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RegenMedEd: An FDA CBER OTAT Webinar Series

Regenerative Medicine 101: Information for Patients, Caregivers & Advocates

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Welcome

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Regenerative Medicine 101: Information for Patients, Caregivers & Advocates

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Q&A/Closing Remarks

PROCEEDINGS

(11:02 a.m.) DR. ROWZEE: Good morning. Hello, everyone. Thank you all for joining today's webinar, titled "Regenerative Medicine 101: Information for Patients, Caregivers & Advocates." Today's webinar is hosted by the Office of Tissues and Advanced Therapies or, as we often say, OTAT, within the Center for Biologics Evaluation and Research at the U.S. Food and Drug Administration. We really appreciate your attendance at today's webinar and for the opportunity to talk with patients, patient advocates, caregivers, and other important stakeholders about regenerative medicine. My name is Anne Rowzee. I am Associate Director for Policy at OTAT, and I'll also be your host for today's webinar.

Before we get started, I'd just like to share a few notes with you. The webinar is being recorded; the recording and slides will be posted on the FDA website in the next few weeks. Closed captioning for this webinar is available via the link posted in the chat box. We do have time reserved at the end of the presentation for questions. Please use the Q&A function if you'd like to ask a question. And we also have the chat box available if you'd like to share general comments. Regarding questions, note we're unable to answer questions about specific medical conditions or diagnoses, but we encourage you to discuss those questions directly with your health care team. We also understand that you might have questions about the status of specific investigational products or drug applications. However, there are laws that FDA must follow that limit the information we can provide about investigational products. We do want to emphasize how important your voice is and that we're committed to helping advance the development of regenerative medicine therapies. We appreciate questions and comments, and we'll do our best to address as many questions as we can. Finally, for any technical difficulties, please use that chat box function and someone will assist you.

All right. To get started, I'm excited to announce that OTAT is launching a new webinar series called RegenMedEd. Today's webinar is the first of hopefully many webinars in this new series. Some of you may have joined our half-day patient engagement workshop that we hosted in May of this year. Because of the great response to that workshop, OTAT wanted to give more

frequent opportunities for engagement with patients, caregivers, advocates, and other stakeholders. The goals of the webinar series are to bring together these stakeholders to discuss regenerative medicine therapies, including things like gene and cell therapies, as well as to explore opportunities for FDA, patients, and advocates to work together and help advance these important therapies. The plan is to host these webinars on a quarterly basis and feature a variety of perspectives and experiences from FDA staff, patients, researchers, and other experts.

As we think through additional topics and educational resources we'd like to put together about regenerative medicine therapy, we appreciate hearing perspectives from you. So following today's webinar, please use the hashtag #RegenMedEd on your social media channels to share ideas for future webinar topics or other educational materials that we could provide. With that, I'm now going to turn it over to Dr. Wilson Bryan—he is director of OTAT—to kick off our RegenMedEd series with his presentation on Regenerative Medicine 101.

Wilson?

DR. BRYAN: Thank you, Anne. And thank you all for taking the time to join us for our first webinar. We have started to see the fulfillment of the promise of regenerative medicine through gene and cell therapy. These are lifesaving and life-changing products that address unmet medical needs for patients. Patients, patient advocates, and caregivers have an important role in advancing regenerative medicine therapies. Patients and their families are experts in their diseases, and their voice and your voice can be especially important in drug development. When it comes to talking about regenerative medicine, there is so much for us to discuss, which is why we're excited to launch this RegenMedEd webinar series. Next slide.

FDA's mission is to ensure that drugs, biological products, and medical devices are safe, effective, and secure for the public's health. In short, we at the FDA want to make sure that drugs and therapies are proven to be safe and effective for patients. In addition to approving

drugs and therapies, the FDA is responsible for ensuring that clinical trials are safe for patients to participate in. We inspect facilities to ensure those facilities meet robust manufacturing standards. We also monitor drugs and therapies that are already on the market to see if there are any adverse reactions. We work with patients, stakeholder groups, and other important audiences to understand patient needs and to find innovative approaches and treatments. Next slide.

