for conditions that we oversee and products that we oversee. We've also participated in listening

sessions and think them very, very valuable. We oversee cell and gene therapy as part of our therapeutic modalities that we oversee within our office. And understanding, again, risk tolerance for gene therapy — and it's very different, depending on the patient population and the particular condition that a patient has. So patients who have viable other options out there, they may not, right away, want to involve with some of the risks that are inherent with gene therapy. But for patients where perhaps there is no therapeutic option, no modality available, then they may actually, from a risk–benefit perspective, choose to utilize gene therapy as a therapeutic option. So I think it's very important for us to understand how patients feel and what their perspectives are.

We have also utilized patient input and patient feedback in our program development, and we had one application, so Luxterna is a biologic license application is a product that has been approved for a rare retinal dystrophy. And it was the gene therapy, very novel product, and there were no products out there for that condition. So when the regulatory program was progressing and we were getting towards the — trying to design a clinical trial, the question came up, what should the endpoint be? And so, we actually engaged with — in a special patient group and got feedback from patients as to what they would find meaningful. So it actually utilized the patient input and feedback in the course of regulatory development to assist with identification of appropriate endpoint.

So I'm going to go see if Sandy is available now.

MS. RETZKY: Can you hear me now?

DR. PUROHIT-SHETH: Yeah, we can hear you.

MS. RETZKY: I think my webcam is not working. Is that right?

DR. ROWZEE: It's a two-step process where you have hit the start and confirm or something like that.

MS. RETZKY: I did start, and I also shared.

DR. ROWZEE: Yeah. So something must be going on there. Maybe there's a cover or something. But we can hear you, which is the important part. So Tejashri gave us a really nice overview of sort of what her different roles in FDA and how patient engagement experience sort of shaped the work that she's done, both pre-OTAT and now within OTAT. So, if you want to sort of introduce yourself, talk a little bit your work at FDA and your work as a patient engagement lead for OTAT.

MS. RETZKY: Sure. Well, good afternoon, everyone. My name is Sandy Retzky, and I'm a medical reviewer. I work with Tejashri, and primarily, I work on gene therapies for rare diseases, so I'm really pleased that I get a chance to speak with you today.

By training, I'm a pharmacist as well as a physician, and I'm also a lawyer as well. And the first time I really understood the impact of patient engagement was when I was in industry. I worked in the pharmaceutical industry for 12 years before I came to FDA. And we would periodically have opportunities to develop drugs for rare diseases. And we wouldn't have any endpoints. And if you don't have any endpoints to be able to demonstrate to provide evidence that your product works, it's impossible to get something through the commercialization process and on the market. So it really struck me during that time how important this was. In the pharmaceutical

industry, we would talk to patients and discuss what was important to them in terms of an endpoint.

And I remember, I had this one file — this one program that was so interesting. It was for this very rare disease called eosinophilic esophagitis. And this is something that would happen to children where they would have a very difficult time swallowing. And if they can't swallow, they can't eat. And so, it became — what was really important to them was things like being able to get off tube feedings, to be able to eat with everybody else at the school cafeteria. And one thing that was most important was to be able to have birthday cake. And it really took me aback at how much I, just going through my life, I hadn't appreciated how important those kinds of things like having a birthday cake and being able to sit in the school cafeteria with everybody else, how important that was. These kids would have to go — for lunch, would have to go to the nurse's office to get a tube feeding. And that was — it really stigmatized them.

And so, that's the first time I really began to appreciate how important it was to have endpoints well in advance of developing a product, because for a pharmaceutical company to not know what the endgame is, to have to start with developing tools, to be able to test the endpoint in a clinical trial — that usually takes a couple of years. And so, to the extent that these tools to measure endpoints, how you feel, function, and survive, if those are already available, it really puts things ahead in terms of drug development. So that's something I really wanted to emphasize.

Since I've been at FDA — it's been about 5 years now, 2 of which I've spent working on rare diseases and gene therapy — very often, the — I find that the tools to measure the endpoints aren't really well-studied. And so, what happens, I find, is that sometimes the drug therapy is out ahead of the work that needs to be done to make sure that the endpoint is something that we can measure and that matters to patients and their caregivers. So, to the extent — I think I'm going to echo what so many people have said today: The earlier we can start on this, the earlier FDA can participate, the better off we can be for patients. So I think early engagement with FDA is important, and I heard — I think you probably have heard "early, often." And I think that's true as well, because, you know, we live in a technologically fast-paced environment. And the way certain diseases are being treated, even in the past 5 years, 2 years, has changed dramatically. So we always have to be keeping up with not only how the patients feel, function, and survive but how that works with the current standard of care, because that's also changing. So I think the more we interact with patients, the better off we'll be. And I'll turn it back to you, Anne.

