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FOOD AND DRUG ADMINISTRATION (FDA)

RARE DISEASE DAY PUBLIC MEETING

Patient Perspectives on the Impact of Rare Diseases:  
Bridging the Commonalities

DATE: Monday, April 29, 2019

TIME: 1:00 p.m.

LOCATION: FDA White Oak Campus

10903 New Hampshire Avenue

Building 31 (Room 1503-C)

Silver Spring, MD 20993

REPORTED BY: Samuel Honig, Notary Public

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## 1 P R O C E E D I N G S

2 DR. MAYNARD: Good afternoon, everyone.  
3 Thank you for being here today. Welcome to FDA's  
4 Public Meeting on Patient Perspectives on the Impact  
5 of Rare Diseases: Bridging the Commonalities. My name  
6 is Janet Maynard, and I'm the director of the Office  
7 of Orphan Products. I will provide introductory  
8 remarks for this meeting.

9 We are pleased to have this opportunity to  
10 engage directly with you. Patients and caregivers are  
11 experts on their diseases, and this meeting will allow  
12 us to learn about the impact of rare diseases on you,  
13 and to assess for commonalities in symptom management,  
14 treatment considerations and clinical trial and  
15 registry considerations.

16 FDA's mission is to promote and protect  
17 public health by helping safe and effective products  
18 reach the market in a timely manner. At today's  
19 meeting we will focus on rare diseases and the impact  
20 of these rare diseases on patients and caregivers.

21 There are over 7,000 rare diseases affecting  
22 an estimated 30 million people in the United States.

1 Rare diseases can be fatal and highly disabling with  
2 significant impacts on patients and families.

3 Notably, it is estimated that about half of rare  
4 diseases affect children. We recognize that there are  
5 unmet needs for patients with rare diseases, as most  
6 rare diseases do not have approved therapies. The  
7 availability and access of safe and effective medical  
8 products for patients with rare diseases is critically  
9 important. With scientific advances, there are new  
10 opportunities for the development of therapies for  
11 rare diseases.

12 We at FDA are dedicated to supporting public  
13 health and rare disease product development. The  
14 Office of Orphan Products Development specifically  
15 advances the evaluation and development of products,  
16 including drugs, biologics, devices and medical foods  
17 that demonstrate promise for the diagnosis and  
18 treatment of rare diseases or conditions.

19 In fulfilling that task, the Office of Orphan  
20 Products Development evaluates scientific and clinical  
21 data submissions from sponsors to identify and  
22 designate products as promising for rare diseases and

1 to further advance scientific development of such  
2 promising medical products.

3 In addition, the Office of Orphan Products  
4 Development provides incentives for sponsors to  
5 develop products for rare diseases. Our programs have  
6 successfully enabled the development and marketing of  
7 drugs, biologics and medical devices for rare diseases  
8 since 1983. To optimally support rare disease product  
9 development, FDA collaborates both internally and  
10 externally with of stakeholders.

11 Today's meeting is one example of FDA's  
12 dedication to rare disease product development and  
13 includes representatives from the medical product  
14 centers, CDER, CBER and CDRH, the Office of Pediatric  
15 Therapeutics, the Office of Orphan Products  
16 Development, and the Patient Affairs staff.

17 I would like to acknowledge and thank the  
18 cross-agency collaboration that supported the planning  
19 and organization of today's meeting. Each of us here  
20 today has a unique perspective and are committed to  
21 working together to achieve success. Developing a  
22 treatment for a rare disease can present unique

1 challenges, such as the small number of individuals  
2 affected and heterogeneous etiologies and  
3 manifestations. While the differences between rare  
4 diseases are critically important, it is also  
5 important to assess commonalities to synergize product  
6 development in rare diseases.

7 We recognize that each patient's experience  
8 is unique. By sharing our experiences together, we  
9 will learn from each other. Further, we may find  
10 commonalities that are not as rare as one would think.  
11 The involvement of patients and caregivers and their  
12 input is critical in addressing the challenges of  
13 developing a treatment for rare diseases. FDA is  
14 committed to working with patients and caregivers to  
15 ensure products address patient needs.

16 The goal of this meeting is to obtain patient  
17 and caregivers' perspectives on the impacts of rare  
18 diseases on their daily life, and to identify common  
19 issues and symptoms in rare diseases to help advance  
20 medical product development. This may potentially  
21 support medical product development in consideration  
22 of novel endpoints or trial design that focuses on

1 commonalities across a variety of rare diseases.

2 We are pleased to see so many patients,  
3 caregivers and advocates in the audience. We also  
4 have many more of you joining remotely from the web.  
5 Thank you for being part of this meeting and sharing  
6 your experiences.

7 Today's meeting builds on FDA's efforts to  
8 hear directly from patients and caregivers. Today we  
9 will hear from those affected with a variety of rare  
10 diseases and conditions rather than focusing on a  
11 specific disease. We appreciate this unique  
12 opportunity to hear directly from patients and  
13 caregivers, and to assess for commonalities across  
14 rare diseases.

15 After this meeting we will take what we learn  
16 and write a summary document. It will take us some  
17 time to produce this document. The document will  
18 consider the transcript and our notes from this  
19 meeting, and the docket comments. We leave open the  
20 docket after the meeting so we can get submissions of  
21 information from people on the webcast, or other  
22 things that may occur to people in the room or on the



1 web after they hear this meeting. We will put  
2 together this information from the meeting and the  
3 docket to develop a summary document. We hope that  
4 the document will be extremely valuable to the various  
5 stakeholders in product development. We hope to  
6 capture as accurately as we can what we heard today  
7 and what we read in the docket. This will serve as a  
8 resource to the staff at FDA and sponsors who are  
9 developing products for rare diseases.

10 We hope this will be valuable to sponsors as  
11 they consider aspects of their development program,  
12 such as the outcome measures and the design of  
13 clinical studies and registries. We will be producing  
14 this document in the coming months after the meeting.

15 Today's meeting focuses on patients with rare  
16 diseases. Similarly, we have portraits on display  
17 that focus on rare disease patients from the "Beyond  
18 the Diagnosis" art exhibit. These portraits help us  
19 see and experience people who are living with these  
20 rare diseases and conditions. The portraits help us  
21 see beyond the diagnosis and instead see people who  
22 live with this day in and day out.

1           The goal of the "Beyond the Diagnosis"  
2 exhibit is to put a face to all known rare diseases  
3 through the medium of art. Artists from around the  
4 world have contributed their talents to this beautiful  
5 exhibit that is traveling to medical schools, research  
6 institutes and hospitals around the globe, encouraging  
7 the medical community to look beyond the diagnosis to  
8 the patient.

9           We are excited to have this opportunity to  
10 have so many portraits on display outside the meeting  
11 room today, and we encourage those attending in person  
12 to take time to look at the exhibit.

13           Thank you for your participation in today's  
14 meeting to support product development for rare  
15 diseases. We are encouraged to work together and  
16 energized to work together so that we can have  
17 stakeholder engagements to bridge the gaps for rare  
18 diseases. I will now turn it over to Andrea from the  
19 Patient Affairs staff. Thank you.

20           MS. FURIA-HELMS: Good afternoon and welcome  
21 to the public meeting, Patient Perspectives on the  
22 Impact of Rare Diseases: Bridging the Commonalities.

1 My name is Andrea Furia-Helms. I am the director of  
2 the Patient Affairs staff at FDA. Welcome to patients  
3 and family members who are here in the Great Room for  
4 this very important meeting. We know it can be a bit  
5 of an effort to get to the FDA campus, so we thank you  
6 for making it here. Thank you to those joining by  
7 webcast as well. We understand not everyone can be  
8 here in person, so we appreciate you taking the time  
9 to participate and contribute online.

10 We also have many participants in the room  
11 and online, such as patient advocacy organizations,  
12 healthcare professionals, academia, industry, and  
13 others in government, including many from FDA. We are  
14 glad you are here, and we hope that the input we hear  
15 from our patients and caregivers today will be  
16 valuable to you as well.

17 So, before we begin the meeting, I just want  
18 to make a few administrative announcements. Please  
19 silence any cellphones or other mobile devices, as  
20 they may interfere with the audio in the room today.  
21 If you haven't already, we ask that attendees sign in  
22 at the registration table outside the meeting room.

1 Restrooms are located in the lobby past the coffee  
2 area to the right and down the hallway. For media  
3 inquiries, our press officer, Sandy Walsh, is here  
4 today. Sandy? If any members of the media are here  
5 today, please sign in, and if you have any questions  
6 or are interested in speaking with FDA about this  
7 meeting, please contact Sandy Walsh. The meeting is  
8 intended to give the FDA an opportunity to hear from  
9 patients and caregivers, so the FDA panelists and  
10 other FDA employees will not be available to make  
11 statements to the media.

12 For the Wi-Fi in the Great Room, the network  
13 and password is displayed on the screen. A public  
14 docket, as Janet mentioned, is open until May 30th to  
15 submit comments. We highly encourage you to do so.  
16 The webcast recording of this meeting will be  
17 available approximately one week after the meeting.  
18 Copies of the transcript will be available  
19 approximately 30 days after this meeting. For urgent  
20 issues, please speak to the registration desk staff or  
21 any FDA staff you see in the room wearing a nametag.  
22 In case of an emergency, please exit the Great Room or

1 overflow room you are in and follow the exit signs to  
2 leave the building.

3 Please let us know how the meeting has gone  
4 today. Evaluation forms were placed on your seats.  
5 If you do not have one, please stop by the  
6 registration table. Now, I'd like to take a moment to  
7 walk through the agenda for the afternoon.

8 As Janet mentioned, the goal of this meeting  
9 is to facilitate an open dialogue on personal  
10 experiences that will identify common issues and  
11 symptoms in rare diseases to help advance medical  
12 product development. To accomplish this goal, we will  
13 have two facilitated discussions led by Susan  
14 Chittooran, who is also with Patient Affairs. Susan?  
15 Just a disclaimer, our color coordination was by  
16 mishap.

17 The first session will focus on symptom  
18 management and treatment considerations, and the  
19 second session we'll explore clinical studies and  
20 registries. During each session we will be hearing  
21 from patients and caregivers on our panels, as well as  
22 from patients and caregivers joining in the room and

1 on the webcast.

2 A little bit about how we selected the panel  
3 members. Panel participants were selected from those  
4 participants who expressed an interest at the time of  
5 registration and submitted summaries that addressed  
6 the discussion questions on the meeting webpage. We  
7 identified individuals with a range of experiences  
8 related to the discussion topics for today.

9 For those in the room, please raise your hand  
10 if you would like to speak. We will bring a hand-held  
11 microphone to you. You may remain anonymous or state  
12 your first name, and we encourage you to state the  
13 disease area you are representing. For transparency  
14 purposes, when you're sharing your comment, we  
15 acknowledges that you please disclose if you are  
16 affiliated with an organization or if your travel has  
17 been funded, or if you have a significant financial  
18 interest in rare disease medical product development.

19 For those of you in the overflow room, you  
20 can write your comment on a notecard on your seat and  
21 pass it to the end of the aisle, where someone will  
22 collect it. For those on the webcast, please type

1 your comments into the chat feature in Adobe Connect.  
2 We will periodically be checking in to see what our  
3 remote attendees are sharing in the chat box. After  
4 session one concludes, we are delighted that the  
5 Principal Deputy Commissioner and Acting Chief  
6 Information Officer, Dr. Amy Abernathy, will provide  
7 remarks with us. We will have a break before we begin  
8 session 2, and conclude the afternoon with the open  
9 public comment period. So, some information about the  
10 open public comment period. Say that 10 times fast.

11 We have time set aside for the open public  
12 comment later this afternoon, and the session will  
13 give anyone in the audience the opportunity to make  
14 any comment. To participate in that, you would have  
15 needed to sign up at the time you registered or sign  
16 up today at the registration table. Participation is  
17 first-come, first-served, and has accommodated up to  
18 10 commenters. We are excited that we have so many  
19 that were interested in the open public comment period  
20 that the slots have already been filled up. The time  
21 allowed for each speaker will be about two minutes  
22 each. After the open public comment period, Janet

1 Maynard will provide some closing remarks.

2           We want you to stay connected. Please know  
3 that once the meeting ends today, that doesn't mean  
4 that this is the last or only opportunity you can  
5 speak with FDA. The Patient Affairs staff and the  
6 Office of Orphan Products Development are here, and we  
7 want to stay connected with you. Whether it's helping  
8 you to stay connected with other activities at FDA or  
9 addressing any further questions you may have, please  
10 do keep in contact.

11           Here is our contact information and Twitter  
12 handle. Speaking of Twitter, if you choose to tweet  
13 about today's meeting, please use #rarediseasefda.

14           Now, just for some rules of engagement for  
15 today's discussion. Patients, caregivers and their  
16 advocates are encouraged to sit near the front of the  
17 room. FDA is here to listen to you and we -- it's not  
18 about us; it really is about you today, so we  
19 encourage you to contribute to the dialogue. Your  
20 stories and experiences are what can move medical  
21 progress forward. Because we really want to hear  
22 about your experiences, we ask that if you have any



1 other FDA questions, please email Patient Affairs at  
2 patientaffairs@fda.gov.

3 The views expressed today are personal  
4 opinions. Please be respectful of others. Have the  
5 courtesy to allow participants to finish sharing their  
6 experiences without interrupting. FDA panel members  
7 will also have the opportunity to ask follow-up  
8 questions of the participants, and participants in the  
9 room use a microphone so that the webcast attendees  
10 can hear their remarks.

11 So, before we begin, I would ask my FDA  
12 colleagues on the panel to introduce themselves. We  
13 can start with Lucas.

14 MR. KEMP: Hi, I'm Lucas Kemp. I'm the  
15 acting associate director for the rare diseases  
16 program within the Office of New Drugs within the  
17 Center of Drug Research, CDER.

18 MS. SPENCER: Hi, I'm Caroline Spencer. I'm  
19 a patient with Friedreich's ataxia. I am from  
20 Cincinnati, Ohio. And for those of you who may not  
21 familiar, Friedreich's ataxia, or FA, is a progressive  
22 neurological disease. It's a degenerative

1 neuromuscular disease that leads to progressive loss  
2 of ambulation or walking. It can also cause cardiac  
3 problems, scoliosis, diabetes and other comorbid  
4 difficulties.

5 MR. ROTBERG: Hi, everyone. Seth Rotberg  
6 here, a patient as well as advocate for the  
7 Huntington's disease community, rare disease community  
8 as a whole. I am a patient as well as I was a  
9 caregiver for my mom. Huntington's disease, also  
10 known as HD, is a rare neurological, genetic disease  
11 that slowly deteriorates a person's physical and  
12 cognitive abilities. It's like having ALS,  
13 Alzheimer's and Parkinson's all into one. I tested  
14 positive at the age of 20; I'm 28 now. And I also sit  
15 on the board for the Huntington's Disease Youth  
16 Organization supporting young people impacted by  
17 Huntington's disease worldwide, and really just  
18 excited to be here.

19 MS. SHAPIRO: I'm Adrienne Shapiro. I am  
20 fifth generation of mothers in my family to have a  
21 child born with sickle cell disease. Sickle cell  
22 disease is just that -- it's a disease where our red

1 blood cells, rather than being round, disk-shaped,  
2 there's a large portion that become sickle-shaped,  
3 which cause problems as you try to circulate through  
4 the bone marrow, ending up in very painful episodes  
5 and damage to major organs of the body. There is a  
6 life expectancy here in the US of 45 years.  
7 Throughout the world, children live less than five  
8 years. And I have a nonprofit called Axis Advocacy,  
9 and we advocate for people living with sickle cell  
10 disease, particularly the adults.

11 MS. WITTEN: Hi. My name is Rachel Witten.  
12 I'm from CBER. I am from Office of Tissue and  
13 Advanced Therapies. One of our activities of work, we  
14 do regulate clinical gene therapy clinical trials for  
15 rare diseases.

16 DR. MAYNARD: Hi. I'm Janet Maynard. I'm  
17 the director of the Office of Orphan Products  
18 Development and the Office of Commissioner.

19 DR. SILVERSTEIN: I'm Doug Silverstein. I'm  
20 a pediatric nephrologist. Most of the diseases that  
21 we treat in our subspecialty are rare diseases, and I  
22 work in the Center for Devices and Radiological Health

1 in the Renal Devices branch.

2 Dr. MCCUNE: Good afternoon. I'm Susan  
3 McCune. I'm the director in the Office of Pediatric  
4 Therapeutics in the Office of the Commissioner. My  
5 background is I'm a pediatrician, and my subspecialty  
6 is neonatology, or newborn intensive care, and I'm  
7 very interested in hearing what all of you have to say  
8 about the impact of rare disease on your lives,  
9 especially with respect to pediatrics.

10 MS. CHITTOORAN: Hi. I'm Susan Chittooran.  
11 I am a social worker by background and I work with  
12 Andrea on the Patient Affairs staff. I'll be  
13 facilitating the meeting this afternoon.

14 MS. FURIA-HELMS: Thank you so much. Just a  
15 few last things. The live webcast is being recorded,  
16 which will be archived on our website, along with the  
17 transcript. You may also notice a film crew around  
18 during the meeting. We are capturing the video  
19 footage of this meeting.

20 And in closing, I want to thank everyone,  
21 including our panelists, for participating today, and  
22 I look forward to a very productive meeting. I'm now

1 going to turn it over to Susan Chittooran. Thank you.

2 MS. CHITTOORAN: Hi, everyone. As I  
3 mentioned earlier, my name is Susan Chittooran. I  
4 work with Andrea and the Patient Affairs staff. I am  
5 very excited to be here today with such a full room,  
6 where the patients and caregivers here on the panel,  
7 as well as here in the room in the front rows. We're  
8 very interested to hear what you have to say, and  
9 we're interested in listening and learning from you  
10 and your experiences.

11 So, as Andrea mentioned, we are going to be  
12 spending this first session here talking about your  
13 symptoms, the way you're managing your symptoms, and  
14 then any considerations in treatment. So, we  
15 recognize here that not everybody in the room may have  
16 the same disease; they may have different diseases,  
17 but we recognize your experiences may be different,  
18 even if you maybe treat diseases or conditions in  
19 different ways. So, we're very happy to learn from  
20 that.

21 So, we have designed this session as  
22 interactive, and what I mean by that is I will be

1 posing questions first to our panel here, and then we  
2 will be asking some questions back-and-forth. My FDA  
3 colleagues here may have follow-up questions, and then  
4 we will turn to you all in the room. We have patients  
5 here, and we also have patients on the web as well.  
6 My colleague here, Wendy -- Wendy, if you'd raise your  
7 hand -- will be kind of filtering some of the stuff  
8 that we're hearing online. So, she'll help us kind of  
9 capture the patient and caregiver voice from online.  
10 And then after that we'll go back to the panel. So,  
11 we'll sort of take turns in terms of how we are doing  
12 the discussion.

13 So, in order to best accommodate the most  
14 number of speakers as we can, we just ask everybody to  
15 please be mindful of their time in responding. Also,  
16 if there are things you think about that you didn't  
17 get to say here today, we do have a docket, as we  
18 mentioned a couple of times, that's open until May  
19 30th. So, we encourage you to utilize that if you're  
20 in the room and on the web.

21 So, yeah, and then finally, just please, as  
22 Andrea mentioned, please just be respectful of others

1 when speaking. Just recognize that people are talking  
2 about personal experiences that may not be easy to  
3 share. So, with that, I'll get started. We've  
4 already done some introductions of our panel, so I  
5 will start off by asking questions to our panel. And,  
6 again, we may have some follow-up.

7 So, Adrienne, I will start with you. So, you  
8 mentioned that you are a caregiver to your daughter  
9 with sickle cell disease. So, in terms of your own  
10 experience as a caregiver, what would you say the two  
11 to three most burdensome symptoms that your daughter  
12 has sort of experienced, from your perspective?