The FDA's a big place. There are lots of centers and offices throughout the agency that review data and ensure that drugs meet high standards for safety, quality, and effectiveness. The staff at FDA include highly trained scientists, doctors, pharmacists, public health experts, health communicators, and many others. And all these folks are committed to making science-based decisions that help patients and their families. Within FDA, the Center for Biologics Evaluation and Research, known as CBER and shown in the yellow box here, regulates many innovative biological products. Next slide.

So what do we mean by "biological products" or "biologics"? Biologics include diverse products such as vaccines, such as the COVID-19 vaccine; blood and blood components, such as convalescent plasma; cells; gene therapies; tissues; and therapeutic proteins. Biological products are regulated by the FDA and are used to diagnose, prevent, treat, and cure diseases and medical conditions. CBER's role is to ensure the safety, purity, potency, and effectiveness of biological products. Next slide.

This diagram shows, at the top, the Office of the Center Director, led by Drs. Peter Marks and Celia Witten, and then the various offices within CBER—highlighted or outlined in yellow, the Office of Tissues and Advanced Therapies, known as OTAT. It's one of the program offices responsible for regulatory oversight of biological products within CBER. Next slide.

OTAT's mission is to promote public health through a data-driven process, to provide regulatory oversight that helps ensure that medical products are safe and effective. In doing

this, we want to make impartial regulatory decisions that are based on data and compassion. Next slide.

Part of OTAT's responsibility is to provide regulatory oversight for regenerative medicine therapies, which we sometimes we refer to as RMTs. Regenerative medicine therapies are defined by law in the 21st Century Cures Act, which became effective in 2016. A simple definition of "regenerative medicine" is a medicine or treatment that replaces or regenerates human tissues, cells, or organs to restore or establish normal function. Regenerative medicine can involve using stem cells, engineered biomaterials, gene editing, and other scientific technologies to repair or replace damaged cells, tissues, organs, or genes. Regenerative medicine is not simple; it's complex. And it's important to note that while regenerative medicine has been around for decades, it continues to evolve and progress through scientific advancements. That's one reason we're here today, to talk about some of the advancements we've seen in regenerative medicine. There are a few types of regenerative medicine therapies. These include gene therapies, which includes gene editing; cell therapies; tissues and tissue engineering products; and xenotransplantation products. Next slide.

OTAT regulates a wide variety of products that can be considered regenerative medicine therapies. While we don't have time to discuss all of them, let's take a look at a few of the gene therapies. In gene therapies, scientists can do one of several things, depending on the nature of the problem. Scientists can replace a gene that's causing a medical problem with a gene that does not cause a problem, so take a bad gene out and replace it with a good gene. Or they can add a gene to help the body fight or treat a disease. Or they can turn off a gene that's causing a problem. Gene therapy can be administered in several different ways. One of the most common ways to administer gene therapies by using vectors, such as viruses. There are several FDA-approved gene therapies on the market, and I'll talk about them briefly. Stem cell and stem cell-derived products are cellular therapies, and cellular therapies involve the transplantation of human cells to replace or repair damaged tissue or cells. The products listed here are just a few examples of stem cell-derived products. Next slide.

OTAT also regulates many products that involve the use of tissues, tissue engineering, blood- and plasma-derived products, and xenotransplantation. Xenotransplantation is really cool. This is any procedure that involves the transplantation, implantation, or infusion of cells, tissues, or organs from a nonhuman animal source into a human recipient. You may have seen in the news—I think it was last week—some scientists and researchers took a kidney out of a pig and transplanted that into a human who was brain-dead. So that—we don't regulate research in people who are brain-dead, but the idea is hopefully that someday transplantation of products from other species may help humans. I don't have enough time today to go into each of these different products and how they work. I just wanted to provide an overview of the wide variety of products that OTAT regulates. If there's a specific OTAT-regulated product that you're interested in learning more about, let us know in that chat box in Zoom, and we may consider doing a future webinar on that topic. Next slide.