DR. ROWZEE: Yeah, thanks, Sandy, and well said. And that really kind of leads into — we have a couple minutes for questions, and it leads into really that first question. You were saying early — we've heard it all day that work — for companies and patients to begin work together early. And you've really driven that point home for us. There you are, we see you. Great. So happy to see you.

And FDA involvement at that time — so how can FDA get involved in these early activities? You know, we've heard about some of the different meeting types today. Would a patient-focused drug development meeting be an appropriate venue to sort of talk about things like trial design or endpoints or things that are important to patients and caregivers?

DR. PUROHIT-SHETH: I can start. I think when we say, "Come early; come often," we are really interested in how patient perspective — patient experience informs drug development — the whole program. And the earlier they can come — that can actually — the earlier the meetings, the earlier feedback that they procure from us can help support designing of the clinical study.

So, from what symptoms we should — what symptoms are important to patients — so these symptoms we can potentially focus on — monitor for and then — and if you're looking at a primary (inaudible) endpoint — and Sandy mentioned that — we may look at a condition or a disease and say, "Oh, we think that this is really important," but a patient may say, "Oh, no, this is the most important aspect of the condition."

So for example, one of our patient-focused drug development programs was on hereditary angioedema. Now, as regulators, the most critical and life-threatening consequence of hereditary angioedema is the laryngeal spasm, right — is the angioedema or the swelling of the throat when the patient can't breathe. It's a life-threatening situation with a potentially fatal outcome. But when we had our patient-focused drug development — so we heard how debilitating the swelling of the intestinal walls, et cetera — how debilitating that aspect of the condition was. And as a regulator, we'd want to ensure that — for future studies that we actually take those symptoms into consideration in the study design and that these were so debilitating, patients would be out of work; they couldn't hold a job — how impactful some of these symptoms are for them.

So when we think of designing clinical studies, we really do want to have an understanding of what bothers the patients the most and what we can do to evaluate and support evaluating really clinically important symptoms for the patient. So, if they come early, it can facilitate designing the study so we can assess what is most important to the patient.

DR. ROWZEE: It's so nice to hear some of the specific concrete examples about how it works in practice. And so, I think we've sort of probably generated a lot of energy among our patient groups today, and I think they're probably really excited about what they could do next. And I was wondering if you had any thoughts about what patients or their advocacy organizations can do to sort of help advocate for research and development of regenerative medicine therapies. If they come together, where can they focus their energies? And Sandy, maybe I'll kick that one to you first.

MS. RETZKY: Sure. So I think there's a couple of things to consider. For the advocacy groups, it depends on the disease and what — you know, if anything's been developed, if there are already tools that are available to measure differences in improvement, whether those are available or not — if there are tools available, I think that's a good place to put effort. I also think it's very helpful to have a registry, if there isn't already a registry, if it's a rare disease. So both tools and the registry — patient advocacy groups can come to FDA and ask for meetings for help with both of those. There are pathways to FDA to get some advice.

As far as working with pharmaceutical companies, we see that all the time and we encourage — just so you know, we encourage all of our pharmaceutical companies and biotech companies when they come to talk to us. If they want to bring patient representatives with them, they are welcome at the table, and we like hearing from patients. And it's something that I think we could use more of. We don't see it happen very often.

I just had a meeting last week where there were patient representatives, both — parents as well as children, and I thought that was really helpful. It was good for the patients just to hear the back and forth between the regulators and the pharmaceutical companies. So I thought that was a very good experience for them.



So those are some of the ways that patient advocacy groups can get involved. We also sometimes get requests from advocacy groups if they have meeting for an FDA representative to come. And we do that, too. We will send people out to those meetings.

DR. ROWZEE: Tejashri, I'll turn it over to you. Do you have any final thoughts on that question before we begin to wrap up for today?