13 MS. SHAPIRO: The disease, the nature of the  
14 disease basically prevents the flow of blood  
15 throughout the body. So, that, sort of, if you think  
16 of the terms if you were sort of gasping for air,  
17 right, or drowning, or that pain that you get, right?  
18 So, she experiences great, great pain within the bone  
19 marrow and as a result has been on opioids since she  
20 was two years old. So, I would say the pain. I think  
21 also being anemic and having the lack of oxygen, the  
22 fatigue. Fatigue is something that most researchers

1 initially, because the pain was so intense and acute,  
2 it was a long time before the researchers realized  
3 that fatigue, of course, was a major, major burden, to  
4 the point where things like a remote feels very, very  
5 heavy.

6 And I think the -- for our population, the  
7 mental health problems that come. First of all, your  
8 foggy brain if you don't have enough energy, enough  
9 oxygen. There's silent infarcts, so that children who  
10 grow up to be adults have some cognitive problems  
11 which only until recently were documented. So, I  
12 would say those were the three major symptoms that we  
13 deal with.

14 MS. CHITTOORAN: Okay. And would you say  
15 that you've seen some of those symptoms change a  
16 little bit over time? I know you mentioned pain from  
17 being very young, but are there other ones?

18 MS. SHAPIRO: Well, the pain, when they're  
19 young, and we do have treatment protocols for when  
20 you're young, so the experience with the disease is  
21 very different as a young person. When they're young  
22 they can go to the hospital, we get immediate



1 treatment, the pain is relieved; but as they get  
2 older, due to the effects, right? So, as these blood  
3 cells block in the capillary it forms scar tissue.  
4 They have, by the time they're 18 years old, vascular  
5 disease, they have lung disease, they have heart  
6 disease, they have kidney disease. On the outside at  
7 18, they might look 13, but have the body of, you  
8 know, a 60-year-old. So, I guess it's the transition  
9 (a) from being a child to being an adult who is not  
10 really an adult, having to deal with advanced disease.

11 MS. CHITTOORAN: Thank you. And you  
12 mentioned a little bit about this in your response so  
13 far, but what types of things are you and she doing to  
14 help manage some of the symptoms that she's  
15 experiencing?

16 MS. SHAPIRO: Oh, well, there are all stages,  
17 right, as there are stages of the pain, so you do  
18 meditation, you do heat, you do massage. And then  
19 there's this step up on the pain scale that you go  
20 until you're actually -- when there is nothing that  
21 you can do other than opioids, and that just sort of  
22 masks it. We have a real problem, because as you do

1 that, of course, year after year after year, her  
2 tolerance for the opioid has become higher and  
3 therefore she needs larger dosages of it.

4 We have -- this may be going ahead of things  
5 -- have at this point our first medicine. The disease  
6 is 100 years old and we've got our first medicine, and  
7 it's made a lot of difference. So, we have hope that  
8 in the near future, you know, there will be something  
9 we can do to prevent this. But as far as all the  
10 damage that's done up to this point, there's nothing  
11 that can be done for that.

12 MS. CHITTOORAN: Would anybody on the FDA  
13 panel have any questions or anything to clarify?

14 DR. MCCUNE: Thank you so much for your  
15 description. Having taken care of numerous patients  
16 with sickle cell anemia, it's very hard to see how  
17 much pain they go through. Can you comment a little  
18 bit about how this has affected your daughter's school  
19 and friends, and kind of just the activities that you  
20 would expect for a normal child?

21 MS. SHAPIRO: What I want to say is this  
22 about all of us, I think, sitting here. A diagnosis

1 of a rare disease is really a lifestyle, and becomes a  
2 lifestyle start and stop. If you look at any of our  
3 calendars, if you could, I'm sure you'll see where  
4 even we'll plan out something and you never know what  
5 you're going to be able to do from moment-to-moment  
6 what known -- I'm going on vacation in three weeks.

7 So, with the school, it was very challenging.  
8 She would be okay and then she would say I'm not okay,  
9 and they'd be looking at her, because she looked  
10 perfectly healthy. And the next thing I'd know, I'd  
11 be running to the school and then we would be taking  
12 her to the hospital, and then there would be a  
13 hospitalization. I think that that really, really, if  
14 we had a category for that, that inability to have  
15 that prediction or that safety or that continuity in  
16 life is a major, major problem throughout all of our  
17 community.

18 So, I think it is the weight of the disease  
19 causes mental stress that is prominent and something  
20 that until now has not really been addressed.

21 DR. MCCUNE: And I just wanted to rescind the  
22 use of the word normal, because none of us are normal.

1 What I would like to say is how you differentiate from  
2 a child who does not have a chronic disease or your  
3 particular daughter's chronic disease, so just for the  
4 record.

5 MS. SHAPIRO: Yeah, yeah. And by that I  
6 think that's exactly what it meant. By normal,  
7 meaning you could have a fairly predictable schedule.

8 MS. CHITTOORAN: Yeah?

9 DR. SILVERSTEIN: Yeah. I echo Susan's  
10 comments about the appreciation about how difficult it  
11 is to get in front of a group like this. Without  
12 identification of where your daughter gets her care,  
13 or anything like that, do you feel that the healthcare  
14 team where you are, or maybe you've been to more than  
15 one place. Has a full knowledge of the various  
16 aspects of the disease that affects your daughter,  
17 more than just the medical effects of it, but also all  
18 the psychosocial and cognitive and mental health  
19 aspects of the disease -- do you feel that they are  
20 adequately trained, and what can be done to enhance  
21 that in general, not necessarily at the center where  
22 your daughter's getting her care?

1 MS. SHAPIRO: My daughter gets care at one of  
2 the most excellent facilities in -- where we live, and  
3 my answer to you is absolutely not. I do not feel  
4 that they're prepared. I do not feel that there are  
5 any protocols or protections in place to ensure that  
6 she gets quality care. And it's not only her; I do  
7 what we call at point of advocacy for other adults in  
8 our area. That means when someone has no one and they  
9 have to go to the ER, or are having problems, I go  
10 out, and we have a team of people who go out, and  
11 anyone can change our care. So, if a hematologist  
12 prescribes a pain regimen, or the pain doctor or  
13 whatever the treatment is, anyone can say I'm not  
14 comfortable with that and change it. So, I don't  
15 think any of us living with sickle cell disease are  
16 safe anywhere.

17 MS. WITTEN: I have a question. Have you  
18 ever considered to participate in any clinical trials,  
19 natural history or clinical trials for drugs or gene  
20 therapies? If it's not, why?

21 MS. SHAPIRO: Yes, actually, we're very  
22 active. I actually got into advocacy when gene

1 therapy became a possibility. We -- bone marrow  
2 transplants were the only curative for us, but getting  
3 a match for us is very rare, so gene therapy was it  
4 for us. So, yes, we are very much involved in  
5 clinical trials and gene therapy, and it is truly our  
6 community's belief that gene therapy will lead to a  
7 cure for us. Of course, until we get to the point  
8 where we can do reconstructive therapies, the damage  
9 to our bodies at whatever point won't be cured -- I  
10 mean, won't be corrected. But we are very, very  
11 interested and very involved in educating people and  
12 getting them into research. But that's not going to  
13 help us, yeah, at this point.

14 MS. WITTEN: Thank you.

15 MS. CHITTOORAN: Thank you, Adrienne. Okay,  
16 Seth, just wanted to move on to you next. So, you  
17 recently learned that you had a genetic marker for  
18 Huntington's disease after caring for your mom, who  
19 also had the disease. And from what I understand you  
20 are asymptomatic right now; is that correct? Okay.  
21 So, given that, when you're thinking about the two or  
22 three symptoms that you're most concerned about in the

1 future, what would you say that those are?

2 MR. ROTBERG: So, with Huntington's disease,  
3 it's a cognitive, psychiatric and movement disorder,  
4 and some of those main symptoms include involuntary  
5 movements, which is known as chorea. So, with my mom  
6 she had poor balance and wobbly movements, where we  
7 had neighbors who thought that she might have been  
8 drinking or had just like these drunk movements. The  
9 other big symptom was mood swings. So, that behavior  
10 of having a perfect conversation and then, I don't  
11 know if she was angry or upset or just simply  
12 depressed. And then I think the last one would be  
13 like that cognitive decline. So, being able to make  
14 decisions on her own, short-term memory loss, among  
15 other things.

16 And for me personally, what's challenging is  
17 that kind of touching upon potential treatments and  
18 how to manage it. My mom did try to take an FDA-  
19 approved drug for Huntington's disease that helped  
20 with the movement aspect of it. The challenge is that  
21 the side effects of that impacted her -- increased, I  
22 guess, her depression and suicidal thoughts.

1           So, what I've noticed with Huntington's  
2 disease as well as other central nervous system  
3 conditions, like ALS, MS, Parkinson's, is that there's  
4 a big focus on that, you know, the movement disorder  
5 biomarkers and not looking at the cognitive or  
6 psychiatric aspect. And so for me, like, yes, I'm not  
7 symptomatic yet, but unless there's a cure in 10 or 15  
8 years, I will end up just like my mom one day, and she  
9 battled it for 17 years before passing away.

10           So, for me, it's thinking about, well, can  
11 researchers now try to figure out biomarkers for these  
12 cognitive and psychiatric symptoms that don't just  
13 impact Huntington's disease, but a lot of these rare  
14 diseases, and that mental health ability of trying to  
15 improve that overall quality of life.

16           For me, it was touched on before, was the  
17 point about, you know, living a normal life, and I  
18 think for young people like myself, it's how do you  
19 live that new normal life? How do you not just  
20 transition in the child care, or from child care to  
21 adult care, but transitioning into that future  
22 planning? So, a lot of these things I have to think



1 about now is how do I build a romantic relationship,  
2 family planning, career choices, and these are always  
3 things that are on my mind and a lot of other young  
4 people's minds of how to live that new lifestyle, but  
5 also how do you plan your life for the future?

6 MS. CHITTOORAN: Thank you. And so what  
7 would you say, and you alluded to this a little bit  
8 already in your response so far, but just sort of  
9 seeing her experiences, what kind of -- what were you  
10 sort of thinking about when you decided to get tested?  
11 Is it something that you sort of knew that you were  
12 going to do sort of sooner rather than later, or how  
13 did you -- how did that impact your decision on  
14 testing?

15 MR. ROTBERG: My mom was misdiagnosed with  
16 major depression and bipolar, which, again, the mood  
17 swings and depression were just symptoms of  
18 Huntington's disease, so probably misdiagnosed for  
19 about seven years. She was officially diagnosed when  
20 I was 15, and so five years later I tested, but I was  
21 definitely in denial because I felt like I lost out on  
22 that normal childhood. And no one truly understood

1 what I was going through. I felt the isolation of not  
2 really having anyone, especially my age, to be, like,  
3 oh, yeah, I get what you're going through. And I kept  
4 thinking about it, again, my future of how to plan for  
5 what those next steps are, and that's kind of what  
6 brought me to the decision of testing. But it was  
7 definitely a very personal decision. Genetic testing  
8 is very, you know, a very big decision in anyone's  
9 life.

10 I mean, the other challenge was that it took  
11 me about, actually, three years to tell my dad and my  
12 sister to try and protect my mom, not wanting her to  
13 feel guilty, not wanting to be another burden to the  
14 family. And so for me it was eventually opening up  
15 more about that and realizing is my story and this is  
16 how I'm going to make a difference. But it's always  
17 about, you know, well, when am I going to start  
18 showing symptoms, and even there's days where, if I  
19 forget something or I can't multitask, which I know is  
20 huge challenges for anyone, I'm always thinking right  
21 away, is that Huntington's disease or is that just me  
22 being just a 28-year-old kid?

1 MS. CHITTOORAN: Okay, thank you. Is there  
2 anything that you are doing now to delay the onset of  
3 your symptoms?

4 MR. ROTBERG: There is not anything in  
5 particular that's like shown, or I guess proven to  
6 help. A lot of people say exercise, and I actually --  
7 even though I don't -- or I can't participate in any  
8 clinical trials because of the criteria and it being  
9 so tight, tight-niched, I am able to participate in  
10 some observational trials. So, I did participate in  
11 an exercise study, waiting to see the results. And I  
12 try to exercise three to four times a week. But  
13 besides that, it's tough, because you see in a lot of  
14 these online communities about different remedies,  
15 like off-label drug use or trying all these different  
16 supplements, and for me it's like that could help, but  
17 I'm trying to focus on today and not worry too much  
18 about what's going to happen in the future.

19 MS. CHITTOORAN: Okay, thank you. Is there  
20 anybody from the FDA panel that has any follow-up  
21 questions? Sure.

22 MR. KEMP: Hi. You mentioned the online

1 community. Sort of wondering how much of a role does  
2 that actually play in your sort of day-to-day life or  
3 decision-making, or just general support?

4 MR. ROTBERG: Personally speaking, it's  
5 definitely challenging because a lot of times I tend  
6 to see a lot of misinformation or information that's  
7 not, I guess, medically backed by people who just  
8 happen to Google something and they just post it in  
9 these groups. But it is challenging to see some of  
10 this stuff. Because for me I'm always trying to -- I  
11 really want to help others and help them realize  
12 they're not alone. And when I see people struggling,  
13 it hurts me, because I wish I could do more, just like  
14 I wanted to do more for my mom but I couldn't.

15 So, I think with the online platforms it's  
16 really trying to figure out the best way to understand  
17 what those needs are, especially with -- depending on  
18 if it's a young adult versus an older adult versus a  
19 child, and really just getting their perspective,  
20 especially when it comes to, I guess, clinical trials  
21 and potential treatments and cures for any rare  
22 disease.

1 MR. KEMP: Thank you. I helped set up  
2 schizophrenia.com when I was probably around your age,  
3 and many of the same things we had in the common  
4 disease issues are exactly what you just said -- a lot  
5 of misinformation, needed medical curation to make  
6 sure that the information was accurate, people weren't  
7 preying on the patients, like trying to sell some  
8 snake oil cures to the population. But it was also  
9 the large benefits that people who were in these  
10 isolated communities. Because we did surveys to see  
11 who was actually using it and what they're using it  
12 for.

13 MR. ROTBERG: Yeah, I mean, I definitely  
14 think it's valuable, online platforms, especially with  
15 some of these rare diseases where your closest  
16 connection could be someone from across the pond. And  
17 so being able to connect with people, whether it's the  
18 same condition or a different condition and seeing a  
19 lot of familiar faces here, it's great to see, like,  
20 okay, I've connected with them through some type of  
21 online platform or through some social media channel.  
22 And just knowing that, you know, I really think being

1 able to connect with one another helps improve that  
2 overall quality of life.

3 MS. CHITTOORAN: Thank you. So, Caroline,  
4 just moving on to you. So, what would you say the top  
5 two or three symptoms are that you find most  
6 burdensome?

7 MS. SPENCER: So, for me, mobility issues are  
8 definitely number one. It leads to progressive loss  
9 of balance, walking. So, right now I use a service  
10 dog, my dog Clark, to help me get around. And then  
11 second-most impactful symptom is fatigue, for me, and  
12 fatigue really ends up being a moderator for a lot of  
13 the other symptoms. You know, when I'm fatigued my  
14 speech is less precise, my voice is quieter. I'm  
15 tired, I can't balance as well, I can't walk as far.  
16 In addition, too, I just maybe need to take a nap  
17 before I respond to an email or something. It could  
18 mean that I don't make it through a whole school day.

19 And to touch on what Adrienne mentioned, it  
20 really -- it takes a lot of planning to deal with both  
21 the mobility issues and fatigue issues, to be able to  
22 anticipate challenges and figure out how to get around

1 that, or to still do what I need to do in spite of  
2 that.

3 MS. CHITTOORAN: Okay, thank you. How would  
4 you say your symptoms might have changed over time?

5 MS. SPENCER: So, up until about three and a  
6 half years ago, I walked independently. I did use a  
7 walker for a few months. So, there are gradual  
8 changes in my mobility, but once they become more  
9 apparent, they're pretty significant. A really  
10 tangible example, so I'm working on my doctorate at  
11 the University of Cincinnati right now, and 10 years  
12 ago I was an undergrad student. At that point I  
13 didn't have a diagnosis. I did have slight noticeable  
14 balance problems, but getting around the campus then  
15 compared to now is so different. So different.

16 MS. CHITTOORAN: Okay. So, you mentioned, in  
17 terms of managing your symptoms, you mentioned Clark,  
18 you were mentioning taking naps and just sort of the  
19 planning that you have to go through. Just wondering  
20 if there are other things beyond what you've already  
21 mentioned that you're doing to help manage some of  
22 those symptoms?

1 MS. SPENCER: Exercise, staying active. I do  
2 focused intensive balancing gait training twice a  
3 week, and I've done that for years. So, really  
4 keeping up on that. Keeping up with walking, even if  
5 I can only walk a quarter of a mile, that is better  
6 than nothing. And I really focus on not doing too  
7 much so that I can't do it again the next day, and  
8 that helps me balance the -- trying to do too much but  
9 sometimes I want to do nothing at all.

10 MS. CHITTOORAN: Thank you. So, I want to  
11 turn to the audience. I just wanted to see if there's  
12 any quick questions the FDA panel has for Caroline?  
13 No, okay.

14 MS. SHAPIRO: I just wanted to clarify that  
15 when you asked about treatment, pediatric treatment  
16 for children with sickle cell tends to be really,  
17 really good. So, my comment about there being none,  
18 no place for sickle patients to be completely safe or  
19 looked after was with adults. I just wanted to  
20 clarify that.

21 MS. CHITTOORAN: Thank you.

22 MR. KEMP: I just had a quick question for



1 Caroline. As your disease progressed and you need  
2 more and more help, very similarly, how did your  
3 physicians and medical team help you with that? Or do  
4 you think they were identifying the issues in sort of  
5 a timely manner and got you the things that you  
6 needed, or was it sort of one-off learning experience?

7 MS. SPENCER: So, I think my doctor  
8 definitely sensed a change in balance and walking and  
9 probably would have recommended using a mobility aid  
10 sooner than I accepted that. So, part of it, my  
11 diagnosis came, like, as a culmination of multiple  
12 symptoms. My diagnosis happened because things got so  
13 bad, whereas, maybe with Huntington's, like you kind  
14 of know and you can forecast out and see ahead a  
15 little bit. Mine, it seemed more like retrospective.

16 MS. CHITTOORAN: Thank you.

17 DR. MAYNARD: This is Janet Maynard. Maybe  
18 just one housekeeping thing as we transition to the  
19 audience. If folks can just remember to identify  
20 themselves before speaking just for the  
21 transcriptionist, just so we know who is speaking.  
22 So, if people don't mind on the panel and the audience

1 as they transition, just to say your name before  
2 speaking. Okay, thank you.

3 MS. CHITTOORAN: So, now I just want to turn  
4 over to the audience, and let me pose the same  
5 questions, some of the same questions I've asked the  
6 panel here already. And then I'm going to go to  
7 Wendy, who can also capture what was being said on the  
8 web as well.

9 So, so far in terms of the two to three  
10 symptoms that our panel has mentioned, I've heard  
11 pain, I've heard fatigue, I've heard difficulty with  
12 movement. Just out of -- by a show of hands, who  
13 here, patients and caregivers, who is experiencing or  
14 has experienced pain as part of their rare disease?  
15 Okay. Okay, so about a third or so. What about  
16 fatigue? Okay, a little bit more hands, maybe about  
17 half. What about challenging with movement, getting  
18 around? Okay, most people. And then one of the  
19 things that we heard a lot through the panel is that  
20 just the challenge of going through the day-to-day  
21 parts of life -- school and work. What about that?  
22 Have the people been -- okay, so about half.

1           Okay, so I want to hear just from a couple  
2 people in the audience. Who would like to share about  
3 some of those symptoms that you're experiencing? We  
4 do -- you know, if you feel comfortable, you don't  
5 have to use your first name, but, again, as Dr.  
6 Maynard said, if you would just identify the name of  
7 your disease or condition? Yes.