As most of you know, FDA regulates drugs and therapies to ensure they are safe and effective for patients. The FDA has a rigorous analysis process to review data and ensure that decisions are based on scientific evidence. Regenerative medicine therapies, such as gene and cell therapies, go through this rigorous review process before they are approved for patient use. We monitor products before and after they come to market to ensure their quality. This means that we monitor therapies even after they are on the market, to make sure they continue to be safe for patients. In addition, the FDA provides oversight of clinical trials and products during development. This monitoring during development helps protect the patients who participate in the clinical trials. The greatest risk in drug development falls on the patients who participate in clinical trials, particularly the patients who participate in the first clinical trials of a product. We think of patients who enroll in clinical trials as heroes, and we owe them a great debt because they're helping to bring forward products to treat diseases, particularly very bad diseases. At FDA, it is our job to protect patients who participate in clinical trials, to ensure that these patients have reliable information before deciding whether or not they want to enroll in a trial. While we at the FDA do not actually conduct clinical trials,

we do work with researchers and scientists to provide guidance on clinical trial design and to oversee clinical trials to verify the quality and the integrity of the data.

Another key FDA role in regulating regenerative medicine therapies is to advance the state of the science. We do this by publishing guidance documents that educate and advise product manufacturers. We also use our regulatory authority and compliance standards to protect patients from poor-quality, unsafe, and ineffective products. Finally, stakeholder and patient engagement is a critical aspect of our work. We collaborate and communicate with patients, caregivers, and advocates and with product and technology developers to facilitate drug development. Next slide.

Regenerative medicine is a rapidly evolving field, and FDA is one of many stakeholders involved in advancing development of therapies. The number of Investigational New Drug applications, or INDs, that FDA has received for clinical studies for gene and cell treatments continues to increase. In fact, from 2016 to 2020, over those four years, the number tripled—the number of new INDs that we received for cell and gene therapies tripled. This means that there are many potential treatments in development. Gene therapy has great potential to benefit patients, particularly with rare diseases. According to the National Institutes of Health, or NIH, an estimated 80 percent of rare diseases are caused by a single gene defect. There are more than 7,000 known rare diseases, and more continue to be discovered each year. If rare diseases caused by a single gene defect could have that gene—that single gene corrected or repaired using gene therapy, that has the potential to mean significant improvements in health outcomes, quality of life, and disease management for patients and their families. I'd also note that the FDA's involved in a new public-private partnership. Now this is led by the NIH, and the partnership is called the Bespoke Gene Therapy Consortium, or BGTC. The Bespoke Gene Therapy Consortium was launched just last month. The primary goal is to accelerate and standardize the development of gene therapies for the 30 million people in the United States who are living with a rare disease. There are currently only two—only two—FDA-approved gene therapies for single gene disorders. And with this bespoke initiative, we

hope to see even more gene therapies for both rare and common diseases come to market. If you're interested in seeing a full list of regenerative medicine therapy products that FDA has approved to date, you can use the link at the bottom of the slide. Let's go to the next slide.

I want to take a few minutes to talk about the danger of unapproved regenerative medicine therapies. Regenerative medicine therapy products of any kind must go through proper clinical trials and FDA oversight, and they must be approved by FDA before they go on the market. Unfortunately, some clinics and companies are offering unapproved regenerative medicine products, often involving unapproved stem cell treatments. This is illegal—and not only is it illegal, but it puts patients seeking effective treatments at great risk. Some patients who have used unapproved regenerative medicine therapies have had adverse events, including blindness, tumors, and infections. Receiving an unapproved regenerative medicine therapy can be dangerous. Patients always have the right to ask questions, such as, "Is this treatment approved by the FDA?" to any health care provider you interact with. Next slide.

FDA has taken action to prevent the marketing of unapproved regenerative medicine therapies, including issuing warnings and pursuing legal enforcement against clinics and companies selling unapproved treatments. I encourage you, please, contact us at this email address if you are being offered or considering treatment of an unapproved regenerative medicine therapy or a regenerative medicine therapy product outside of a clinical trial, or if you've already received such a regenerative medicine therapy and you wish to report any adverse effects or file a complaint. I want to reiterate that regenerative medicine is still an emerging field that can offer hope to many patients. It's just so important that patients, advocates, and caregivers ask questions to ensure that you're making informed health decisions. Next slide.