DR. PUROHIT-SHETH: Sure. So I think patient advocacy groups can be involved in a variety of different activities, and Sandy mentioned several. I think that there are patient advocacy — there are different manufacturers and sponsors that do a lot of work that may potentially be repetitive. The patient advocacy groups can work on trying to get our sponsors to leverage existing information. That's very valuable information. The other place is, like Sandy said, a registry — natural history studies, especially for rare disorders where the natural history is not very well understood or delineated. It would be very helpful to have the natural history data that can actually inform the design of future clinical studies, you know, so what should we be focusing on with respect to signs or symptoms? What is most bothersome to patients? And also, patient-reported outcomes are very important. If they can try to validate quality of life instruments or clinical outcome assessment instruments. Those are very, very helpful to us in our regulatory decision making. So I think — and also, when there are opportunities to participate, as Sandy mentioned, inside these meetings we have with our sponsors, we welcome patient feedback and patients to come to these meetings. So we, in our division and our office, are encouraging our sponsors to request that patients participate in these meetings. And sponsors have the ability to bring patients to our internal meetings, our sponsor meetings with them, to discuss their perspective.

DR. ROWZEE: Excellent. And yeah, I was in that meeting with Sandy last week, and I agree: It was really great to hear a patient or caregiver provide their perspective. So thanks again. And just thanks to you both for your time today and your persistence in getting online with us. It was great to see you both. We don't get to see each other very much anymore. So thank you again. It was really good. And I think the information we've heard is really valuable for our audience.

So I'm going to now actually — before we wrap up, before we close, we actually have a quick thank you from our director, Director of OTAT Wilson Bryan. He wanted to share a few remarks. And you'll need to use your computer audio to hear the video, so make sure you turn that up. And we'll get that video started. Thanks.

MS. RETZKY: Thank you.

MR. BRYAN: Good afternoon, I'm Wilson Bryan. I'm director of the Office of Tissues and Advanced Therapies for OTAT in the FDA's Center for Biologics Evaluation and Research, or CBER. I am honored that you participated in today's workshop. I hope that you found the conversation to be informative and insightful. As you heard today, OTAT, along with all of the other medical product centers at the FDA, is committed to creating opportunities for communication and collaboration that will help us to better understand the perspectives of patients and caregivers. Your input gives us a better appreciation of patient needs and helps us to advance the development of new, regenerative medicine therapies. The field of regenerative medicine has grown exponentially and holds much promise for treatment of genetic diseases and treatment of rare diseases.

Our goal today was to share how FDA is working to ensure that innovative products are safe and effective. And we wanted to start a dialogue with patients, advocates, and caregivers

around continued collaborative opportunities. I hope today's event has empowered you to help advance the state of the science and the development of treatments for a specific disease or condition. Our presenters today shared several opportunities for collaboration. Please consider taking on projects, like natural history studies or patient registry programs. Consider hosting a patient-focused drug development meeting or a patient listening session. Or maybe you can join forces with other groups targeting the same disease or a related disease or condition and work with others to focus and streamline your efforts. Thank you so much for being a part of today's event. I look forward to continuing this conversation in the future. I now turn it back to Anne Rowzee for any final thoughts.

DR. ROWZEE: Hi, everyone. As Dr. Bryan said, I just wanted to share a final thank-you to everyone for attending today's workshop and for sharing your questions and comments. I also want to thank those who have spread the word about our workshop among your communities by word of mouth and by social media. You've made it a success by helping us to reach the patients and organizations we were hoping would join us today. In the coming months, OTAT will be developing and sharing patient educational materials about regenerative medicine therapies. Please follow us on Twitter and visit our website to stay updated on the latest news and resources. We look forward to our continued work together to advance regenerative medicine therapies. Have a great day. (Whereupon, at 3:08 p.m., the PROCEEDINGS were adjourned.)

\* \* \* \* \* CERTIFICATE OF NOTARY PUBLIC COMMONWEALTH OF VIRGINIA

I, Yilinase Mqadi, notary public in and for the Commonwealth of Virginia, do hereby certify that the forgoing PROCEEDING was duly recorded and thereafter reduced to print under my direction; that the witnesses were sworn to tell the truth under penalty of perjury; that said transcript is a true record of the testimony given by witnesses; that I am neither counsel for, related to, nor employed by any of the parties to the action in which this proceeding was called; and, furthermore, that I am not a relative or employee of any attorney or counsel employed by the parties hereto, nor financially or otherwise interested in the outcome of this action.

(Signature and Seal on File)

Notary Public, in and for the Commonwealth of Virginia