8           MS. HARTMAN: Hi, thank you. I'm Christina  
9 Hartman and my daughter, Charlotte, has a rare genetic  
10 disorder called NAA10. It's also known as Ogden's in  
11 boys. And one of the things I wanted to add to the  
12 list of difficulties is speech. So, one of the  
13 challenges for a lot of folks with rare conditions is  
14 being able to communicate. And my daughter Charlotte,  
15 like many of the NAA10 girls, had a very difficult  
16 time learning to walk. In fact, most of the girls are  
17 still in wheelchairs, but I think in large part due to  
18 really early and intense intervention, physical  
19 therapy twice a week for a year, Charlotte started  
20 walking at 2-1/2. However, we have not had the same  
21 intervention in terms of speech, and so she really  
22 struggles to communicate. You know, she can make a

1 few sounds and says "Da-da." She says "Mom" when  
2 she's mad and has started saying, "Head, head," but  
3 she'll be 3 this summer and that's about it. And so I  
4 would say that that's a pretty big concern as well,  
5 you know, for the future.

6 MS. CHITTOORAN: Okay, thank you. Yes?

7 LORIE: Hi. My first name is Lorie. My  
8 sister had Lennox-Gastaut disease, and I have two  
9 grandchildren, 1 and 3, with a rare genetic disorder  
10 called MECP2 duplication syndrome. I've noticed from  
11 having been a caretaker for my sister with Lennox-  
12 Gastaut, and also from my grandchildren, a few  
13 commonalities just in those two syndromes, and some of  
14 the things that were already said with the fatigue.  
15 One of the things that is a real big concern, I think,  
16 for a lot is the blow to the system in terms of  
17 immunity, because it makes the body so susceptible to  
18 comorbid problems. I see Adrienne shaking her head.  
19 And I think, Seth, you also mentioned having trouble  
20 sometimes telling the differences, is it the disease  
21 or is it a normal going through at your age. And  
22 that's something that parents have problems with

1 whenever my grandchildren catches a cold. Some of the  
2 kids with MECP2, they can't -- they have asthma, they  
3 have lung conditions, they end up on, you know, with a  
4 lot of breathing difficulties. And so every time one  
5 of the kids gets a cold, we wonder is this a precursor  
6 of something worse or is it just a normal cold?

7           And another common thing that a lot of rare  
8 diseases in children is seizures. And one of the  
9 doctors, I believe, asked about how well the medical  
10 community understands. And a tremendous problem that  
11 I had in taking care of my sister was that the medical  
12 community did not even recognize when my sister was  
13 having a seizure because there are so many different  
14 kinds of seizures, that just a staring spell or a fall  
15 could be seizure activity.

16           So, I don't want to hog up everything, but  
17 those are some of the things that I noticed in  
18 relation to what other people said as well.

19           MS. CHITTOORAN: Thank you so much for  
20 sharing. Is there anybody else in the room? We'll go  
21 to this side.

22           MS. WELT: Hi. This is for Adrienne. My

1 kids -- my name is Patricia Welt, and my kids have  
2 Ehlers-Danlos syndrome, which is an invisible illness.  
3 And you spoke a little bit about that, and when we go  
4 to the emergency room, oftentimes because my children  
5 look fine, their symptoms are dismissed, especially  
6 since they're young women. And my concerns are  
7 dismissed because I'm a woman, and I'm wondering if  
8 you run into -- you know, even if they're in severe  
9 pain, that it's something that -- it's kind of a fine  
10 line.

11 MS. CHITTOORAN: Just a reminder, we can  
12 address that in a little bit. I just want to make  
13 sure I'm hearing from other symptoms first before we  
14 go back to the panel. Thank you. Does by anybody  
15 else, maybe one more, and then I'll go to the web.  
16 Sure.

17 MS. STONE: Hi. I'm Geneva Stone, and this  
18 is my son Robert. Robert has dystonia 16, and he also  
19 has -- it's a genetic -- we used sequencing to get  
20 that diagnosis, and it's a very rare form of dystonia.  
21 And he also has the clinical symptoms of biotin-  
22 thiamin-responsive basal ganglia disorder. And he

1 experiences all of the common symptoms that were  
2 brought up here, and one thing that the FDA and  
3 doctors might think more about is Robert can't swallow  
4 due to his gastric symptoms. He has severe GERD, and  
5 he currently uses a G-tube and a J-tube for medication  
6 adjustment, and it can sometimes be extremely  
7 difficult to get liquid forms of medications. And we  
8 spent much of Robert's early years trying to compound  
9 medications ourselves until Robert had Medicaid waiver  
10 and we've been able to get pure vitamins, thiamin and  
11 biotin compound by our pharmacy. But those patients  
12 who are using vitamin therapies face significant  
13 hurdles because over-the-counter vitamins are  
14 typically, like, chock a lot with fillers and many  
15 people cannot tolerate fillers at that level. So,  
16 just wanted to talk about method of administration  
17 briefly.

18 MS. CHITTOORAN: Okay, thank you. Wendy,  
19 what are we seeing on the web? Do people have -- are  
20 people talking about some of the symptoms they're  
21 having and how are those maybe similar to the ones  
22 that we've heard already?

1 MS. SLAVIT: So, I'd like to address a few of  
2 the symptoms that have been mentioned online and then  
3 there is a question for Caroline. So, people have  
4 been talking about shortness of breath, lack of  
5 oxygen, feeling breathless. We also had someone  
6 online talk about inability to speak and swallow, and  
7 how difficult that is.

8 We also had a few people talk about pain and  
9 fatigue and pain management. And so the question for  
10 Caroline is, actually, how has your mobility dog  
11 affected your ability to get around and interact with  
12 society?

13 MS. SPENCER: So, I get way more attention  
14 now than I used to. It's very, like -- "Oh, there's  
15 Clark," and I'm like, "I'm here, too." So, and  
16 actually, like, being a quiet person and being more  
17 private about wanting to share with stranger about my  
18 -- why I walk like that or talk like that, that was an  
19 adjustment at first. But now -- so, I meet friends  
20 everywhere I go. He's a great conversation-starter,  
21 and really it's opened the door for me to be  
22 comfortable and relate to people who may not have



1 heard about Friedreich's ataxia before, and so it  
2 gives me a chance to educate, to help educate others.  
3 So, it really helps me relate to other people.

4 MS. CHITTOORAN: Thank you, Wendy.

5 MS. SLAVIT: No, not at this point.

6 MS. CHITTOORAN: Okay, thank you. So, we  
7 talked a little bit and heard a little bit about  
8 symptoms you're experiencing that are similar. I'm  
9 curious, are there others in the room that are  
10 experiencing symptoms that you feel are burdensome  
11 that we haven't already heard about or talked about?  
12 Yes?

13 MS. GILAZZO: Hi. I'm Elizabeth Gilazzo  
14 (ph), and my 4-year-old daughter has a rare  
15 neurogenetic syndrome called Angelman syndrome. So,  
16 as we think about our kind of two to three most  
17 burdensome symptoms, I'll echo two that have been  
18 talked about and bring up a third that we haven't  
19 mentioned.

20 So, children with Angelman syndrome, or  
21 individuals with Angelman syndrome are completely  
22 nonverbal, so I'll absolutely echo the impact on daily

1 life of having a child who can't communicate any of  
2 her basic needs and the frustration that comes from  
3 the lack of appropriate services and coverage of  
4 services for children with complex communication  
5 disorders.

6 The second is epilepsy, and access to  
7 appropriate care of people who recognize what is a  
8 seizure and how seizures might best be treated in  
9 individual syndromes. And then the third is sleep  
10 disturbances. So, I'm also a pediatrician. That's  
11 the other hat that I wear, and I care for lots of kids  
12 with pervasive developmental disorders, and this is a  
13 huge problem across the community. And if you want to  
14 talk about something that impacts caregivers' lives,  
15 is sleep disorder. So, kids in the Angelman community  
16 might sleep two to three hours a night, which really  
17 means that they need 24-hour-a-day supervision, and  
18 this -- the impact that has on caregivers and families  
19 is really quite huge and not unique only to the  
20 Angelman community.

21 MS. CHITTOORAN: Thank you. Anybody else?  
22 Yes?

1 MS. MAUGHAN: I'm going to stand up because I  
2 don't stand out enough in the hat. My name is Annette  
3 Maughan, and I am the CEO of KGB Foundation. It's a  
4 rare genetic disorder caused by mutation of the  
5 ANKRD11 gene. For our patient population, the number  
6 one complaint that they have is behavioral or impulse  
7 control. Those prevent the majority of our patients  
8 to go to school, especially when you have, you know,  
9 they're typical in every other sense, but you have  
10 this behavioral issue because of, probably, a lack of  
11 communication skills, because that is the second most  
12 impactful thing is delayed speech, delayed milestone  
13 walking. But it's always amazing to me to hear  
14 patients and their caregivers talk about that impulse  
15 control changes everything in a social setting, right?  
16 Because, I mean, we've all been there where you're out  
17 and somebody has an outburst and then everybody else  
18 in the room is kind of, "Oh," they're whispering, "it  
19 must be autism." Well, sometimes it's not autism;  
20 sometimes it's just something else. So, that for us  
21 is number one. Seizures are also up there, but I  
22 think that's about it for me. Just wanted to get that

1 out there. Thanks.

2 MS. CHITTOORAN: Thank you. And let me just  
3 go to the web. Is there anybody on the web, Wendy,  
4 that has anything different than what has already been  
5 mentioned?

6 MS. SLAVIT: No. I just wanted to say,  
7 actually, there was someone on line who was speaking  
8 on behalf of Angelman syndrome, also, and they also  
9 talked about difficulty sleeping.

10 MS. CHITTOORAN: Okay, thank you. I want to  
11 ask the panel, do you have anything, any questions for  
12 anybody you've heard from today? Otherwise, we can  
13 move on to the next question. Yes?

14 DR. MCCUNE: I just wanted to ask Seth a  
15 question, if I might?

16 MS. CHITTOORAN: Sure.

17 DR. MCCUNE: You had mentioned sort of three  
18 different domains -- the cognition, the movement and  
19 the mood swings, and that I believe your mother was  
20 treated for one, but it made -- the movement, but it  
21 made the mood swings oh, so much worse. Do you have a  
22 feeling for which one of them -- so, clearly, there's

1 kind of a risk-benefit discussion there. Is there one  
2 that you would have rather treated and taken the side  
3 effects? And would you answer that question  
4 differently?

5 MR. ROTBERG: I know we'll be discussing that  
6 in the next question, but I'll try to sum it up.  
7 Personally, I mean, unfortunately, I can't speak -- I  
8 can try to speak on what I would think she would want.  
9 The biggest thing is because you can actually visibly  
10 see the physical movements. That's, I think, why  
11 obviously they wanted to treat that aspect, but I  
12 definitely, you know, in my mind, I feel like I would  
13 personally rather deal with that mental aspect of it,  
14 the cognitive and psychiatric symptoms because, one,  
15 you can't see it and, two, those are just as  
16 important, if not more important. But I think it was  
17 tough, because, again, as we'll talk about in the next  
18 question, that was the only option.

19 So, what other options does she have? Yes,  
20 she took medication, like, for anxiety and depression,  
21 but there's only so much that that can do to offset  
22 the other medication. And, again, it did work for a

1 good amount of Huntington's disease patients, it just  
2 didn't work for her and there's, again, unfortunately  
3 no -- there wasn't an ideal drug or alternative for  
4 her to take.

5 DR. MCCUNE: Thank you.

6 MS. CHITTOORAN: Thank you. Okay, so I want  
7 to talk a little bit about how some of the symptoms  
8 you're experiencing, how you're managing them. We've  
9 heard the panel mention several things already --  
10 medication, opioids, Clark. We heard a little bit  
11 about supplements and vitamins. So, want to hear from  
12 a couple people in the audience. How are you managing  
13 your symptoms?

14 BONITA: Hi. Good afternoon. I am a patient  
15 living with Ehlers-Danlos syndrome, hypermobile type.  
16 For me, I've had so many different things, but the  
17 number one gold standard for treatment for Ehlers-  
18 Danlos is physical therapy. Now, the problem with  
19 that is insurance companies provide barriers, such as  
20 copays and the deductibles and caps on visits. I've  
21 tried medications. I've tried naproxen, I've tried  
22 Robaxin, I've tried heat. I've tried all kinds of

1 different things, but the number one most effective  
2 thing, from my second round in PT I've gone from at  
3 least 80% of pain down to about 60% of pain. So,  
4 that's like the number one thing, but, like I said,  
5 there are barriers.

6 MS. CHITTOORAN: Thank you. Anyone else like  
7 to share? Hi.

8 AUDIENCE MEMBER: I just wanted to add to  
9 Bonita's comment about physical therapy. So, with my  
10 daughter, the reason she's walking is that early  
11 intense intervention in terms of physical therapy.  
12 One of the challenges, at least in the state of  
13 Maryland, and I'm sure this is common across-the-  
14 board, the insurers are not required to cover physical  
15 therapy until you have a diagnosis. It took me a year  
16 of fighting to get a diagnosis. It's impossible to  
17 get insurance coverage for genomic sequencing most of  
18 the time, and I had a platinum Aetna Insurance plan at  
19 the time. So, I would just add that it all ties  
20 together.

21 MARIA: Hi. My name is Maria and I have a 6-  
22 year-old daughter with Prader-Willi syndrome, and the

1 hallmark symptoms of PWS are hyperphagia, which is a  
2 chronic insatiable hunger, and obesity. But for my  
3 daughter, the most burdensome symptoms are disordered  
4 sleep and slow processing speed. And what I love  
5 about this conversation is that she was just recently  
6 diagnosed with narcolepsy, and so we're actually  
7 managing her symptoms right now using natural  
8 stimulants and behavior modification. Because we now  
9 know that what the problem is, we're helping her get  
10 back to sleep, get better sleep at night, and we're  
11 exploring medications to treat the symptoms now of  
12 narcolepsy. And to your point about insurance,  
13 because we have a diagnosis now, it's easier for us to  
14 get medications covered.

15 MS. CHITTOORAN: Thank you. Yes?

16 AUDIENCE MEMBER: As far as physical therapy  
17 you mentioned, I will say we're managing for my  
18 daughter, but it's a double-edged sword because the  
19 children need physical therapy, occupational therapy,  
20 speech therapy, and they also need pediatric  
21 gastroenterologist, an ophthalmologist, you name it,  
22 they need the specialist. And my daughter ended up



1 having to quit her job just to care for the children,  
2 and so that's what I mean it's a double-edged sword,  
3 because so much isn't covered on insurance and it  
4 takes so much attention. And the sleep issues are  
5 sleep issues for my daughter, for mental health and  
6 psychiatric needs, my daughter needs to go for  
7 counseling. It's -- we can't neglect the family  
8 members. As a sister growing up, my parents tried to  
9 give them a normal life but, you know, the identified  
10 patient takes so much attention. And about speech,  
11 when you've got cognitive decline, that paired with  
12 the lack of speech makes it doubly scary and  
13 difficult.

14 MS. CHITTOORAN: Thank you. Yes?

15 MS. WELDON: Hi. Monica Weldon, and my son  
16 has a neurologic disorder called SYNGAP1, and I wanted  
17 to add this symptom because it's something that's kind  
18 of a hidden symptom. But I know that several of my  
19 rare disease leaders who are in our gut cohort that  
20 we've put together is constipation. And I will have  
21 to say that for us, we have to help my son go twice,  
22 twice a week because of a low muscle tone. And you

1 can -- it seems very simple, but with some of our kids  
2 that have the gut issues between, of course, the brain  
3 and the gut and the muscles and the peristalsis not  
4 working correctly, I mean, that could be maybe why  
5 they lack sleep. That could be why they're acting  
6 out. What are those things? And so, of course, us  
7 together as a group in the rare disease community  
8 looking at the autism component, I think it's very  
9 important. Because some of our kids have been  
10 hospitalized because they just simply cannot go. And  
11 so I wanted to kind of throw that out there as a  
12 symptom, and I know the moms and parents out here that  
13 live with that know that that's a big issue around  
14 that, and having to treat it, because it's something  
15 you can't not let go on a weekly basis.

16 MS. CHITTOORAN: Thank you. Okay, Wendy, I  
17 just want to hear if there is anything on the web that  
18 people are mentioning in terms of how they're managing  
19 their symptoms?

20 MS. SLAVIT: We also have someone mention  
21 speech therapy, which was already mentioned earlier.  
22 So, I just wanted to reiterate that as one of the

1 comments people have made.

2 MS. CHITTOORAN: Okay, thank you. Okay, so  
3 know that I didn't get to everybody so far, but I just  
4 want to remind people that we do have a docket open.  
5 It's open until the 30th of May, so just, please, make  
6 sure if you have comments that you didn't get to make,  
7 or if there is something you said and you missed out  
8 on adding something, please do so in the docket.

9 I just want to turn back over to our panel  
10 for about the last 30 minutes or so. I have a couple  
11 more questions for you and then we'll turn back to the  
12 audience and have sort of another discussion.

13 So, we'll start again with you, Adrienne.  
14 So, and this question is sort of related to the  
15 benefits of treatment and how you weigh those against  
16 -- how you and she might weigh those against potential  
17 side effects. So, when you're thinking about managing  
18 her disease, what potential benefits would you and her  
19 consider most important in your sort of decision-  
20 making process?

21 MS. SHAPIRO: Well, again, it depends if  
22 she's in the chronic phase or the acute phase. So,

1 the acute phase is when the pain is really, really  
2 awful. And so when she's in acute phase, of course,  
3 there is nothing for us to do but to go to the  
4 hospital and get more opioids, or whatever  
5 combination. It's funny, many of the warriors think  
6 of it as the decision to go to the hospital is a  
7 decision to -- between their mental wellbeing and  
8 their physical wellbeing. I mean, they really  
9 struggle to go for that.

10 So, I think for us it is really kind of -- we  
11 manage as much as we can before we have to go to that  
12 high level of meds. The meds in themselves, when  
13 you're on painkillers or opioids, and those kind of  
14 things, they cause a lot of these problems we've been  
15 talking about. There's constipation, there's foggy  
16 brain, there's more fatigue. There is this itching  
17 from -- if you can imagine itching from inside of your  
18 bones, you know, and then they need Benadryl, and then  
19 that becomes a struggle to get that. And so, really,  
20 pretty much it's always against am I so bad that I  
21 have to do these things? I mean, that's really what  
22 it is for us. And many times we end up with some real

1 complications because we put off going to the hospital  
2 for so long because of that.

3 I guess for us, when you talk about potential  
4 benefits, we have, like all communities, we have some  
5 really brave young people who are into doing trials,  
6 going and having bone marrow transplants, even though  
7 they know there's a percentage that it won't work,  
8 that they might end up with graft-versus-host, that  
9 particularly the girls will end up being infertile.  
10 And so that's kind of the thing is, I want this  
11 treatment. I want to help the community.

12 There's also a sense of others saying I'm too  
13 sick to get into trials, which is an interesting  
14 discussion to have. I had to have that one with my  
15 daughter, because her fear is that she was so sick  
16 that if anything went wrong, it might affect the  
17 outcome of the trial.

18 So, I think for us, because it's kind of a  
19 different than -- we have a diagnosis, it's part of  
20 newborn screening, so pretty much we're told from the  
21 beginning what the kids have. We have a protocol that  
22 pretty much -- 95% of our children live, where sickle

1 cell is sickle cell any other place in the world and  
2 it's the opposite -- 95% of the children die. And so  
3 I guess we're always kind of trying to weigh what --  
4 you know, what's the loss? What's the gain, what's  
5 the loss, and in many ways we're much more fortunate  
6 than many of the other people in this room. But it is  
7 a daily struggle, and in that daily struggle I think  
8 we're all united.