Now let's talk about patient engagement at FDA and specifically at OTAT. OTAT is committed to engaging with patients and caregivers and wants to learn from patients about their experiences. The patient voice is critically important to FDA, whether it's about regenerative

medicine therapies or any other area of medicine. A central focus of FDA's patient engagement activities is to learn from patients. We want to know about the impact of disease and treatments on patients. This could include things like the most bothersome or serious symptoms patients experience because of their disease or condition, what the day-to-day management of the disease is, and how the disease impacts their daily lives. We also want to hear patient perspectives about current and potential treatment options. For example, what are patients' expectations of benefits from a treatment? What preferences do patients have for their treatment options? What risks are patients and their families willing to tolerate? And we want to hear what patients and their families think about when considering participation in a clinical trial. Because FDA provides oversight of clinical trials, we want to understand the challenges of participating in clinical studies so that we can try to reduce those challenges. We also want to help patients understand the potential risks involved in participating in clinical studies. This information helps us educate clinical investigators about designing studies for patients and their families. Next slide.

There are different ways that patients can share their perspectives with OTAT. First of all, patients can participate in public meetings and workshops hosted by FDA, such as the patient engagement workshop that OTAT hosted this past May. These are open to the public, and patients and representatives from patient organizations are often speakers or panelists at these types of events. OTAT is planning to host its second annual regenerative medicine patient engagement workshop this spring. So please keep an eye out for that. Patients can also join what is called a patient-focused drug development meeting. In 2012, FDA established the Patient-Focused Drug Development initiative to more systematically obtain patients' perspectives on specific diseases and currently available treatments. These meetings are intended to engage patients and give them a space to share their views with FDA staff that attend the meetings. Since 2015, FDA has supported externally led patient-focused drug development meetings, which are organized by a variety of patient groups. We also encourage patients to join an FDA-NORD—N-O-R-D—rare disease listening session. NORD stands for the National Organization for Rare Disorders, and they are a patient advocacy organization that

supports patients with all kinds of rare diseases and disorders. Since 2018, there have been 29 FDA-NORD rare disease listening sessions. In a listening session, patients with rare diseases, caregivers, and advocates can meet with FDA staff to enhance FDA's clinical and regulatory understanding of disease and provide a common understanding of the most urgent needs of patients, caregivers, and advocates. And lastly, patients can join meetings between FDA and the patient organizations. Sometimes patient groups are looking for specific input from FDA on designing surveys, on organizing natural history studies, or conducting research to help move drug development forward. Next slide.

OTAT works to enhance collaboration between FDA, patients, and drug developers or sponsors. We encourage sponsors to invite patients and advocates to the sponsors' meetings with OTAT. When patients attend these meetings, it gives us the opportunity to hear the patient voice on the issues with that specific product. We recognize that patients who attend such meetings have been selected by the sponsor and therefore their views might not necessarily represent the entire patient community. However, when patients participate in these meetings, it can help make the regulatory and drug development process more transparent for everyone. We encourage patients, patient representatives, and advocacy group representatives to approach drug developers directly and request to attend these sponsor-FDA meetings. We also encourage patient groups to take on translational science activities, such as organizing a natural history study or participating in a patient registry. A natural history study collects information about the natural history of the disease in the absence of an intervention. The data from natural history studies can be particularly helpful in developing drugs for the treatment of rare diseases. Natural history studies guide the design of clinical trials.

We also encourage patient groups to work together. Often patients with rare diseases feel isolated because the diseases are, by definition, rare. By joining forces with patients with other rare diseases, patients can reduce competition for resources and take advantage of collaborative opportunities to advance the science.

Finally, we encourage patient advocacy groups to begin collaborations early, be transparent about projects, and consider participating in scientific consortia. We recognize that the field of regenerative medicine has much to offer, and we at OTAT—and throughout the FDA—believe that patients are at the center of these scientific advancements. Next slide.

The last thing I'd like to share today are a few ways you can stay in touch with CBER. In the coming years, OTAT will be providing more educational resources and hosting more webinars like this one about regenerative medicine, including gene and cell therapies. For the latest information, you can visit our website, follow us on Twitter—we're at @FDACBER. You can also sign up for email updates from us. We've also included some helpful links and resources here for you.

Thank you all so much for your time. I'd now like to turn it back over to Anne. Dr. Rowzee?