9 MS. CHITTOORAN: Okay, thank you. So, I  
10 think what we'll do is, just to make sure we're  
11 maximizing our time, I'll just go down the panel and  
12 then I'll turn to the FDA table and just see if you  
13 have any follow-up questions for any of the individual  
14 panelists. So, okay, so thank you. Thank you very  
15 much, Adrienne. So, Seth, if there was a treatment  
16 available for Huntington's disease, what benefits do  
17 you think -- would you think about when you're  
18 comparing, thinking about potential risks and side  
19 effects?

20 MR. ROTBERG: So, for me, I'm very fortunate  
21 enough that with Huntington's disease there's a lot of  
22 upcoming, I guess, trials in the pipeline, whether

1 it's -- we have one in Phase 3, we have some that are  
2 starting Phase 1. I think the biggest thing that I'm  
3 trying to teach myself is learning about the ins and  
4 outs of clinical trial development and getting the  
5 patient perspective from the beginning. Because when  
6 you think about it, there could be, as mentioned, an  
7 oral medication, but is it the liquid form or is it  
8 the pill form? There's gene therapy, there's a spinal  
9 injection, and try and understand what the needs are  
10 and interests of those patients. Can they take it  
11 once a week, once a month, once a year, once in a  
12 lifetime, and really try to understand and be patient  
13 with knowing what's in the horizon. So, am I going to  
14 take something that's now or am I going to wait for  
15 this next drug that could help me even more, three to  
16 five years down the road, or even 10 to 15 years down  
17 the road?

18 So, that's kind of something I really want to  
19 emphasize is, like, what are the options? What are  
20 the alternatives? And then for me it's kind of like  
21 let's see the data on the animal models and then the  
22 preclinical discovery stage, but let's get the young

1 adults' perspective, the older adults, the caregivers,  
2 all of them involved from the beginning versus once  
3 we're going through it. Because what I've learned is  
4 that once a patient is in a trial, you know, a lot of  
5 people see it as, hey, I need to get in this, I need  
6 to get in this, but it's a big commitment, and if you  
7 drop out, it's not like the next person can just come  
8 in. And that can really impact enrollment as well as  
9 if it eventually gets FDA approval.

10 MS. CHITTOORAN: Okay, thank you. If you  
11 were taking a medication that delayed the sort of  
12 development of the symptoms that you're experiencing,  
13 what kind of risks would you sort of be willing to  
14 tolerate, the side effects you would be willing to  
15 tolerate?

16 MR. ROTBERG: I mean, anything that's really  
17 going to slow it down, I definitely would be  
18 interested in. The challenge is, as mentioned before,  
19 because of the criteria, I can't even get involved or  
20 even get my perspective involved currently in any  
21 trials, whether it's preclinical or it's in a Phase 3.

22 So, for me it's trying to understand the ins



1 and outs about trials, that opportunity, and then  
2 trying to go from there. I mean, personally, if I had  
3 to choose between those three different aspects of it,  
4 I'd probably rather have the movement disorders, only  
5 because, you know, that cognitive and psychiatric, I  
6 think I'd rather try to manage that first, if I had to  
7 pick, based off what I saw in my mom of being  
8 depressed, wanting to sleep all the time, you know,  
9 having these mood swings. And then knowing that  
10 there's a higher risk of suicide is very scary,  
11 especially already dealing with a rare diseases and  
12 then having those thoughts on top of that can be  
13 challenging.

14 MS. CHITTOORAN: Thank you. Caroline, so I'm  
15 going to pose the same question to you. So, when  
16 you're thinking about managing your disease, what  
17 potential benefits you consider most in your decision-  
18 making process?

19 MS. SPENCER: Well, you know, based on the  
20 symptoms that affect me most, I would say better  
21 mobility, better balance, increased ability to walk on  
22 my own. You know, I have a cart, but I can't take two

1 steps without some kind of assistance. So, if I was  
2 able to take five steps without assistance, that would  
3 be an improvement. And improving my ability to keep  
4 up with friends, keep up with schoolwork, being  
5 engaged in daily life activities, that would be kind  
6 of the most benefit to me.

7 MS. CHITTOORAN: Okay. And how about when  
8 you consider that when you're thinking about the side  
9 effects, whether they're serious, very serious, or not  
10 very common, or maybe more common side effects?

11 MS. SPENCER: So, kind of the biggest thing  
12 that I weigh as far as side effects is my ability to  
13 do my job, to go to school. I feel it's really  
14 important to me to be able to have a life aside from  
15 FA, to do the things I would have done anyway. So,  
16 you know, there currently are no treatments, FDA-  
17 approved treatments for FA, but if there were, I think  
18 I would most weigh the impact on me still being able  
19 to go to school and do my job and, you know, have a  
20 normal life, so-to-speak. Yeah.

21 MS. CHITTOORAN: Okay, thank you. Does  
22 anybody, yes, I see a question.

1 DR. SILVERSTEIN: So, here at the FDA, I can  
2 speak for my center, but I think it probably goes  
3 across all of our centers. I'm in the Center for  
4 Devices. We consider patient preferences, patient  
5 perspectives, and patient-related outcomes as  
6 something that we must think about when we're looking  
7 at clinical trials that companies, investigators are  
8 interested in doing.

9 And with that as a background, I'm curious to  
10 know what's happening on -- as all of you have either  
11 yourself or your child comes to -- is introduced into  
12 a new therapy, and how much is that being considered  
13 what your preferences are? How much risk, exactly  
14 what Susan was trying to get at -- how much is risk  
15 considered related to benefit, but also what you might  
16 want to happen in a trial in terms of what you're  
17 willing to risk for a certain benefit? How much is  
18 that considered by your physicians or by trial  
19 specialists who are conducting a trial? Is that part  
20 of the equation or is it simply not part of the  
21 equation?

22 MS. CHITTOORAN: Sure. Adrienne?

1 MS. SHAPIRO: Well, I think I can -- and I  
2 can only speak for the ones that we've been involved  
3 with in the last, I guess, five years. It is very  
4 much part of the conversation. I think that for a  
5 long time it wasn't. I think for a long time that the  
6 patient voice or the patient experience was not a part  
7 of clinical trial development, and that through PCORI  
8 and other organizations, patients' perspectives have  
9 now been put front and center. I know the stem cell  
10 trials, even from the beginning the actual design of  
11 the trial has a patient or patient advocate there, and  
12 I think that they're changing now to even encompass in  
13 their trials that looking at the patient as a whole  
14 patient and the caregiver and family and what the  
15 effects of not only the trial, the medicine, whatever  
16 they're testing, but the experience of going through  
17 that will be.

18 So, I think it's a sea change in research,  
19 and I think it has to do with the fact that medicine  
20 at this point, medical research, doing stem cells and  
21 using your own tissue is so much -- I mean, it's much  
22 more personal than it's ever been before. And I

1 think, also, that we've learned some stuff, I mean,  
2 from software development, right? In the beginning  
3 with software they would create something somebody  
4 said be cool to be able to do that, but they didn't  
5 have the end user in the room. And now we've got the  
6 end user in the room, and I think it's going to help,  
7 or is helping to escalate and to streamline the  
8 process.

9 MS. CHITTOORAN: Anybody else on the panel  
10 have anything to add?

11 MS. WITTEN: Hi. It's Rachel Witten from  
12 CBER. I have a couple of questions for the audience.  
13 I know pediatric patient with rare diseases is very  
14 commonly presented with lack of sleep. They do sleep  
15 two, three hours, and my question to you, do you have  
16 any help from insurances, from the community? Because  
17 lack of sleep in our kids can affect our life, and how  
18 you as a parent, how you can go through the day if you  
19 don't sleep? What do you do?

20 MS. CHITTOORAN: Do we have a mic?

21 MS. WITTEN: And I'll explain why I'm asking.

22 MS. RASKIN: I'm Julie Raskin, and I have a

1 22-year-old son with congenital hyperinsulinism, and  
2 I'm also the executive director of Congenital  
3 Hyperinsulinism International and the Rare Action  
4 Network ambassador for New Jersey. And we have a  
5 campaign at CHI, which we call HI, which is  
6 hyperinsulinism for short. HI never sleeps, and so I  
7 don't have a solution, but this is such an enormous  
8 problem. With us, it's not about our kids not being  
9 able to sleep, it's about us needing to be up all  
10 night taking care of them and checking their blood  
11 sugars and making sure that they eat when they might  
12 not want to eat. We might have to trick them into  
13 sleeping in order to feed, and when you are dealing  
14 with something that's metabolic, it goes on and on and  
15 on, and it doesn't understand the normal biorhythms of  
16 day and night. And in the community of other rare  
17 diseases that I know, people living with the diseases  
18 in their parents, this is so much the case, that one  
19 part of it is a sleep disorder. But so much of it is  
20 just the weird rhythm of our lives and, yes, this  
21 affects our lives in so many ways -- ability to work,  
22 our ability to make money, our ability to care for our

1 other children, our ability to go to school, and so on  
2 and so forth. So, it's an enormous problem that needs  
3 a tremendous amount of focus.

4 MS. CHITTOORAN: Thank you. I think this is  
5 a great opportunity to hear more from the audience  
6 here in the room and then on the web. I'm just going  
7 to pose the same question to those of you in the  
8 audience. So, when you're thinking about managing  
9 your disease or condition, what potential benefits  
10 factor in most?

11 AUDIENCE MEMBER: I'm actually here  
12 representing two communities, because I have a newly  
13 diagnosed son, so I have two with Duchenne muscular  
14 dystrophy and an 11-year-old with primary immune  
15 deficiency. And you might think that the Duchenne is  
16 what I want to talk about, but I'm actually really  
17 interested in talking about our treatment of immune  
18 deficiencies today. My son was 11 when he was  
19 diagnosed in November, and the symptom was major  
20 infections that were causing hospitalization, so it's  
21 chronic antibiotic use followed by allergic reactions  
22 to antibiotics and immunities to the ones that he

1 could still take. And so now he's on a plasma  
2 treatment once a week, which comes with a lot of the  
3 same symptoms that he was getting because he was sick  
4 all the time -- chronic headaches, fatigue, rashes,  
5 unexplained vomiting, nausea. And so it's becoming a  
6 really pertinent discussion right now. He's not  
7 getting sick as often, but he's really sick from his  
8 medication, and so I think as we start to -- the  
9 physician and clinician environment was such that they  
10 said, hey, great, this kid has a treatment. Your  
11 other boys, you know, we didn't have much we could do  
12 for Duchenne muscular dystrophy, but there's a  
13 treatment for immune deficiency. Unfortunately,  
14 there's not a lot of research going on into new  
15 treatments because there is one, but it's a really  
16 horrible treatment.

17           And I now infuse my son once a week by myself  
18 because there isn't insurance coverage for nursing in  
19 a subcutaneous infusion at home. And then we have  
20 horrible side effects for half the week until it's  
21 time for his next infusion. So, really wanted to put  
22 that into the hopper, that just because it's a treated



1 disease doesn't mean that the treatment is sufficient.

2 MS. CHITTOORAN: Thank you. Anyone else?

3 MS. CISCO: Hi. Jill Cisco from acromegaly  
4 community. In our disease state we also have some  
5 approved medicines that treat our condition, but if  
6 they were perfect, I wouldn't be here today. Most of  
7 our injectables are long-acting injectable, and  
8 they're supposed to last 28 days, and patients start  
9 to have symptoms again typically at about day 21. And  
10 when you do the math, seven days every 28 days, that's  
11 more than three months a year that patients are  
12 suffering. And I would love to find something that  
13 could give patients a continuous amount of medicine  
14 every single day so that they weren't symptomatic.

15 Acromegaly, I don't know how many of you know  
16 about acromegaly, but it affects every single symptom  
17 in your body, because it's hormonal. It affects your  
18 appearance, it affects -- growth hormone makes you  
19 aggressive. It affects your moods; it affects your  
20 joints. It affects every little aspect of your life.  
21 And so, in my opinion, patients suffering seven days  
22 every single month because they're on a long-acting

1 medicine, we need to do better.

2 MS. CHITTOORAN: Thank you. Yes?

3 MS. O'BOYLE: Hi. This is Megan O'Boyle from  
4 Phelan-McDermid Syndrome Foundation, and Phelan-  
5 McDermid syndrome is on the terminal end of the 22nd  
6 chromosome shank, 3 is one of the guilty genes. And  
7 like countless syndromes, we look a lot like many of  
8 them -- autism, intellectual disability, epilepsy,  
9 sleep issues, GI issues, and I think the panel is  
10 probably going, okay, we're up to about 30 different  
11 syndromes that all look the same without seeing the  
12 genetic report.

13 Although my daughter suffers from a lot of  
14 the comorbidities, epilepsy is hideous, and I know  
15 families with PMS that have tried all 18 anti-seizure  
16 drugs. They all have side effects. They get given as  
17 cocktails, and it's really difficult, especially with  
18 a nonspeaking community, to really know the extent of  
19 the side effects, until you get the bloodwork done.  
20 And let me just tell you, getting bloodwork from this  
21 population is not a walk in the park.

22 So, I think to these other points that just

1 because there's an anti-seizure med doesn't mean it's  
2 working, especially for the genetic causes of these  
3 syndromes that also crossover into autism, GI issues.  
4 I mean, we could probably do a show of hands of who  
5 has these comorbidities. And so I just wanted to  
6 point to that, that just because there's, you know,  
7 seizure treatments, it doesn't mean the seizure  
8 treatments are working on this population, because for  
9 our families, they don't.

10 MS. CHITTOORAN: Thank you. I want to ask my  
11 FDA colleagues here, do you have any questions for the  
12 audience about anything that you've heard or any other  
13 questions?

14 MS. WITTEN: I have a comment. I'm from  
15 CBER. We do evaluate gene therapy trials, and what we  
16 start doing in trying to have some trial for a  
17 specific indication, we try to invite the community  
18 and talk to them about the endpoints. Because  
19 sometimes, you know, when you receive this clinical  
20 trial, the endpoint is kind of meaningless, and I'd  
21 like to hear from the community what would be this  
22 reason, you know, what we think and what condition you

1 will enroll your child in the clinical trial. And two  
2 different communities have told me, you know, the  
3 sleep disorder, we would like to treat sleep disorder,  
4 but we're not sure if it's reasonable or possible, I  
5 can tell honestly.

6 And what we're also started, we try for  
7 specific indications for clinical trial to have maybe  
8 one, if it's available, to have special government  
9 employee, somebody who knows the disease, or somebody  
10 has a child with the disease, to look at our clinical  
11 trial and kind of thinking, would they enroll myself  
12 or my child in this clinical trial when you have to go  
13 back every month, spend four or five hours for  
14 evaluation, or you will stay away from this clinical  
15 trial? And receive and we'll try to receive some  
16 comments from the parents or patient caregiver, what  
17 is your opinion on this design? Again, it's usable or  
18 friendly or whether the endpoints, and this is where  
19 we need your help.

20 MS. CHITTOORAN: Okay. Just take one more  
21 comment and then I'll go to the web because we're  
22 getting short on time.

1 MS. FOSS: My name is Beth Foss. I'm with  
2 the Choroideremia Research Foundation, and we have  
3 clinical trials happening now. We have two, and we  
4 have a third that's coming, so I appreciate your  
5 question and, sir, you asked that, too. I have a son  
6 -- actually, I have two sons. I'm really glad they  
7 sleep. At least we sleep, so I'm really appreciative  
8 of that. So, my older son, who is 26 now, was treated  
9 with a gene therapy up at Mass Eye and Ear, and so we  
10 are starting year three post, and it's been  
11 challenging in the sense that we go up -- as you say,  
12 we go up every six months and we spend a good day or  
13 day and a half. Any little aversion or change in his  
14 vision is -- it weighs heavily on them. Is it worth  
15 it? I would say yes. As a mother, it is worth it to  
16 undergo that. What we have a challenge with, and I'm  
17 with my colleague and friend, Eric Hartman, who has  
18 choroideremia, I'm a carrier mother, my father is  
19 blind, I have two sons, as I say, who have been  
20 diagnosed with it and are starting to see the  
21 blindness. It starts peripherally, and we lose about  
22 7%, 8%, 10% a year. And so one of the things, one of

1 the things that we are having -- the indicators is  
2 they measure the central vision, and much of our  
3 central vision is still perfect, 20/20, but we're  
4 losing peripheral vision. So, we have older patients  
5 that are in the trial, and if we could start with  
6 younger, when they're kids, we can preserve so much  
7 more of their vision. So, the endpoints is something  
8 that we're really trying hard to work with our  
9 researchers and with the FDA.

10 One other thing real quickly, I will say, as  
11 many of you are mothers and parents of children,  
12 that's one thing. I was looking at how do you weigh  
13 potential benefits versus less common risks? I think  
14 some of the younger in the Choroideremia Research  
15 Foundation, we have a robust organization. It's 1 in  
16 60,000, 50,000 people get this disease, and there's a  
17 lot of talk about sex selection studies. Do we have  
18 children? Do we not? And a lot of our members deal  
19 with guilt as a parent, and that's something that's  
20 really hard on them. Not sure how we tackle that one,  
21 but being a parent with kids that are hurting, I don't  
22 think there's anything quite worse than that.

1 MS. CHITTOORAN: Thank you. Wendy, just for  
2 the next 20, 30 seconds, is there anything you're  
3 hearing on the web in terms of this question?

4 MS. SLAVIT: Two people that have diseases  
5 that are quickly progressing, they say anything to  
6 slow down the progression until they're able to get a  
7 cure. One parent of a child mentioned that his career  
8 is impacted by not being able to sleep due to caring  
9 for his child in the evenings. And then also another  
10 parent mentioned that IV medication can be really  
11 difficult to administer to children.

12 MS. CHITTOORAN: Thank you so much. Okay.  
13 Well, I know we are sort of running out of time here,  
14 but I just want to reiterate that if you didn't get a  
15 chance to share it today, either in the room or  
16 through the web, please submit comments to the docket.  
17 Again, it's open until May 30th. We'd love to hear  
18 more about you, your symptoms your experiencing and  
19 the things that you're considering in terms of  
20 treatment.

21 So, now I just want to introduce a special  
22 speaker we have here, Dr. Amy Abernathy. Dr. Amy

1     Abernathy has a dual role here at FDA as principal  
2     deputy commissioner and acting chief information  
3     officer. As principal deputy commissioner, she helps  
4     oversee the agency's day-to-day functioning and  
5     directs special and high priority initiatives that cut  
6     across offices overseeing FDA's regulation of drugs,  
7     medical devices, tobacco and food. As acting chief  
8     information officer, she leads our data software and  
9     computer hardware efforts to help us to continue to  
10    advance public health agenda. Dr. Abernathy is a  
11    hematologist and oncologist and palliative medicine  
12    physician. Before coming to FDA, she was a professor  
13    of medicine at Duke, and most recently worked in the  
14    health technology industry at a cancer-focused  
15    software and data company. She is an internationally-  
16    recognized clinical data expert and a clinical  
17    researcher, and one of the early pioneers in  
18    bioinformatics. We are very proud that Dr. Abernathy  
19    has joined us in February to continue to advance our  
20    mission in patient-centered care and public health.  
21    She has been a longstanding patient advocate and is  
22    well known across the patient community for her



1 commitment to ensuring that the voices of patients are  
2 heard. So, please welcome Dr. Abernathy.

3 MS. ABERNATHY: Thank you. Thank you very  
4 much. So, first of all a hearty and appreciative  
5 thank you and welcome to all of you here. We greatly  
6 appreciate your being here, contributing your voice to  
7 this meeting, to the docket. We also appreciate all  
8 the folks who are on the web. We know you're there,  
9 so also continue to participate.

10 So, as you just heard, I'm new to the FDA,  
11 and I've been here about two months, both as principal  
12 deputy commissioner as well as the chief information  
13 officer. And I thought I would take just a few  
14 minutes to tell you a little bit about myself, how I  
15 got here, and then bring that to the conversation  
16 we're having here today about the patient perspective  
17 in rare diseases.

18 As you just heard, I'm an oncologist. So, I  
19 spent a fair part of my career taking care of patients  
20 with melanoma, and particularly patients who had a  
21 family history of melanoma and who had advanced  
22 disease. And, really, to this day, one of those

1 patients, who actually in some ways represented all of  
2 the different patients coming through my clinic, but  
3 she had a way of sort of sitting on my shoulder and  
4 talking to me for the rest of my life. And her name  
5 is Janet, and Janet and I got to know each other in  
6 the mid-2000s. And I remember her coming into my  
7 clinic. She's got this really curly red hair. She  
8 had freckles on her nose. She was about 35. She  
9 always wore scrubs because she was an ER nurse,  
10 happened to be an ER nurse from not far across town,  
11 and had this bounding energy. And I have this little  
12 couch thing in my office. She never sat in any of the  
13 patient spaces; she insisted she was going to sit on  
14 the couch when we had these conversations in clinic.  
15 And one of the first things I noticed here in my  
16 melanoma clinic was that a woman with a fairly  
17 advanced cancer at that time was on prenatal vitamins.  
18 And as we were trying to figure out how to take care  
19 of her, one of the issues was that she was asking me  
20 about the impact of the treatments that we were  
21 talking about and what was going to happen to her  
22 fertility. And, in fact, I couldn't answer any of

1 those questions. And we would talk about whether or  
2 not her mother's melanoma and her sister's melanoma  
3 had any impact on how her personal illness was going  
4 to go. I wouldn't have any answers to that, either,  
5 although I was pretty confident that this was, indeed,  
6 a genetically derived and motivated illness.

7 I remember sitting in clinic as we were  
8 talking and she's like, "You're typing it all into  
9 that machine over there, gosh forbid, just go over  
10 there and do some queries and you'll be able to answer  
11 all these questions for me." And I couldn't do that.  
12 And, in fact, I was so frustrated with not being able  
13 to do that, and she was so frustrated with me for not  
14 being able to do that, that we really worked through  
15 that period of time in trying to figure out ways to  
16 unlock the patient personal story from the computer  
17 systems and turn that into reliable underlying  
18 information that we could use to figure out how to  
19 take care of Janet, and so that Janet's story could be  
20 reinvested in her legacy in the future.

21 In fact, all of the work I did of the  
22 subsequent 10 to 15 years, and how I landed at FDA was

1 really to figure out how could Janet's story be  
2 informed by all the people who came before her who had  
3 similar problems and questions -- what does this mean  
4 for my fertility, and how her story could live on as  
5 we continue in the future. And we worked on this at  
6 Duke at the time through building computer systems and  
7 patient reported outcome systems. Ultimately, I  
8 discovered that any one organization in one place just  
9 sort of hit the boundaries of what's possible, so I  
10 jumped the line and took a right-hand turn and ended  
11 up in a tech company, in a startup, thinking maybe if  
12 I motivate the tech industry we can do it from that  
13 direction. Actually, we got a long way, but then one  
14 of the things I realized was that in order to continue  
15 to motivate the story going forward, one of the places  
16 that's got as much bloody pulpit as anywhere in the  
17 world was to come to FDA, and that's why I came to  
18 FDA, and in a lot of the ways, the same reason that  
19 you're here today at FDA as well.

20           Interestingly, in December of 2016, Congress  
21 passed a piece of legislation called the 21st Century  
22 Cures Act. That piece of legislation asks us to

1 modernize our process of drug development, a medical  
2 product development overall so that ultimately we can  
3 figure out what works as efficiently as possible and  
4 right size those treatments for the patients who need  
5 them. And I have been very encouraged by 21st Century  
6 Cures and all the other legislation that goes along  
7 with it, but it's only a starting point. It asks us  
8 to do things, like learn how to use data better to  
9 modernize our clinical trials, to include the patient  
10 voice and patient report outcomes, to include the  
11 patient race in developing our evidence development  
12 programs. It asks us to do that, it doesn't specify  
13 how. And ultimately going from the motivation of,  
14 please, let's move this process forward and setting  
15 the guideposts to now moving to the how do we do this?  
16 How do we get the patient perspective, the caregiver  
17 perspective, the advocate perspective into this  
18 process is one of the things that this meeting is  
19 intended to be a part of and the work going forward.

20 Here at FDA, my core responsibility as a  
21 principal deputy commissioner is a series of trans-FDA  
22 policy responsibilities and commitments. So,

1 including, for example, rare orphan diseases in  
2 pediatrics, where, really, what we're trying to think  
3 about is how do we advance the work in these critical  
4 areas? And also includes the Patient Affairs staff,  
5 who also sits right next to Rare Diseases and  
6 Pediatrics, so we can make sure that we incorporate  
7 the patient voice, and we also have as many two-way  
8 communication channels as possible.

9           But you're probably wondering how in the  
10 world did a person who is the principal deputy  
11 commissioner also end up as the acting chief  
12 information officer? That's because ultimately if  
13 we're going to do this work, if we're going to scale  
14 to the many, many diseases that affect you, and the  
15 continuous learning processes it's going to take to  
16 get this done, we've got to scale our capabilities  
17 inside of FDA, and that includes using data and  
18 technology to do so. And we have to build the  
19 handshake points so that we're able to handshake as  
20 efficiently as possible to all of the other different  
21 parts of the biomedical community so they can scale  
22 their processes as well. So, that's why these two

1 pieces are together.

2 A bit about incorporating the patient  
3 perspective for rare diseases into the work that we  
4 do, and I just want to kind of hint on a couple of  
5 things. You know this better than me. You are the  
6 experts in your conditions. You're the experts in the  
7 lives that you're living and what this feels like to  
8 you every day. There are as many commonalities are  
9 there are differences. Part of our goal today is to  
10 identify those commonalities, but also to be very  
11 respectful of the fact that there are differences.

12 As I was just sitting here listening even for  
13 just the last half hour, I was thinking about some of  
14 the commonalities that come through, for example, the  
15 conversation about sleep. The end user, the fact that  
16 ultimately you want to go to school and do the other  
17 things that are important in your life, right? And  
18 many of these aspects we can share across our stories,  
19 but I also think about what's unique about our disease  
20 and how do we have to understand and deal with those  
21 capabilities as well? At FDA, we need to understand  
22 the full totality of the story so that when we think

1 of clinical trials and also the understanding of how a  
2 medical product works, we're able to do so in putting  
3 into the context of the experiences that you have. To  
4 develop endpoints and outcomes that align with the  
5 experiences that you have, and to be able to  
6 communicate across the overall biomedical spectrum  
7 within the context of the experiences that you have,  
8 including into the payer space.

9           Somebody asked me a little earlier today if I  
10 had any advice for the patients communities, what that  
11 might be. And the first piece of advice is use your  
12 voice. You're here today because you're using your  
13 voice. You're on the web today because you're using  
14 your voice. But continue to stand up and to tell your  
15 stories, because there is no other way to do this  
16 besides to break those stories, come together in  
17 totality as a whole.

18           My second piece of advice, though, is  
19 actually almost exactly the opposite. As an  
20 oncologist, I always had to remind myself our jobs are  
21 always to have compassion. As a patient and as a  
22 daughter and as a mother, I also need to have



1 compassion. So, one of the things that can be really  
2 hard when we've had too much pent-up energy to use  
3 your voice is also to want it to move really fast, and  
4 so have compassion that we're listening, but also  
5 we're going to need to figure this out together, and  
6 it may not move always as fast as we all want it to,  
7 but that making sure that we don't give up in  
8 continuing to put our voice to the story.

9 Use the docket. Put your information into  
10 the docket, encourage your compadres to do so as well,  
11 because that is truly a mechanism where we call and  
12 create a robust totality of the story to be able to  
13 use for our internal work and process making.

14 And I think my last point is that as we think  
15 about the patient community, think about our caregiver  
16 community as well. Often, we talk about patients  
17 because we're thinking about developing medical  
18 products individually for diseases and for patients,  
19 but as we have compassion for each other, we also have  
20 to have compassion for caregivers, because it is also  
21 a huge part of the story. So, with that, I just  
22 wanted to say thank you. I'm here to answer

1 questions, and thank you for all that you do. So, I'm  
2 going to do this or should do questions?

3 MS. CHITTOORAN: Any questions? Yes, sir.  
4 Do we have the microphone?

5 MR. HARTMAN: Thank you, Dr. Abernathy. My  
6 name is Eric Hartman. I told you I'd get choked up.  
7 And if I get choked up, it's fine with Eye Foundation.  
8 (inaudible) and I can go right through it without  
9 getting choked up. Weird reaction. But I wanted to  
10 talk to you about your idea of a commonality of data.  
11 Eye foundation is incredibly fortunate. We've been at  
12 it for almost 20 years, and we have two gene therapy  
13 trials underway. Here we go with British, sorry about  
14 that -- and a third about to start. The biggest  
15 problem we are having, and I see it as a commonality  
16 with all our rare and ultra-rare diseases, is fighting  
17 the fatigue of those patients who are available to be  
18 in natural history studies or any of the other medical  
19 device studies, anything along like that. Because our  
20 natural history study, our personal health information  
21 is locked. I'm a prime example.

22 I was in a two-and-a-half-year study, natural

1 history study quarterly. I had to fly all the way to  
2 Portland, Oregon to be in that study. After two and a  
3 half years, as it turns out, my progression, I only  
4 have one degree left in my central eye, it's too  
5 advanced for me to have -- or it's too risky to have a  
6 subretinal injection. So, there's another potential  
7 therapy or even other, like, cell replacement therapy.  
8 My two and a half years of data is locked. I can't  
9 get to it because they say the study is still ongoing.  
10 And there should be something for the rare disease  
11 community and the ultra-rare disease community where  
12 we can at least get our natural history study stuff  
13 that is observational, not therapeutic, to get an  
14 electronic copy of that so we as a patient community  
15 can move forward. The duplicity and the expense of  
16 all of that is huge. And if there is some way that  
17 the FDA can get together where we as a patient  
18 community or as the ultra-rare community can gain  
19 access to this natural history data, it will advance  
20 the science quicker and reduce the costs involved in  
21 these multiple therapies that are now available, or  
22 soon to be available.

1 MS. ABERNATHY: (Off mic.) Because the  
2 information, the natural history data, is critical to  
3 essentially setting the baseline for which different  
4 new treatments are compared. It also helps you  
5 predict what's going to happen, and helps you  
6 understand how to compare your story against others.  
7 So, it's important for all those reasons. Solving  
8 this problem from a technical perspective is different  
9 in the rare disease community than it is for an  
10 illness like type 2 diabetes, and so the technical  
11 solve needs to be available for situations where  
12 there's only a few hundred or a few thousand patients.

13 So, my advice is that we need to put this on  
14 our list, our working list of things to continue to  
15 think about. It actually is a problem that has to be  
16 solved from multiple sides, across government, across  
17 tech, across the patient community, but as a patient  
18 community, continue to agitate for it, because you've  
19 got the most powerful voice in the story.

20 I think we have time for one more question  
21 and then we'll need to --

22 KRISTEN: Hi. I'm Kristin, and I represent a

1 community of PTEN hamartoma tumor syndrome patients, a  
2 rare genetic heredity cancer syndrome with a whole  
3 neurocognitive component. This is more just a comment  
4 to what he said and what you said. The importance for  
5 sharing data and being able to collect that data for  
6 patients is critical. And what we're seeing more and  
7 more in the rare disease community is patient  
8 organizations are becoming wise to that in starting  
9 patient-driven registries, which we have recently  
10 done. But the purpose is not to hold the data for  
11 ourselves; it's to be able to open it up to all  
12 researchers and also share snapshots of that data with  
13 our patient community, which we're doing. So, it's  
14 possible; you just have to have motivated patient  
15 organizations.

16 MS. ABERNATHY: I 100% agree. I'll tell you  
17 that Janet, my patient, this was exactly what she  
18 ultimately pushed to do, and I do see it as a part of  
19 how all of us in this room can solve the problem  
20 together.

21 I am going to stop and switch gears for a  
22 moment, because we have a special announcement. So,

1 you've already heard earlier about the Beyond the  
2 Diagnosis exhibit, which is really intending to  
3 provide a face to all 7,000 and more rare disease.  
4 This whole exhibit is traveling across the country.  
5 It's going to medical schools and research institutes  
6 and hospitals around the globe, even, so even beyond  
7 United States, to look beyond the diagnosis of the  
8 patient. And so today the founder of the Beyond the  
9 Diagnosis exhibit -- oh, she's right next to me, there  
10 you go -- is Patricia Welton, and she's joined us to  
11 unveil the newest piece.

12 MS. WELTON: Do you mind if I just say  
13 something about the girls? Before I unveil this,  
14 these girls have Ehlers-Danlos syndrome, like my kids.  
15 They both had several brain surgeries. One is tube  
16 fed. They cannot attend school because they're too  
17 sick. And I want to say that in rare diseases, things  
18 are not always the way they seem, and this is -- I  
19 think this is a perfect example of that. Are you  
20 ready?

21 MS. CHITTOORAN: Yes.

22 MS. WELTON: You guys are the first to see

1 this. [Applause.]

2 MS. CHITTOORAN: Okay, everyone. Thank you  
3 so much as we just wrapped up the beautiful unveiling  
4 here. I know we're a couple of minutes behind, but I  
5 just wanted to thank the panel so much for sharing  
6 your perspective with us, as well as those in the  
7 audience and on the web. It is -- I want to make sure  
8 we have a 15-minute break. So, it's like 3:01. Let's  
9 try to be back here about 3:15 or so, to get started.  
10 Thank you so much.

11 [Break.]

12 MS. CHITTOORAN: Okay. Hi, everyone. We're  
13 going to get started, just because I want to maximize  
14 the use of our time here. So, we are getting ready to  
15 start Session 2. This will be another facilitated  
16 discussion between our panel here and our audience in  
17 the room and on the web. This session will be focused  
18 on commonalities in clinical studies and registry  
19 considerations.

20 So, just some reminders before we get  
21 started. If you don't get to provide feedback today,  
22 please do so on our docket, and the just so everybody

1 knows, we do have a large audience on the web as well.  
2 There's almost -- about 500 people or so on the web as  
3 well, so in addition to what we have in the room, so  
4 that's very exciting.

5           So, what I will do is, we will go do some  
6 introductions here for our panel, and then I will pose  
7 the questions to them, and then we will then turn back  
8 to you. So, if we would just start with Julie here,  
9 and we'll just work our way down. Just, if you would,  
10 please introduce yourself, your name, your role, if  
11 you're a patient or caregiver, and a little bit about  
12 the disease or condition that you're representing here  
13 today. And then just because people may not be  
14 familiar with it, please just provide like a couple  
15 sentences, a little bit about what that is. So, thank  
16 you. Julie?

17           MS. RASKIN: My name is Julie Raskin and I  
18 live and work in New Jersey with my husband, and we  
19 have two children, Hannah and Ben. They're both young  
20 adults. And Ben, who is the younger one, was born 22  
21 years ago, almost 23, with congenital hyperinsulinism.  
22 And congenital hyperinsulinism is a disease that



1 causes the overproduction of insulin, and that causes  
2 severe hypoglycemia, or low blood sugar. And the  
3 brain and the body need blood sugar to grow and  
4 develop, so if it's not controlled early on, it could  
5 cause brain damage or death. And a lot of what you  
6 all talked about this morning really, really resonated  
7 with me. And we sort of think of hyperinsulinism, a  
8 lot of us it can be like a global insult to the  
9 family, not necessarily just the brain, but to the  
10 family, for all the reasons that you all spoke about.  
11 And I think that in and of itself is a commonality  
12 among so many of the rare diseases. So, thank you for  
13 giving an opportunity to meet you all.

14 MS. CHITTOORAN: Thank you, Julie. Michael?

15 MR. BUSBY: Good afternoon, everyone. My  
16 name is Michael Busby. My wife Kristin and I have  
17 three children, Alexandra, 8 years old; Michael, 6;  
18 and Joseph, 3. Alexandra and Joseph were both born  
19 with progressive familial intrahepatic cholestasis, or  
20 PFIC2. We are -- anyhow, sorry. We are -- I'm happy  
21 to be here to talk about rare disease. Like Julie  
22 just said, a lot of what you all said this morning

1 certainly resonated with us. One of the topics was,  
2 you know, sleep disorder, or patient care due to lack  
3 of sleep, so that definitely hit home. I also sit on  
4 the board of directors for the American Liver  
5 Foundation in Greater New York. Thank you.

6 MS. CHITTOORAN: Thank you, Michael. Monica?

7 MS. WELDON: I'm Monica Weldon, and I am the  
8 mother of five children. I'm married to my husband  
9 Chris. My children, I should start, I guess, with my  
10 oldest three: Hailey, Taylor and Sawyer. They are my  
11 three adult children. Started over and I have twins,  
12 11-year-old twins next week, and one of my twins,  
13 Beckett, has a condition called SYNGAP1, and this is a  
14 neurological disorder, developmental disorder that  
15 causes intellectual disability. It is also an  
16 epilepsy gene or condition, where he has absence  
17 seizures and it is also an autism gene, where we  
18 struggle, of course, with the behaviors, sleep issues,  
19 a lot of sensory processing issues, muscle  
20 coordination problems. And, of course, now, as we  
21 move through to learning more about this disorder,  
22 because it's newly -- pretty much newly discovered, a

1 lot of immune system problems, a lot of digestive  
2 problems. And I am also the president and CEO of the  
3 Bridge the Gap SYNGAP Education Research Foundation,  
4 who is focused on research for this disorder, and I  
5 appreciate the opportunity to come and share.

6 MS. CHITTOORAN: Thank you so much. Okay.  
7 So, I'm going to move into some of the questions. And  
8 I will address you by name, and then like before, my  
9 colleagues here at the FDA table may have some  
10 additional follow-up questions for you, so we'll make  
11 sure that we pause and have that opportunity, and  
12 after that we'll move to the audience.

13 So, Julie, we'll start with you. So, I  
14 understand that you have some experience with both  
15 clinical trials and registries. I was wondering if  
16 you could tell us a little bit about both your  
17 experiences with those things?

18 MS. RASKIN: Sure. So, I have those  
19 experiences personally with my son, and also as the  
20 executive director of Congenital Hyperinsulinism  
21 International. And in terms of clinical trials, we --  
22 when my son was born, there weren't any FDA clinical

1 trials, but there were some -- well, they might have  
2 been FDA, but they were smaller, investigator-led  
3 trials. And we very much benefited from some  
4 activities like that that were at one of the leading  
5 centers in the world, the Children's Hospital  
6 Philadelphia, for treating congenital hyperinsulinism.  
7 My son basically could not get home from the hospital  
8 and start his life without clinical experimental  
9 protocol that included using two off-label medications  
10 in an off-label device. And so after some surgeries,  
11 we were able to go home on that kind of regime. So,  
12 that was an absolute godsend.

13           And through the years, through the work with  
14 CHI, for short, Congenital Hyperinsulinism  
15 International, I've had the opportunity to work with  
16 investigators, researchers and some biotech companies  
17 starting early in preclinical phases where we have an  
18 opportunity as a patient organization to share our  
19 experiences. And then moving forward with some work  
20 on the clinical trials themselves and developing  
21 protocols. So, that's clinical study. Did you want  
22 me to talk about registries?