DR. ROWZEE: Great. Thanks, Wilson. As Dr. Bryan mentioned, there were some things in the presentation we just didn't have time to cover. We could spend hours talking about these topics. So please let us know in the chat box or by using the hashtag #RegenMedEd on social media if there are specific topics related to regenerative medicine that you'd be interested in hearing more about. I saw in the chat we've already received some suggestions, so thanks for those. We will have some time now to open up for question-and-answers. Again, as I mentioned earlier, we may not be able to get to everybody's question today, and there may be some questions we just actually can't answer, such as about very specific topics. But please use the Q&A box on Zoom, and we will do our best to answer the ones we have. So I just—Wilson, just to kick off and go back to some of the approved products, can we talk a little bit about the regenerative medicine therapies that have been approved? And if someone wanted to participate in a clinical trial or was offered a product outside of a clinical trial, perhaps what questions should they ask about that product?

DR. BRYAN: So the products that have been approved are really, if we think about—particularly gene therapies—these are scientifically very advanced products. We have seven gene therapies approved at this time. Five of them are what are called chimeric antigen-receptor T-cell products, or CAR T-cell products, and these are products for treating various forms of hematologic malignancies, like leukemia, lymphoma, and multiple myeloma. These are lifesaving products that really are used for people who have failed all the available therapies. So these are wonderful products. The other two gene therapies that are approved—one is for treating a rare neuromuscular disorder, spinal muscular atrophy. It's a lifesaving product. These—it's given to infants with spinal muscular atrophy, who would typically die by age 2. And this is lifesaving. The other gene therapy is Luxturna. It's a treatment for a rare form of retinal dystrophy, and these are patients who are going blind, and it helps blind people see. So really lifechanging products.

And the regenerative medicine therapies that we're worried about, that are being used and that are not approved and are being marketed to patients in clinics, stem cell clinics, and well over 1,000 clinics across the country are using these unapproved products—it's important to ask, is a product approved by the FDA? Is there a clinical trial? And to be honest, one tip-off that the product is not approved by the FDA is your insurance company won't pay for it. And so the people that are promoting these products are asking patients to pay thousands—often tens of thousands—of dollars for products that really have not been shown to have any benefit. And patients are desperate. So it's a bad situation.

And what's going to help us get out of this bad situation is science. And good science and doing clinical trials is the way we're going to figure out which products work and which ones don't. And once we do that, the products that don't work will fall out of favor.

DR. ROWZEE: Thanks for that. I think we have another question actually about the RMAT program, the regenerative medicine advanced therapy designation. And our inquirer wants to know a little bit more about the program or the designation. What does it mean? And what

does a potential treatment need to qualify for the RMAT program? And what is the vision for this program in terms of expediting therapies?

DR. BRYAN: The regenerative medicine advanced therapy, or RMAT program, came into existence in 2016 with the passage of the 21st Century Cures Act, which, by the way, was signed into law with strong support from both houses of Congress, signed by President Obama at the end of his term. And the products that are eligible for RMAT designation are basically cell therapies, gene therapies, some tissue engineering products, xenotransplantation products. And it has been very popular. To get the designation, the sponsor, the drug developer, has to provide preliminary clinical evidence that this product may address—has the potential to address an unmet need for the treatment of a serious or life-threatening disease.

So we have received well over a hundred—probably over 200 applications for RMAT designation so far. There are over 60 products that have received the designation. And the success rate of applications is about 35, 36 percent, something in that range. And the benefits of the program are basically that you get more attention. The drug developer gets more attention from the FDA. We have many applications. Right now OTAT has over 2,700 active INDs. So there's a lot of competition for our time and attention. And if a product gets RMAT designation, then it gets extra attention. And it's important that we pay extra attention to the products that look like they're going to work. And those are the ones that have such preliminary clinical evidence. So that's the RMAT program, and it's been very popular and very successful so far. We actually this past year approved our first two products to go on the market that had RMAT designation. Those were, I think, StrataGraft®, a treatment for partial thickness burns, and Abecma, a treatment for hematologic malignancies. So very glad to see that the RMAT program has led from preliminary clinical evidence to definitive clinical evidence to get the products on the market.