1 MS. CHITTOORAN: Sure.

2 MS. RASKIN: Okay, great. So, as a  
3 community, our congenital hyperinsulinism community  
4 started a -- just an Excel spreadsheet, where we  
5 collected some information, natural history  
6 information about the condition, and that was done by  
7 a lovely colleague of mine, Isabel Calderon. And it  
8 was really a backbone, very important to our community  
9 in understanding how the condition affected others in  
10 our community and that helped us to benchmark sort of  
11 where we are and what was happening to our children.  
12 And that was our sort of launching pad. And then we  
13 wanted to go farther and develop a really -- a real  
14 patient registry with an institutional review board  
15 and with steering community members, professional  
16 scientists and researchers, and patient advocates from  
17 around the world to really guide this work. And so we  
18 did that and it took us years and years and years.  
19 We're the opposite of Monica. Very slow in our  
20 development, but with a very focused purpose to try  
21 and be as comprehensive as we could in creating it,  
22 and we went live in October. And then I've had the

1 experience with Benjamin, my son, being part of that.  
2 And so that was extremely interesting, and to see the  
3 effect on him of sharing his data and what it meant to  
4 him to be part of that was very meaningful and  
5 compelling for him.

6 MS. CHITTOORAN: Thank you. So, what -- when  
7 you're going back to the clinical studies, so what  
8 factors did you consider when deciding to participate  
9 in that?

10 MS. RASKIN: Well, as I said, this disease  
11 was such a global insult to the family. My son was  
12 always just a love, just delightful, and we adored  
13 him. And he brought us so much happiness even in the  
14 depths of our depression and grief over the disease.  
15 But we were really desperate, and there was no way to  
16 go home without participating. And we, you know, in  
17 terms of -- we were already in a hospital. We were  
18 already cycling through potential different treatment  
19 possibilities, and so it wasn't even in our mind that  
20 this was somewhat experimental, because everything  
21 seemed kind of experimental. There was no treatment.  
22 So, our biggest consideration really was listening to

1 the professionals, and we were so lucky that CHOP had  
2 been studying and caring for patients with this  
3 disease for already a pretty long time, even though it  
4 was so rare. And so we really trusted in them in  
5 terms of deciding to do this, but over time, fast-  
6 forward to know, and my son was in -- he traded his  
7 rare disease for a common disease, because his  
8 pancreas was removed. So, he went from having  
9 hyperinsulinism to being diabetic. And so he had an  
10 opportunity to participate in a trial that was a pilot  
11 study also at CHOP, and for the artificial pancreas.  
12 And it was an incredible experience for him to be able  
13 to give back to science and to be part of something  
14 that could really be life-changing for him in the  
15 future.

16 He had some disabilities because of the  
17 hyperinsulinism, and so one thing that I think is  
18 important to think about in clinical studies is how  
19 disability affects the ability to participate. Even  
20 if it's -- it's not about inclusion-exclusion, but  
21 just accessing the basics of being in a trial, and  
22 that's something maybe people want to get into later.

1 MS. CHITTOORAN: Thank you. Does anybody on  
2 the FDA panel have any questions they'd like to ask  
3 Julie? And I just want to remind you that when you're  
4 speaking, just make sure that the mic is close to your  
5 mouth so that everybody on the web, especially, can  
6 hear. Anybody from the FDA table? Any questions?  
7 Okay, we answered them all.

8 Okay, Michael, we'll move on to you. So, if  
9 you would tell us a little bit about your caregiver.  
10 So, will you talk a little bit about your daughter's  
11 experience with clinical studies?

12 MR. BUSBY: Sure. So, for us at two months  
13 old, we knew something wasn't right with our daughter.  
14 She was consistently scratching herself, you know,  
15 pretty regularly. There wasn't a minute that went by,  
16 and no matter how hard we tried to cover her hands,  
17 she would get out of it. It was pretty incredible,  
18 actually. But she would tear her nose and tear her  
19 ears and tear her eyes up, and it was really baffling  
20 to us, because most people that would come over or  
21 talk to us family members, they would say, "Oh, she's  
22 probably colicky or gassy," or, "She's going to grow



1 out of it."

2 At two months old we first went to the  
3 pediatrician and the pediatrician really, you know, he  
4 didn't have anything. And from there we saw every  
5 specialist you probably could imagine and not one  
6 person could tell us that there was something wrong  
7 with our daughter. It was actually pretty incredible.  
8 We didn't sleep. She scratched herself all night  
9 long. We couldn't put her in a bed. People talk  
10 about co-sleeping; that didn't work. It was pretty  
11 incredible to watch a baby scratch herself  
12 legitimately 24 hours a day. She wasn't jaundiced,  
13 she wasn't anything that you would think of. She just  
14 scratched.

15 So, fast-forward, we saw numerous doctors at  
16 about 13 or 14 months old. It kind of just went away  
17 on its own. It was very strange. And she slept, and  
18 it was very new, and we were waiting for something  
19 else to happen, because something just didn't seem  
20 right. And so we said maybe everybody is right; maybe  
21 she just is colicky.

22 Twenty-four months old, it was incredible

1 that it came back and even worse than it was before.  
2 At that point, we had already seen about 12 different  
3 specialists and nobody could really understand what it  
4 was. We went back to a pediatric dermatologist, and  
5 this is kind of -- and I talk about this and it really  
6 was that aha moment that was a complete accident. And  
7 the dermatologist said this is not dermat-related, but I  
8 want to give you some type of medicine that will help  
9 her sleep. He prescribed her a drug, and on Thursday  
10 night we started giving that drug to her. On Saturday  
11 night I was in a wedding and I came back on late  
12 Saturday night, Sunday morning, and my daughter was as  
13 bright yellow as she could possibly be. It was  
14 actually incredible.

15 Thinking back to it, you know, we knew  
16 something wasn't right. To see where it wound up  
17 putting us, you know, it was almost satisfying that we  
18 found out that there's something not right. By the  
19 time we got to the doctor, they rushed us to Mount  
20 Sinai in Manhattan and we were told our daughter was  
21 in acute liver failure.

22 So, it's a lot. That's a lot to handle. She

1 was just about two and a half years old at that point.

2 When we got through Mount Sinai and the  
3 doctors there, who had seen a lot of pediatric liver  
4 stuff or disease, they informed us that she had PFIC2.  
5 I didn't touch on this before, but PFIC2 is a genetic  
6 mutation within the liver that prevents the excretion  
7 of bile out of the liver. The bile winds up building  
8 up and thus gets into your bloodstream and body.

9 So, when we found out that there was  
10 something legitimately diagnosed here, it was kind of  
11 -- it was a relief and an "Oh, my God" moment at the  
12 same time. I read a lot. I recall that I read  
13 probably 20, 30 hours a day. And one of the things  
14 that I read was on clinicaltrials.gov, and there was  
15 nothing at that point available. You know, there was  
16 no trials, you know, drugs that just kind of limit the  
17 pruritus, but she was itchy just all the time. At the  
18 worst she had a 23 bilirubin. That is pretty yellow  
19 for being jaundiced. And her INR was extremely  
20 elevated, which her liver function was pretty bad.

21 You know, about a year after that we kept  
22 reading and reading, and ultimately on

1 clinicaltrials.gov found a trial that was starting and  
2 in Phase 2 that basically offered a solution to  
3 getting bile through the system so that the itchiness  
4 or the pruritus would go down. And fortunately she  
5 has been in that trial since, I think it's been about  
6 four years now, and her bile acids are within range  
7 and she is functioning at a normal 8-year-old life.  
8 It's a pretty amazing experience, for sure.

9 MS. CHITTOORAN: Thank you. Thank you so  
10 much for sharing that. When you -- so, when you  
11 mentioned that you initially saw the trial on  
12 clinicaltrials.gov, where there things that you  
13 considered in terms of whether or not to join that  
14 clinical trial?

15 MR. BUSBY: So, for my family, when you're  
16 faced with the -- and much like everybody. You know,  
17 when you're faced with the decision of the end result  
18 is a transplant, you kind of look at every solution  
19 before you have to get to that decision. For us, it  
20 just seemed -- to me it seemed unreasonable that there  
21 was not some type of medication that could help this,  
22 or at least prolong it to get to me not making a

1 decision at two years old or three years old to have  
2 my daughter liver transplanted. And just to kind of  
3 going back to how far we would have gone, I would have  
4 gone all the way to that point of transplant to try  
5 something. You know, and we were told very  
6 specifically that there was no guarantees and side  
7 effects, and so forth. And fortunately for us, the  
8 side effects are minimal and she's doing really well.

9 MS. CHITTOORAN: So, if you're sort of  
10 searching for clinical trials in general, including  
11 the one that you already were doing, was there  
12 anything, or is there anything that you might see that  
13 might make you less willing to consider being a part  
14 of a clinical trial, whether that's time commitment or  
15 distance from your home? Is there anything that you  
16 could identify that might make you less willing to  
17 consider?

18 MR. BUSBY: No. And I'll back that up by  
19 saying that we currently, much like Julie, travel from  
20 Long Island, New York to Children's Hospital in  
21 Philadelphia on a regular basis for that treatment.  
22 And I would have gone to California. Oh, yeah, I

1 would have gone anywhere. It would not have stopped  
2 us, and fortunately Philadelphia is a three-hour trip,  
3 you know. And I look at that, to me there is nothing  
4 I would stop doing or say, oh, that's just too far or  
5 too much. I wouldn't.

6 MS. CHITTOORAN: Thank you. And the panel, I  
7 see, questions?

8 DR. MCCUNE: So, it sounds to me like the  
9 most critical thing for you at the time was really the  
10 itching. For the clinical trial, do you know what the  
11 endpoints are for the clinical trial?

12 MR. BUSBY: I do not. I do know what they  
13 are; I cannot recall right at this point. And I do  
14 know that without going into too much detail, I see  
15 that there's a lot of secondary endpoints that have  
16 been met, but not the primary.

17 DR. MCCUNE: And I wasn't -- was itching a  
18 primary endpoint? I guess because we see a lot of  
19 endpoints that -- and itching, we hear a lot about  
20 this, but not necessarily that it's a primary  
21 endpoint. That's all I was getting at.

22 MR. BUSBY: Yes. The itching for this

1 specific one was the primary endpoint.

2 DR. SILVERSTEIN: I wanted to follow up on  
3 Dr. McCune's comment, and I think it's really  
4 important. This is more of a statement as opposed to  
5 a question. But I get from all three of you who are  
6 up here and those who were here before that you're  
7 very, very intelligent, well-informed persons. But  
8 not everybody out there in the world is as informative  
9 as I think many of you. You may be more of a selected  
10 group. If you come to this meeting you're very  
11 engaged in all the aspects of your children's care and  
12 the care of other children who have similar diseases.  
13 But I think it's important when you're considering  
14 putting your child in a clinical trial, is to consider  
15 exactly what Dr. McCune is saying, what are the  
16 endpoints? So, basically, what are they trying to  
17 show? What are they trying to assess as an outcome of  
18 the study? And so if your child, as in your child's  
19 situation, the itching was the most problematic part  
20 of her disease at that time, you would want to find  
21 something where they're addressing that particular  
22 outcome. That doesn't mean that they don't -- if the

1 outcome of that endpoint is not achieved in the trial  
2 that your child may not benefit from that, because the  
3 outcome is a general result. But it's important to  
4 know when you look on clinicaltrials.gov, and I think  
5 also when you talk to your child's physician is, what  
6 problems are being addressed in the trial, and is that  
7 appropriate for your child's problem? Because disease  
8 may have various symptoms and it depends what you're  
9 trying to achieve with that trial.

10 So, being in a clinical trial is only useful  
11 if it is appropriate for your child. We talked about  
12 the preferences of the family and of the child  
13 especially, if the child is the one with the disease,  
14 and to try to match it together. Because there's a  
15 desperation to try and get yourself or a child into a  
16 clinical trial, which I can only understand through  
17 patients I've had and friends I've had who have had  
18 children who have diseases. But at the same time you  
19 want to make sure it matches well so that you're  
20 giving your child the best chance to have the symptom  
21 relieved that needs to be relieved. And is that how  
22 you guys approached it, you all approached it? I'm



1     sorry, I'm from New York; we say guys. Is that how  
2     you all approached it? How did you figure out which  
3     trial? What got you to that point where you can say  
4     this is the right trial for my child, or --

5             MS. RASKIN: So, with congenital  
6     hyperinsulinism, we deal with a lot of the issues that  
7     are common to many rare diseases, but for us the main  
8     thing is to be able to live in the world without being  
9     hooked up to sugar 24 hours a day. So, the main --  
10    the main thinking that goes around that, yeah, it's  
11    definitely considering the endpoint, which can be a  
12    variety of different things to get you to that goal,  
13    which is to live in, really, the least restrictive  
14    environment, hopefully home, and to have a life that  
15    is not tethered to being connected to a pump that  
16    gives you sugar. Also, to preserve your organs, you  
17    know, to keep your -- to keep your pancreas so that  
18    you can lead as normal a life and not develop another  
19    disease. So, that's, you know, kind of dicey as an  
20    endpoint, but it's a goal. So, it's really looking at  
21    the very central issue. That's where we are, yeah.

22             MS. CHITTOORAN: Thank you.

1           MR. BUSBY: And just to add to what Julie had  
2 said there, for PFIC2 patients, pruritus is an all-day  
3 event, and so failure to thrive, you know, the open  
4 wounds become an issue. So, when we were considering  
5 trials, fortunately/unfortunately, depending on how  
6 you look at it, there was only one. So, fortunately,  
7 it was specific to pruritus, and if you can take away  
8 the major symptom, right, then you can prolong the  
9 disease until there is a better, you know, genetic  
10 advancement.

11           MS. CHITTOORAN: Thank you. Okay, let's move  
12 to Monica. You've been waiting patiently, so thank  
13 you. So, you started your own registry. So, can you  
14 tell us a little bit about that and sort of why --  
15 like, how and why you got involved with that?

16           MS. WELDON: Well, I started out, I'll give  
17 you a brief synopsis of my son's story. It kind of  
18 was similar to yours. And you notice when they're  
19 young infants and they're not progressing the way they  
20 need, and we knew that Beckett was not progressing the  
21 way he should at four months -- not sitting up in  
22 comparison to his twin sister Piper, and noticed

1 different things along with him. And, of course,  
2 after having five children, I knew that there was  
3 something wrong with him not being able to walk within  
4 the normal range and meet his milestones.

5           So, we had -- you know, fast-forward into a  
6 year later, where I thought he was having seizures  
7 and, of course, went through -- I think we went  
8 through about 19 different specialists -- four  
9 neurologists, two pediatricians, geneticist, psychs,  
10 you name it, we've seen, I think, everyone, everybody  
11 at Texas Children's Hospital knows me, I think, now.

12           But fast-forward, we finally ended up getting  
13 the genetics and he was diagnosed at the age of 4.  
14 And so when we had gone to the genetics doctor and he  
15 handed me one paper that had been written on this --  
16 you know, written about this disorder, SYNGAP1, saying  
17 it just caused intellectual disability. We didn't  
18 even know at the time it was an epilepsy gene, or a  
19 gene that caused a multitude of different types of  
20 epilepsy. And so I knew then that -- I remember  
21 looking at my geneticist and said, "Is this it?" And  
22 he goes, "That's all we have." And I said, "Well, are

1 there any others?" And he said, "Well, we only know  
2 of about five others in literature, and he's No. 6."  
3 And I said, "Oh, wow, okay," just kind of sitting  
4 there in shock and feeling probably the most alone  
5 I've ever felt. Drove home. I think it took me about  
6 two days to process and realized I couldn't live -- I  
7 could not allow my child to continue living the  
8 quality of life he was living with the sensory  
9 processing -- we didn't know at the time he was having  
10 seizures with the behavior problems and things like  
11 that. I had to do something.

12 And so I started the organization and we  
13 found, of course, a group of people in between by the  
14 time I started on Facebook, started out with three  
15 families on Facebook. And that grew into the  
16 organization, and within the first year, after getting  
17 involved with the rare diseases community, I realized  
18 everything is revolving around data, and I had to  
19 educate myself on the drug development process. And  
20 so I took a shot at writing my very first grant. I  
21 taught school for 23 years and my background was  
22 science, so I knew there was something there. Ended

1 up winning one of the FDA pilot program registries  
2 through the National Organization of Rare Disorders,  
3 which has been up and running for two and a half years  
4 now, and it was a lifesaver for our organization.  
5 Launched at Christmas Day. That as the best Christmas  
6 present anybody could ever have, because with that  
7 registry we ended up finding trends. We found that  
8 SYNGAP1 is the gene, that mechanism that controls  
9 sensory processing in our patient community, possibly  
10 overlapping into other autism communities as well.  
11 And I just realized that watching my own son and the  
12 symptoms that he had at home, and reading through the  
13 trends, of course, on our social media, that we needed  
14 to start asking these questions under an IRB-approved,  
15 organized way of collecting this data.

16 And that brought us to our new finding with  
17 the scientist who used our data to find our very first  
18 biomarker that was just published on Friday this last  
19 week. And it has just been phenomenal, and those  
20 types of things has motivated our community, those  
21 results, to showing them how the data and how these --  
22 and we're not in a clinical trial right now. I'm

1 shooting for that. I'm going to get there with our  
2 group, I'm bound and determined. But on a side, the  
3 clinical studies that we are involved in, that we have  
4 set up with our researchers, and gathering all those  
5 scientists together, basically pleading and begging.  
6 I think I begged Dr. Jimmy Holder, which I love him to  
7 death. He's at Texas Children's, and he goes, "Okay,  
8 I'll study." No, he was excited to come and study  
9 with us, but he was our first clinician ever to study  
10 SYNGAP1. And being a part -- involving these  
11 scientists and these clinician researchers in on our  
12 disorder changed the game for us. And so now we're in  
13 these clinical studies to help find more biomarkers  
14 and more clinical endpoints, because you made a really  
15 good point that I didn't think about is, what is going  
16 to be primary for us? And we're only going to get  
17 that from the patient community, which we have to  
18 prioritize. What symptom do we want to treat? And,  
19 of course, seizures is probably our primary. But then  
20 we have behavior, then we have sleep, then we have all  
21 these different things. And how are we going to  
22 incorporate and measure those endpoints and get those

1 biomarkers? And the only way to do it is through data  
2 and through our registry. And I hope I didn't talk  
3 too long and I hope I answered your question.

4 MS. CHITTOORAN: No, you did. Are there any  
5 other factors -- you mentioned you are working on  
6 trying to get the clinical trials started. So, are  
7 there other factors that you are considering when  
8 doing so?

9 MS. WELDON: Well, I think right now, the  
10 biggest thing that -- time is everything, right? And  
11 we have to accelerate, and we have to accelerate in a  
12 fashion that makes sense and that's strategic. And,  
13 of course, educating your community is one on the drug  
14 development process. I think the biggest thing for us  
15 is we're spread out everywhere. We're all over the  
16 world. I think that organizations like our  
17 organization and the other patient community  
18 organizations that are out there, I think it's  
19 important to help your families eliminate some of the  
20 challenges, like travel, and offering -- you know,  
21 we're going to start raising money for travel  
22 stipends. We've already provided some for some of our

1 European families to get to clinical study in Europe.  
2 And I truly believe that all that tied together, and  
3 it's not just about policy, driving policy and  
4 legislation. It's not just about advocacy and spread  
5 awareness, but you also -- in parallel with all of  
6 that, you've got all these gears that you need to  
7 focus on, and I think that as an organization, you  
8 need to try to help create protocols, like with our  
9 gait study that we're doing at Texas Children's right  
10 now. All of those are to look for these endpoints,  
11 and we want as many families to be involved with those  
12 pilot programs as possible so that we know exactly how  
13 to design this clinical trial. Because, like most of  
14 you know, 95% of all clinical trials fail. Why? Why?  
15 Because, well, participation, retaining your patients,  
16 but also some of the clinical designs suck. I mean, I  
17 don't mean to be blunt, but, you know, I'm from Texas,  
18 I'm blunt, so I just speak my mind. And that's  
19 because the patient voice wasn't included on that.  
20 And I believe that real world data, along with the  
21 clinical data and all of the -- all of that has to tie  
22 in together, and if you're not designing a trial to



1 make sure it is waterproof, you're setting yourself up  
2 for failure. And I'd rather have a 50/50 chance than  
3 a 5% of it succeeding.

4           And so I think all of these things, it's  
5 going to be a complicated mess trying to get through  
6 all those challenges. I personally believe that if  
7 you strategically plan it out right, it can be done,  
8 and I think that we can change that number of 95%  
9 failure right to at least a 50/50. And call me, like,  
10 out of the world like crazy, but I think it can be  
11 done, but I think people have to be onboard to do it,  
12 and I think educating your patient population is  
13 critical in that. Because I would, I'd fly. I would  
14 move hell, high water, snow, sleet, wherever. Like I  
15 said, if I can't get it done and I die, somebody's  
16 going to get haunted until it does get done, because I  
17 would do anything for -- and all of you would -- for  
18 yourself and for the love of your children. You don't  
19 want to see them suffer. You don't want to see your  
20 loved ones suffer. And I think that is the  
21 motivation; we just have to help remove some of those  
22 -- some of the most obvious barriers, like travel.

1 And then also educating on side effects and -- because  
2 it is scary. You know, I pray we have gene therapy,  
3 but then I'm scared to death that day comes and I'm  
4 sitting there with my child in a chair ready to take  
5 an injection, or go through a surgery, where the only  
6 way it can be administered is through intracranial,  
7 you know, gene therapy, and then it maybe not working.

8 So, I think we have to put realistic  
9 expectations out there, because I know the  
10 desperation. These families are desperate. But also  
11 you have to not necessarily take the emotion out of  
12 it, but just be a little bit more realistic about  
13 educating them on these things, because they're lost.  
14 They don't understand, and that's one of my goals  
15 personally as an organization leader is to try to  
16 educate these families on why it's important to take  
17 every little bitty step, because you can't eat an  
18 elephant all at once; you've got to eat an elephant a  
19 bite at a time and you've got to do it right.

20 MS. CHITTOORAN: Well, thank you very much.  
21 So, before I move into the audience and expand this  
22 conversation to you all, I just want to check. Does

1 anybody on the FDA table have any questions before we  
2 move on?

3 MS. WITTEN: I have a question.

4 MS. CHITTOORAN: Sure.

5 MS. WITTEN: Thank you so much for this  
6 story. It's just -- it definitely was you and your  
7 journey. I have a question. You started, your child  
8 was diagnosed with this disease, well, just  
9 practically you were No. 4, right? Oh, No. 6, and  
10 it's a very difficult diagnosis to make. How did you  
11 put this community together? How did you find these  
12 people? And do they have the same mutation, or they  
13 have the same symptoms?

14 MS. WELDON: Well, it all started, I guess,  
15 if you've ever been to Houston, just like in DC,  
16 traffic is horrible. So, I'm stuck in traffic on my  
17 way home and I realized I was alone in the world, but  
18 I knew, I knew that he wasn't the only one. There had  
19 to be more. I mean, you've got 8 billion people on  
20 this planet; what are the chances of him being the  
21 only one? And I've heard of more even ultra-rare  
22 disorders, but I had to -- how I processed is I

1 started to blog. And I started to actually track my  
2 own natural history of my son's symptoms and his and  
3 own lives dealing with this. And I started putting,  
4 you know, the hashtag thing was the -- I don't know  
5 what people did before social media and hashtags. But  
6 that's how, you know, finding -- actually, I didn't go  
7 out finding anyone; they found me. They put in, I  
8 guess, in search SYNGAP1, and then all of a sudden, I  
9 guess six months later, after I was blogging and just  
10 praying that something had to change, she reached out.  
11 And I think we stayed on the phone until 2:00 or 3:00  
12 -- it was 3 a.m., I know, Eastern. It was 2 a.m.  
13 Central, but we stayed on the phone for about two or  
14 three hours talking about our kids, and we were, like,  
15 I think I've found my child's you know,  
16 boyfriend/girlfriend, because, you know, she was a  
17 little girl and my son was, you know, my son. And  
18 we're like, "We're not alone anymore." "Oh, you don't  
19 sleep, either?" "Yeah, okay." You know, it was  
20 crazy. And she goes, "Well, you know I know of one  
21 other person," and I was like, "Can you contact her?"  
22 And then we said, okay, well, let's do the Facebook

1 group thing. Well, there's only three of us. I said,  
2 "Well, let's do it anyway."

3           And then I started a page an open page, and I  
4 started researching all of the papers I could find. I  
5 also found a lot of mouse model data, but no human  
6 data. But I posted it anyway, because then people  
7 started going, oh, well, you know, and it snowballed.  
8 And so not all of the -- we have two -- I know that  
9 there are right now in one of the databases here in  
10 the United States -- I can't remember all of the  
11 acronyms, but it's -- I think we have 262 different  
12 SYNGAP1 variants. I know that we currently have about  
13 209 registrants in our database. I know that we have  
14 close to about 350 families within our Facebook group.  
15 Not all of them participate in the research, and I'm  
16 trying to coerce them to do that because we're such a  
17 small population. And that's when I just took a leap  
18 of faith and quit my job teaching and went into this  
19 full time and started just telling my story. Anybody  
20 who would listen. And the people out in the audience  
21 who know me, know I never shut up. You can probably  
22 tell right now.

1 MS. CHITTOORAN: Thank you very much to our  
2 panel. So, I want to hear from those of you in the  
3 audience who are patients and caregivers, and maybe  
4 hear from somebody who didn't get to speak last time.  
5 I see one hand here, if you would like to start. So,  
6 just curious, just in general, just before we get to  
7 you, just a show of hands who has participated in a  
8 clinical trial before, clinical study? Okay. Okay,  
9 so let's start with you, if you wouldn't mind sharing  
10 your experience and what sort of factors you considers  
11 in deciding to do so?

12 MR. LACEY: Sure. My name is Patrick Lacey.  
13 I'm with Beat Nb, a nonprofit in Boston. My son was  
14 diagnosed with neuroblastoma -- it's a pediatric  
15 cancer -- as a child, and he enrolled in probably nine  
16 or 10 different clinical trials, Phase 1s and Phase  
17 2s. I founded the nonprofit and we've funded probably  
18 18 clinical trials at this point. And what I've  
19 discovered first as a father searching for clinical  
20 trial options, hoping to save my child, I discovered a  
21 lot of things about study design that I would change  
22 if I had the power to do so. And one of those is the

1 patient voice, as many people have mentioned.

2           Some of these trials are designed in a  
3 perfect way that isn't necessarily what patients would  
4 want to enroll on. It doesn't necessarily take into  
5 account, if you're looking at the preclinical data or  
6 the Phase 1 or Phase 2 adult data, safety data, a  
7 Phase 1 dose escalation 3 by 3 design study starts at  
8 a much lower dose than was effective in adults. So,  
9 why am I going to put my kid in that first cohort? It  
10 makes no sense -- single agent, low-dose drug. So,  
11 those types of studies.

12           The idea of randomizing versus maybe using  
13 historical control in the disease is fairly well  
14 understood. That's something that's very strong to me  
15 in a belief that maybe randomizing isn't the best  
16 choice.

17           Looking at travel considerations, cost. You  
18 know, everyone in this room is here for a reason --  
19 they're passionate, they have the desire to help, and  
20 we have the ability. And as you alluded to earlier,  
21 not everyone has that same capacity to either attend  
22 these meetings or to search for those clinical trial

1 options. So, having clinical trial options that are  
2 available close to home, that travel and losing a job  
3 and doing all these other commitments makes it  
4 challenging for families to access clinical trials.  
5 So, having them available in regional locations is  
6 really important as well so that families, if they  
7 want to make that choice and they think it's a good  
8 study, they're not precluded from doing so because  
9 they have to travel halfway across the country and  
10 make sacrifices that would be impossible for some  
11 families to do.

12 So, those are the things that I've seen in  
13 terms of clinical trial and decision-making for our  
14 own choices that kind of influenced the clinical  
15 trials that we enrolled my son on, and also our  
16 involvement in a research consortium, and having a  
17 voice from the patient side on how those studies are  
18 designed. Because at the end of the day, for me, I  
19 think enrollment tells the story.

20 When you see a clinical trial consortium  
21 that's enrolling patients, patients are traveling from  
22 other countries and all over the globe to get on those



1 studies, that's because of great trial design. And  
2 that's because of trials that are putting the patient  
3 and their outcome, both of those things, and the  
4 questions that are asked in the study, those all-  
5 important considerations, and melding all those  
6 together in such a manner that you address all of  
7 those needs, is something that I find to be critically  
8 important going forward in helping all these patients.

9 And before I give the microphone up, I just  
10 want to thank you to the panel and the earlier panel  
11 for sharing your stories.

12 MS. CHITTOORAN: Thank you. Anyone else? I  
13 see a hand here.

14 MS. SHELTON: Thank you. So, my name is  
15 Deborah Shelton, and I'm here on behalf of the ACPMP  
16 Research Foundation, which is a research foundation  
17 for patients and their caregivers with appendix cancer  
18 and pseudomyxoma peritonei, which is a very rare and  
19 lethal form of cancer, effects about one to two people  
20 per million.

21 So, thank you very much. This meeting has  
22 been just really informative for me. I'm new to the

1 patient advocacy world. My spouse was diagnosed with  
2 ACPMP about six months ago. I'm an FDA regulatory  
3 lawyer by day, by training, and so now I'm trying to  
4 kind of use my skills to do some patient advocacy  
5 work.

6 This question kind of channeling our patient  
7 constituents that I work with. We have a Facebook  
8 group of about 3,200 patients, and the first thing  
9 that comes to my mind when I see this question is how  
10 many of our patients would love the luxury of being  
11 able to talk about these factors. Right now,  
12 unfortunately, they don't, and I just kind of have a  
13 quick list that I was brainstorming on, kind of  
14 channeling patients and caregivers that I talk to on a  
15 regular basis, what would they say? What are the  
16 reasons for that?

17 And so one of the reasons is eligibility  
18 criteria, which I've heard a lot of discussion about,  
19 is very, very narrow. And when we're talking about  
20 randomized clinical trials, that has its own set of  
21 issues. Some of the eligibility criteria, just for  
22 example, a big one is to do with the dosage form. So,

1 a lot of our patients have small bowel obstruction as  
2 a result of the appendix cancer, which essentially  
3 produces mucinous tumors throughout the abdomen and  
4 just compresses the digestive system, compresses the  
5 lungs. But with a small bowel obstruction, so many of  
6 these trials are for immunotherapies and are capsules,  
7 and so that's an exclusion criteria. Even if the  
8 patient wanted to, could not participate because they  
9 just can't swallow and have bioavailability issues.

10 A huge issue there with eligibility criteria  
11 is that for appendix cancer, the trials, most of these  
12 are not focused on appendix cancer specifically, but  
13 rather the tumor agnostic clinical trials with all the  
14 fantastic immunotherapies coming down the pike. The  
15 problem there is you have to have genomic sequencing,  
16 and we've got patients who are having real issues  
17 getting insurance to cover that genomic sequencing,  
18 which is quite, quite expensive. And often the  
19 clinical trials are designed that you have to come in  
20 the door with those sequencing results in hand. So,  
21 that's a real problem.

22 Very quickly, just a couple of the other big

1 problems. The distance, the frequency. Would love to  
2 have more remote monitoring, more regional. You know,  
3 I have to tell you that when my spouse was diagnosed,  
4 and I knew it at the time, but, boy, do I know it now  
5 more than ever, we are so privileged that we could go  
6 and search and talk to specialists all across the  
7 country and pick and choose who we wanted to treat my  
8 spouse. But the reality of it is, most patients do  
9 not have that luxury. Boy, they have the passion.  
10 They'd travel to the ends of the earth for their loved  
11 ones, but they can't. They have to work, they have  
12 financial constraints. I'm working with a Medicaid  
13 patient now whose daughter is dying. Probably within  
14 the next couple of months she'll be gone if she can't  
15 get into a clinical trial. But she's on Medicaid, and  
16 so she's really bootstrapped and it's heartbreaking.

17 MS. CHITTOORAN: Thank you.

18 MS. SHELTON: It gets me emotional. Just  
19 last but not least, the real shocker of the reason for  
20 why our patients are having problems with clinical  
21 trial is they have no knowledge of them. Their  
22 doctors are not talking to them about them unless

1 they're clinical trials at their own institution, and  
2 even then it's a longshot. And, you know, I sat in  
3 with a patient and they were told, "Just go to  
4 clinicaltrials.gov." I'm a lawyer. I helped with the  
5 legislation that created clinicaltrials.gov. It's  
6 difficult for me to navigate, and so to tell some of  
7 these patients, just go do that, especially when  
8 you're searching for biomarkers and whatnot, it's just  
9 a real challenge. Thanks for listening.

10 MS. CHITTOORAN: Thank you. Thank you.  
11 Wendy, I just want to go to you. Are there folks on  
12 the web that have participated in clinical trials, and  
13 what factors were they considering when deciding to do  
14 so?

15 MS. SLAVIT: Yeah, so we've actually heard a  
16 lot of the same things that people have been talking  
17 about in the room. The difficulty of the time  
18 commitment, that it can be disruptive. There are also  
19 concerns about the treatment being conflicting with  
20 what the treatment regimen they are currently on.  
21 Also not knowing maybe what some of the side effects  
22 of some of the medications will be. A few people are

1 concerned about being on the placebo and not getting  
2 the drug that's being tested. As a lot of people have  
3 mentioned, the location of the trial, and so just the  
4 cost of getting there. People have talked about the  
5 endpoints and really looking at the endpoints as a  
6 decision of whether they're going to participate or  
7 not.

8           Someone talked about remote support, which  
9 was just actually mentioned, so if you're involved in  
10 a trial that's not in your area, when you're not  
11 actually there for the trial and you're back home,  
12 being able to access information that you need for the  
13 trial.

14           People have also talked about wanting to see  
15 the data kind of in real time whenever they can. They  
16 also want to see an impact. And a few people also  
17 talked about gene therapy trials can be invasive, so  
18 that's a barrier to potentially participating.

19           MS. CHITTOORAN: Okay, thank you. So, just a  
20 show of hands. Has anybody here who is a patient or  
21 caregiver wanted to participate in a clinical trial  
22 and wasn't able to? Okay. So, I'd like to hear from

1 a couple folks about that, if you wouldn't mind  
2 sharing. Do we have a mic coming up?

3 MARIE: Hi. Marie again. So, my daughter is  
4 6, she has Prader-Willi syndrome. And I didn't  
5 mention before, I do also have a for-profit company  
6 called TREND Community. But all the clinical trials  
7 that are currently recruiting, the primary endpoint is  
8 hyperphagia, and though I think she might benefit with  
9 regards to some of the secondary endpoints, she's not  
10 in hyperphagia, so she doesn't qualify for any of the  
11 clinical trials.

12 MS. CHITTOORAN: Okay. I think the gentleman  
13 across -- do you want to share?

14 MR. HARTMAN: Hi. Eric Hartman again with  
15 the Choroideremia Research Foundation. I was in a  
16 natural history study for the selection for a Phase 3  
17 for my eye disease, and the surgery involves a  
18 subretinal injection and you need to have a certain  
19 amount of elasticity in the retina to take the  
20 injection of the vector. And both the original  
21 principal investigator from England and the surgeon  
22 out in at the other institution here in the United

1 States, they both said they would do it, but they had  
2 serious concerns. And would I be willing to risk my  
3 one degree of central vision? I mean, I see you, I  
4 don't see you guys. But to risk what little I have  
5 became incredibly difficult for me, especially knowing  
6 there's a potential intravitreal delivery that may be  
7 in the offing. So, I chose not to risk it because of  
8 that, but I would have traveled anywhere for it.

9 MS. CHITTOORAN: Thank you. Anyone else? I  
10 saw a couple other hands. Hi. Go ahead.

11 LENNIE WOODS: Hi. My name is Lennie Woods.  
12 I'm with Sarascure.org. We are a patient advocacy  
13 group, but I'm also the mother of Sarah, and I feel  
14 like I've got a sister in Monica, because we were told  
15 my daughter was the only living one with clear cell  
16 sarcoma at the time. An interesting fact, there was  
17 another patient in the same hospital being seen by  
18 another doctor with a different pathologist. He found  
19 us, God bless social media and Facebook. And when we  
20 -- we didn't confront, but when we brought it to the  
21 attention of the hospital, Memorial Sloan Kettering,  
22 there were some very upset people. But there were



1 papers being written on him and studies being done on  
2 my daughter, and no one was communicating. And we've  
3 since -- I mean, that's hurtful. We've since found 25  
4 patients living and about 10 years of natural history  
5 data on a Facebook page, yes. It was a secret page,  
6 so it wasn't infiltrated by others, and I want to talk  
7 to you.

8           But we have problems with trials in that  
9 we're considered soft tissue sarcoma, and clear cell  
10 is extremely different. We're almost hurt by being  
11 called that. So, they want to throw us into trials  
12 for soft tissue sarcomas and they're never successful.  
13 And on papers, when you read papers and you try to out  
14 clear cell sarcoma patients, they're thrown in with  
15 other soft tissue sarcomas. And this is also a  
16 problem. We support other small groups, like  
17 epithelioid sarcoma, who are parents doing the same  
18 thing we are. So, there's a whole bunch of us out  
19 here with a lot of good information. But I will tell  
20 you, the people from the FDA should know, when we try  
21 to share information and talk to our doctors, we get  
22 the eye-roll, we get -- I mean, we are shut down. I

1 call them the gatekeepers. They will not let us  
2 patients get beyond to make the change. So, I say I  
3 had to jump over them, but thank you.

4 MS. CHITTOORAN: Okay. So, I know we are  
5 very short on time here, but, Wendy, do you have  
6 anything from the web that you'd like to share with  
7 the group here in the room?

8 MS. SLAVIT: Nothing additional at this point.

9 MS. CHITTOORAN: Okay. Does our FDA table  
10 have any other questions for anybody in the audience  
11 at all? Sure.

12 DR. SILVERSTEIN: Yeah, I'm sorry to hear  
13 about your experience. As a physician, that's  
14 disappointing, obviously, but it's disappointing for  
15 all of us. Just the one comment, and the question, I  
16 think, focused on two different, very different  
17 aspects of getting patient data, clinical study and  
18 registry, and I think it's appropriate to put it in  
19 the same question. But I would also say that registry  
20 data is what we consider real world evidence, or real  
21 world data. Five years ago I don't think we put much  
22 stock into the value of registry data. I think

1 nowadays at the FDA, especially for children, where  
2 data is very, very hard to generate under the best of  
3 circumstances, we really do take seriously registered  
4 data. As a reviewer myself, on several applications  
5 I've accepted registry data as the proof either as a  
6 control group or whatever. So, I would encourage  
7 everybody, especially those that are here and on the  
8 phone, to consider entering your child or your own  
9 data into registries. And if you need to, I'm sure  
10 Monica would be able to show you how to basically just  
11 Google "registry" and put in -- and I'll bet you'll  
12 find somebody who will get you some information about  
13 how to do it. Because that data, even if it's just as  
14 a control group, can be very, very useful to help  
15 assess whether an intervention, a device or a drug is  
16 beneficial.

17 So, I'm just curious, how many of you here  
18 either entered your own or your child's data into a  
19 registry over the last 10 years? That's great. For  
20 those who didn't raise your hand, try every way you  
21 possibly can to connect with people on social media.  
22 I'm not a social media person myself, but connect with

1 people. I'll bet if I had a situation like yours and  
2 I needed to, I would become a social media person  
3 tomorrow. But try to use that, because it's extremely  
4 important. And I'm speaking from the device world --  
5 we do look at registry data very differently than we  
6 did a few years ago. We see the value in it,  
7 especially for rare diseases, which includes a lot of  
8 pediatric diseases. So, it's a small pitch, but I'm  
9 glad a lot of you are doing it.