DR. ROWZEE: The first two of many, I'm sure, to see coming. This question might be more of a Center for Drugs question, but as a neurologist, I'm going to kick it to you. So the question that

we have here is, what is the Agency's perspective on repurposing drugs for neurodevelopmental disorders caused by genetic mutations? You may be able to get the CBER biologics perspective in there as well, but your thoughts on that?

DR. BRYAN: When a product goes on the market, you got to start somewhere. And you start by showing that it's safe and effective for some particular disease. And then, with time, the drug developer may develop it for other diseases and get to an additional indication for the product. But sometimes products on the market that might be useful in some disease where the drug developer is not particularly interested, for whatever reason—usually business reasons—in developing the product for that particular indication. And it's not unusual for researchers, particularly academic researchers, to take a drug that's on the market and repurpose it and study it for some other indication, based on what's known about that other disease and what's known about the product. And there have been many efforts to do this over the years, and occasionally it bears fruit. It's not an easy thing to do, but it can be done. And sometimes patient advocacy groups— patient groups working with academic investigators, particularly—have gone this route of trying to repurpose drugs. It's a long shot, but sometimes it works. And for cell therapies and gene therapies, there just really aren't many products on the market right now, as opposed to the thousands of—maybe it's tens of thousands—of small molecules that are regulated by CDER that are generally available and can be considered for repurposing. But repurposing, like everything else, should be based on good science. There should be a good understanding of the disease and a good understanding of the mechanism of action of the drug to support going down that road of investigating repurposing.

DR. ROWZEE: So switching back to stem cell products and investigational stem cell products, we have a question of—could you provide some strategies or tactics that people could try to confirm that a stem cell product is part of a clinical trial? So I think you mentioned earlier one clue or tip-off could be that your health insurance company won't cover it. Are there any other things that folks can do to check on a product that they're being offered?

DR. BRYAN: Sure. Clinical trials in this country are now supposed to be regulated—supposed to be registered—I'm sorry—on ClinicalTrials.gov, which is an NIH website that lists all types of clinical trials. And also, if you're a patient or a family member looking for clinical trials for a particular disease, that's the place to go. It's got a good search function. You can type in the disease, the name of the disease, and find all the different clinical trials. It'll tell you the sites where the studies are being done, to give you an idea of whether it's a place near you, and give you an idea of what the eligibility criteria are and the stage of development. So ClinicalTrials.gov is a very informative website. It can be useful for patients to find clinical trials and, if you're being offered a regenerative medicine therapy, to look to see is there a clinical trial for this.

DR. ROWZEE: We have a question getting back to xenotransplantation products and the very interesting scenario that you described of a recent experiment. I just wanted to ask, when it comes to clinical trials and oversight—FDA oversight of xenotransplantation products, does FDA regulate the research on brain-dead patients, or is it only—where does the line get drawn there?

DR. BRYAN: We don't regulate research on animals unless you're planning to develop a genetically modified animal to use for humans. We work closely with our Center for Veterinary Medicine in looking at these genetically modified animals, because sometimes there's concern about having genetically modified animals that might get into the general animal population and whether there might be some risks there. So we work closely with the Center for Veterinary Medicine when that issue comes up. But really, the Center for Biologics gets involved when you want to start using that genetically modified animal or an organ from that genetically modified animal for treatment of patients.

Anne, we can't hear you.

DR. ROWZEE: Sorry. It was really informative stuff, I promise you—just kidding. So one question that we had is, are gene therapies cures?

DR. BRYAN: So the idea that you have a patient with a single gene defect—and these are often very rare diseases that—and these are the kind of diseases that a parent would have a child that was not developing properly for some reason or other and would take the child to the doctor, and the doctor either couldn't diagnose it—now that we have genetic sequencing, probably can diagnose it, and—but even then, if you can diagnose it, couldn't treat it—and often very bad diseases—just nothing to offer. And the idea that you could go in and either replace that gene or edit that gene to make it have the normal sequence so it doesn't cause the problem, that—if you've got a bad gene and you make it into a good gene, you put in a good gene, that—at least theoretically, that sounds like it should cure the disease. And that's what we want. We want to cure diseases. That's one of the best things about working at OTAT, is that what's happening in cell and gene therapies, the folks who are developing these products are not trying to have small impacts on diseases. They're really trying to cure diseases. And that's exciting. Now, will they cure diseases? Will they make the disease go away? Well, maybe not.

For instance, on Luxturna, the treatment for retinal dystrophy, it takes patients who are effectively blind and improves their vision. It doesn't make their vision completely normal. You take Zolgensma, a treatment for spinal muscular atrophy given to infants, infants that were going to die, and now they live. And the kids now are 5, 6, 7 years old. They can learn. They can talk. But will that benefit last forever? We don't know. We've seen some gene therapies in development where the benefit seems to wear off. And so we have to follow patients to see how long will that benefit last and will it be necessary to readminister the gene therapy, if we can find a way to readminister safely. So for each product, it's going to be different. We have to—we're going to have to follow patients closely to see how long the benefit lasts. To be honest, we—I think we should admit what we're trying to do is cure diseases, but it's unlikely that these first few gene therapies will do that. Let's just see what we can get.

DR. ROWZEE: Well said. We have a question here about—actually, switching from gene therapies and cell therapies to devices, are there regenerative medicine devices?

DR. BRYAN: So there are devices that are used for—particularly for processing cellular therapies, I will say. You could think of them as being regenerative medicine devices, because they're for processing cellular therapies and the output of the device is a cell therapy. So I think it's fair to call them regenerative medicine devices.

DR. ROWZEE: One question that we had received that I had just misplaced is sort of how has the pandemic, which is top of mind for so many people—what have you seen in terms of the pandemic affecting drug development for cell and gene therapies? And even further or more specifically, have you seen any changes to clinical trials?

DR. BRYAN: The pandemic has affected everything, and it's certainly affected drug development and drug development for cell therapies and gene therapies. The number of new IND applications that we receive each year has been increasing rapidly for gene therapies. It increased by 140 percent from 2016 to 2019—more than doubled. And then for gene therapies, the number from 2019 to 2020 basically didn't change. It was going up rapidly, and it just plateaued all of a sudden. We don't have numbers yet for 2021. But I think that that sudden plateau is because all these scientists couldn't get into their labs to do their research, to do the background work to submit gene therapies. So we hope that will pick up again as the pandemic gets under control. In contrast, for cell therapies, the rate of increase from 2016 to 2019 was good but not as dramatic as the change from 2019 to 2020. From 2019 to 2020, there was a—in that one year, there was a 66 percent increase in the number of new INDs for cell therapies. And that sudden increase for cell therapies, I think, is because there's a lot of interest in developing cells and cellular-derived therapies for the treatment of COVID-19, because we think that some of these products might have anti-inflammatory properties that will be useful. The pandemic has slowed down drug development in a variety of ways. Supply

chain problems have hurt drug developers. And also, certainly, the pandemic has changed clinical trials. There's a lot more telemedicine, so a lot fewer visits to a study center, and we've had to start to figure out how to monitor patients remotely. I think ultimately this will be a great boon for the development of products and particularly in gene therapies for rare diseases. If the—if only 5 or 10 or 20 patients around the world have a product, these patients can't all travel to one location in the United States to enroll in a trial. But they may be able to travel once to the United States to get administration of the product and then be followed remotely wherever they are in the world. So I'm thinking that what we've learned in the pandemic with regard to remote enrollment and monitoring and outcome measurement will really facilitate development of the products, particularly in gene therapy for rare diseases.

DR. ROWZEE: That's great. I think we're at time. So thank you so much for those answers, and thank you to everybody who submitted questions and topic ideas. I really appreciate—sorry for those questions that we couldn't get to today. But these are also really going to be helpful in terms of helping us see what people are interested in hearing about from us. So just to close out, I'd just like to thank everyone for joining our first RegenMedEd webinar. I hope you found it useful. I encourage you to stay tuned for our next webinar. The materials from today's webinar will be available in a few weeks on FDA's website. Again, please use the hashtag #RegenMedEd to share your thoughts on today's webinar. Let us know again what topics you'd like us to feature in a future webinar. And again, thank you all for your time and your interest, and I hope everyone has a great day. Take care now.

(Event concluded at 12:01 p.m.)