10 MS. CHITTOORAN: Thank you so much. And I  
11 just want to thank our panel for sharing so much, such  
12 personal experiences and personal stories, and to the  
13 audience as well. Thank you so much for doing so, and  
14 the folks on the web. Recognize it's not always easy  
15 to talk about things that hit so close to our heart.  
16 But thank you so much for that, and if you didn't get  
17 to finish your thoughts or share, we, again, I know I  
18 sound like a broken record, but I encourage you to  
19 please submit those comments to the docket so we can  
20 capture your perspective that way.

21 So, I just want to turn over to Andrea, who  
22 is going to be moving to the next portion of the

1 meeting, which is the open public hearing. Thank you  
2 so much.

3 MS. FURIA-HELMS: Okay. Thank you all so  
4 much. I think it is so important to hear your stories  
5 and experiences, and I think it's been a really,  
6 really helpful meeting today, to hear those  
7 situations. We are now going into the open public  
8 comment portion of the meeting. So, today we have  
9 registered speakers, and each of them will have two  
10 minutes to speak. If a speaker finishes early, we  
11 intend to move on to the next speaker. We will call  
12 each speaker by name. When it is your turn and if you  
13 are able, please approach the microphone at the middle  
14 of the room for your comments, right here at the front  
15 in the middle aisle. Otherwise, raise your hand when  
16 your name is called and someone will bring the hand-  
17 held microphone to you for your comments.

18 For transparency purposes, again, we ask,  
19 please disclose if you are affiliated with an  
20 organization or if your travel has been funded, or if  
21 you have significant financial interest in rare  
22 disease medical product development. As you are

1 speaking, you will notice that there will be a timer  
2 and lights to guide you. The light will indicate you  
3 can begin speaking, when it is green. It will turn  
4 yellow when you have 30 seconds left in your time, and  
5 the timer will turn red when your time has come to an  
6 end. If you have not concluded your remarks at the  
7 end of the allotted time, I will ask you to do so,  
8 gently. As a reminder, you also have the option to  
9 submit comments to the docket, which will remain open  
10 until May 30th. You can find additional information  
11 about this in the federal registered notice. So, with  
12 that, let's get started with the first speaker. And  
13 I'm calling up the first speaker, who is Kristin Moro  
14 (ph).

15 MS. MORO: Hi. I'm very happy to be here  
16 today. My name is Kristin Moro, and our daughter Anna  
17 is 13 years old and was diagnosed with Friedreich's  
18 ataxia, the same disorder as Caroline on panel 1. She  
19 -- I'm going to speak today about her participation in  
20 clinical trials and how it's affected our family.

21 I guess four years ago, when she was  
22 diagnosed at age 9, we were noticing that her symptoms

1 of fatigue were minimal at that point, but upon her  
2 diagnosis realizing that she would lose mobility,  
3 there was risk of speech and vision loss, scoliosis,  
4 cardiomyopathy, potential diabetes. We were very  
5 eager to participate in any clinical trial.

6 On her 10th birthday I was on the phone, and  
7 the only place that was open to her was Iowa or UCLA,  
8 and we were in Baltimore and California sounded more  
9 fun. So, we got on a plan, but we were aware from the  
10 beginning that it would be nine trips within the year.  
11 And I have to say we were taken care of so well there.  
12 Our travel was paid for, hotel stay was paid for, car  
13 while we were there. So -- and we had a great time.  
14 But the biomarkers at the end, you know, she does  
15 experience -- you know, her handwriting has decreased,  
16 so looking at the clinical trials and what the  
17 endpoints were was a concern just with what we -- with  
18 what was the end result and whether that was a good  
19 determination of what was successful.

20 MS. FURIA-HELMS: Thank you so much. Our  
21 next speaker is Daniel Campian.

22 MR. CAMPIAN: Good afternoon. Thank you. My

1 name is Dan Campian. I am an account management  
2 director with IQVIA, a human data science company, and  
3 for the past 10 years I've worked with patient  
4 advocates and medical societies to build patient  
5 registries. The registry collects real world  
6 information about patient symptoms and treatments and  
7 care experience, either directly from patients and/or  
8 with their doctors. My colleagues and I submitted  
9 comments to the FDA a couple weeks ago about this  
10 meeting, and based on those comments I have one plea  
11 and a couple of questions.

12           The plea is for cooperation. It is spelled  
13 out in our recent whitepaper that we submitted,  
14 Registries for Rare Diseases, a foundation for multi-  
15 arm, multi-company trials. By working together, rare  
16 disease stakeholders have an opportunity to create  
17 broad-based registries that share common technology  
18 platform and collect data to address all their needs.  
19 The toughest challenge for these partnerships is not  
20 finding a multi-tenant technology platform, but  
21 getting people to cooperate, to agree on common data  
22 definitions and to use a common data hub rather than



1 setting up separate registries for their natural  
2 history studies, quality improvement projects, post-  
3 market studies, or other projects.

4 The Cystic Fibrosis Foundation and the  
5 Muscular Dystrophy Association are outstanding  
6 examples of nonprofit groups that are successfully  
7 bringing together their communities around these  
8 multi-stakeholder registries.

9 So, in terms of today's discussion, thank you  
10 all for your comments. The two questions to keep  
11 focused on are, if you're going to invest your time to  
12 push -- to submit data for yourself or a loved one to  
13 a registry, what kinds of information or analyses do  
14 you want to get back from those registries in order to  
15 keep feeding that and to encourage others to do so?  
16 And, second, would you be willing to submit  
17 scientifically validated questionnaires regarding your  
18 experience of care? So, those are the two things that  
19 we're thinking about when we're designing registries.  
20 Thank you.

21 MS. FURIA-HELMS: Thank you so much. The  
22 next speaker is Rachel Sher.

1 MS. SHER: Good afternoon. I'm Rachel Sher,  
2 Vice President for Policy and Regulatory Affairs at  
3 NORD, the National Organization for Rare Disorders.  
4 For those of you who don't know, NORD was founded in  
5 1983, and we represent more than 290 individual rare  
6 disease patient groups. We have been focused this  
7 whole time on the identification, treatment and cure  
8 of rare disorders through programs of education,  
9 advocacy, research and patient services. We really  
10 today just want to thank FDA for holding this meeting  
11 and for its leadership on these issues.

12 As many of you know, this meeting is coming  
13 on the heels of several other meetings that FDA has  
14 held in which it's really put the patient voice front  
15 and center in the drug development process, and in  
16 FDA's own thinking about the regulatory process, and  
17 we just fully agree with that approach.

18 We also thank FDA for its continued  
19 flexibility with respect to its oversight of medical  
20 products in the rare disease space, including looking  
21 at alternative sources of data, like registry data,  
22 like we've been talking about.

1           As you may know, NORD has started a registry  
2 program for rare diseases. It's called the IAMRARE,  
3 natural history patient registry program. It is a  
4 very easy to use program for patients and providers  
5 and patient organizations to create quality data.  
6 More information is on NORD's website, the  
7 rarediseases.org, and we encourage you to check it  
8 out. We have several programs already up and running  
9 for various disease sates, and are always happy to  
10 work with more programs.

11           I also want to just give a shout out to the  
12 Patient Affairs staff here at FDA, who has been doing  
13 an incredible job. They made the plea to be in touch  
14 with them and in our experience they've had an open  
15 door and we would just encourage everyone to continue  
16 to work with them closely, too.

17           In short, I won't take the full two minutes,  
18 just want to say we stand ready to continue to be a  
19 partner to FDA and just thank you for holding this  
20 meeting and for your leadership. Thank you.

21           MS. FURIA-HELMS: Thank you so much. And I  
22 apologize if I mess up people's names. So, don't take

1 it personally; I'm doing my best. Next speaker is  
2 Robyn Himick.

3 MS. HIMICK: Hi. My name is Robyn Himick,  
4 and I am here to speak on behalf of the Amyloidosis  
5 Consortium, also known as ARC. ARC is a patient-led  
6 organization with the vision of accelerating the  
7 development of and the access to new treatment through  
8 the collaboration and innovation. For those of you  
9 who may not know, amyloidosis is a term of a group of  
10 rare diseases in which the abnormal proteins deposit  
11 its amyloid into tissue and organs. It is progressive  
12 and fatal disease with currently no approved cure.  
13 Amyloidosis can develop as part of a genetic mutation  
14 that's passed on within families, or can develop  
15 during a person's lifetime for unknown reasons.

16 The diagnosis of amyloidosis is often delayed  
17 because the symptoms are so varied and the delays in  
18 diagnosis are uncommon. It can also be very  
19 challenging to find a specialist with the appropriate  
20 expertise, and seldomly are these conveniently  
21 located, which only adds to the additional stress and  
22 burden to the patients and their families.

1           In the past several months, the landscape of  
2 amyloidosis treatments have dramatically changed, and  
3 for the first time we've seen two new approved  
4 treatments for ATTR amyloidosis. While these  
5 treatments were recently approved, the struggle for  
6 accessibility and affordability has proven to be just  
7 as problematic, particularly for patients and families  
8 affected by the hereditary nature of the disease,  
9 since multiple members of a single family can be  
10 affected with the same disease complications and  
11 financial burdens.

12           Considering the new available treatments, ARC  
13 recently developed an online survey designed to obtain  
14 the perspective from patients and caregivers to  
15 understand the burden of the disease, the impact of  
16 quality of life, and the treatment perception. From  
17 the survey we learned that patients diagnosed more  
18 than five years ago struggled with the most burdensome  
19 symptoms of numbness and pain, dizziness and fatigue.  
20 These symptoms only left patients unable to engage in  
21 the basic activities of their daily lives with  
22 significantly impaired independence.

1           Amyloidosis, like many rare diseases, causes  
2 a high burden on patients and families, impacting all  
3 aspects of their life. In our surveys, patients  
4 reported that the greatest impact of the disease was in  
5 their work and professional life and financial  
6 wellbeing, whereas, caregivers reported their  
7 emotional wellbeing and relationships were most  
8 greatly impacted. The complex nature of amyloidosis  
9 coupled with the limited access to treatment and  
10 services means that caregivers are often the primary  
11 source of support and care for their loved ones, often  
12 leaving them to balance all of life's priorities and  
13 manage the wellbeing of their entire family. Thank  
14 you.

15           MS. FURIA-HELMS: Thank you very much. The  
16 next speaker is Jill Cisco.

17           MS. CISCO: Hi there. Jill Cisco with  
18 Acromegaly Community. I wanted to discuss just for a  
19 couple moments, you know, we have an online community,  
20 and although it sounds like some of the things that  
21 happen online, that they're open. We have a closed  
22 group that we interview every single person before we

1 allow them in. We only allow medically approved  
2 documents to be posted. They have to be from  
3 accredited sites. You know, we try to educate our  
4 patients. Our last conference that we did, we held in  
5 concession with the Pituitary Society, because we try  
6 to put the correct information out to our patients.

7           One of the biggest complaints that we see  
8 worldwide with our patients is the fact of symptom  
9 control. And there's a couple clinical trials that  
10 are going on right now, and the main complaint that I  
11 hear from the patients is I've worked so hard to get  
12 my numbers within the normal range; I don't even want  
13 to take the chance of being a placebo patient. And I  
14 hope that you all will understand that. When you have  
15 a disease that dramatically affects your quality of  
16 life, in every aspect of your life, you don't want to  
17 have to take the chance of taking a placebo. And I  
18 think that is a huge dramatic thought process. It's  
19 not the travel, it's not anything else; it's the  
20 placebo that stops patients from going into these  
21 clinical trials.

22           MS. FURIA-HELMS: Thank you very much. The

1 next speaker is Christina Hartman.

2 MS. HARTMAN: Hi. My name is Christina  
3 Hartman and I'm with the EveryLife Foundation for Rare  
4 Diseases. I have been in Washington for the past 20  
5 years. I was hired to run policy and advocacy for the  
6 foundation following their move to Washington DC. I  
7 came to the foundation because my youngest daughter  
8 was recently diagnosed with a rare genetic disorder,  
9 NAA10, also known as Ogden in boys. The boys  
10 typically die in infancy and early childhood. Because  
11 it's an X-linked disorder, the girls live, of course  
12 with lots of health issues. We, too, have a Facebook  
13 group and the parents post their daughters' mutations  
14 on the group. We have a researcher, thankful, many  
15 organizations or disease groups don't, and he is  
16 currently looking for funding from the NIH to run the  
17 phenotypes of these girls. There's less than 100 of  
18 them that we know of in the world. We are currently  
19 seeking money from NIH. He got a good score on his  
20 NIH R35 recently, so fingers crossed.

21 But one of the challenges that I've seen --  
22 you know, I've worked for HHS for the first third of



1 my career in the Office of the Secretary, and there  
2 doesn't seem to be a ton of collaboration between NIH  
3 and FDA. And I would really like to see more of that.  
4 The other challenge that many folks in the rare  
5 disease community have, my daughter's group in  
6 addition, is we don't have any treatments. There are  
7 no clinical trials. Now, my daughter is not dying, so  
8 I'm very thankful for that, but many of the families  
9 and the children that I work with are. So, almost 95%  
10 of the rare disease community does not currently have  
11 an FDA-approved treatment. Some people have nothing;  
12 some are taking conditions -- taking medications off-  
13 label so they don't have the proper dosage, safety or  
14 efficacy information, and they often don't get  
15 insurance coverage for their drug. So, this is a  
16 major issue. The other major issue that we've seen is  
17 the lack of diagnosis, and without diagnosis, of  
18 course, you can have no treatment.

19 So, one of the things that EveryLife is doing  
20 is we are asking Congress for \$1.5 million to do a  
21 burden study to demonstrate the true public health  
22 crisis of rare disease in the United States. And so

1 this would be a comprehensive study that the National  
2 Academy of Medicine would do, and it would look not  
3 only at direct medical costs of rare diseases, but it  
4 would also look at the cost to caregivers and  
5 families, the comprehensive societal burden. And I  
6 hope that that would provide the justification  
7 necessary to put the resources behind this and to  
8 justify the collaboration across the Department of  
9 Health and Human Services, including FDA.

10 MS. FURIA-HELMS: Thank you very much. The  
11 next speaker is Shazia Ahmad.

12 MS. AHMAD: Hi. I'm Shazia Ahmad. I want to  
13 disclose I'm with UBC. UBC is a service provider, and  
14 my role there is providing -- working with sponsors  
15 and patient and stakeholder engagements specifically  
16 in the rare disease area. But I'm more here as a  
17 patient advocate. My daughter was diagnosed with  
18 Kawasaki disease at the age of 3. Thankfully, she  
19 recovered because she got the treatment in time. My  
20 question or really more statement is there really is  
21 more of a need for early education and awareness of  
22 rare diseases, especially in the medical community.

1 We were in a small area in Chattanooga, Tennessee when  
2 she was diagnosed, but we were very lucky because she  
3 was near a teaching hospital. My husband was a  
4 physician at the time doing his training. But,  
5 really, my statement is more what we can do more  
6 professionally, the CLOs, service providers, sponsors  
7 in raising education awareness and working with  
8 patient advocacy groups. Thank you.

9 MS. FURIA-HELMS: Thank you very much. And  
10 our next speaker is James Valentine.

11 MR. VALENTINE: Hi. Good afternoon and thank  
12 you, Andrea, and thank you FDA for putting on this  
13 fabulous meeting, and really tremendous gratitude for  
14 all of our patients and caregivers here today sharing  
15 their experiences and truly being brave. My name is  
16 James Valentine, and I'm an associate at Hyman, Phelps  
17 & McNamara. Prior to joining the firm I worked at  
18 FDA, actually as a patient liaison, and among other  
19 things helped implement the patient-focused drug  
20 development program.

21 In the past several years I've had the  
22 pleasure and opportunity to help plan and moderate 16

1 externally led patient-focused drug development  
2 meetings, four rare disease patient communities. Of  
3 those 16 meetings I was involved with, my colleague,  
4 Larry Bauer, and I, looked at the 11 published Voice  
5 of the Patient documents that summarize the findings  
6 of those meetings, and we want to share some of what  
7 we found for the theme of this meeting today.

8           So, some meetings identified issues common to  
9 most of the diseases, and others were unique to  
10 specific diseases. One of the overarching themes from  
11 all of the patient communities and consistent with  
12 what we have heard here today is the willingness of  
13 patients and caregivers with rare diseases to share  
14 their experiences and provide invaluable input into  
15 how their diseases impact their lives. Patients are  
16 the experts and can articulate what symptoms have the  
17 most impact, what kind of treatments are helping, and  
18 what amount of risk they are willing to tolerate in a  
19 new treatment.

20           All the rare diseases except one that we  
21 looked at shared the commonality affecting multiple  
22 body systems; however, every community was still able

1 to identify one or two symptoms that most patients had  
2 that caused the most severe impact. Every disease has  
3 phenotypic variability with different symptom clusters  
4 and different levels of disease severity, and every  
5 rare disease has unmet medical need, and 8 out of 11  
6 diseases cause premature death.

7 All the patients talked about impact on  
8 activities of daily living, including impact on school  
9 and work; all were progressive; all had mental health  
10 consequences, and all cause fatigue.

11 For future treatments, every community  
12 identified the desire to improve quality of life and  
13 to slow disease progression, and patients say that  
14 they're willing to tolerate some risk as there are  
15 potential life-impacting benefits.

16 What was unique to some meetings was the  
17 specific body symptoms and types of symptoms that were  
18 experienced. Some still had challenges related to  
19 early diagnosis; some communities identified  
20 challenges through the route of administration; and  
21 others stated that they were willing to participate in  
22 research to help others in the future.

1           So, in the near future we plan to provide a  
2 more detailed analysis of our observations from these  
3 16 externally led meetings, including looking to  
4 themes that we heard today -- things like pain, speech  
5 impairment, sleep disorder, caregiver burden and more,  
6 and we will be sure to provide those to the docket.  
7 Thank you.

8           MS. FURIA-HELMS: Thank you very much. We  
9 have two additional slots open, so we are opening it  
10 up to anyone who would like to provide a two-minute  
11 comment at this time.

12           MS. YOUNG: Thank you. My name is Ni Young.  
13 My name is Ni Young. I congratulate FDA at this time,  
14 allow people to speak their own voices, but I have my  
15 own consideration. One is this type of disease, I  
16 need some professional to tell me is it preventable  
17 and how many from here are allowed to speak, but how  
18 many lost their loved ones? I suppose the statistics  
19 should be variable, and this is maybe the result of  
20 many call malpractice, and how are we going to train  
21 the physician to do a better job, or the government to  
22 have a better responsibility to supervise them, in a

1 sense? Otherwise, the people's complaint should be  
2 documented. So, to count on physicians should be  
3 avoided.

4 And also I'm thinking the financial burden is  
5 a big, huge issue, and environmental -- the  
6 environment, people are adversely impacted, so should  
7 we ask the government to do a better job in this  
8 direction? Because currently other people are forced  
9 to be homeless, and if I'm in financial trouble, that  
10 should be very much the number one issue. The  
11 priority of our government is to protect people's life  
12 and protect people's properties, businesses, their  
13 home, their car. Currently, the government is part of  
14 the problem, because they rob people's home and life  
15 and the car and everything. So, we've got to ask FDA  
16 to do a better job, too. Thank you.

17 MS. FURIA-HELMS: Thank you very much. Any  
18 other takers? Okay, we have one more.

19 MR. FELDMAN: Hello. I'm David Feldman at  
20 the National Kidney Foundation. I want to thank FDA  
21 and especially the panelists for this wonderful  
22 meeting. I've had the opportunity to work with James

1 Valentine on two externally led, patient-focused drug  
2 development meetings, and one of the things that I've  
3 struggled with, and I believe that probably everybody  
4 in this room who is trying to improve clinical trial  
5 design has struggled with this. How do you get to the  
6 issue of risk-benefit? What do patients really think  
7 about this? And my problem is what question to ask to  
8 get that information? Because it's a very difficult  
9 issue to probe. So, I would like to suggest that the  
10 FDA have a meeting like this with patients  
11 specifically focused on the question, how do we get  
12 this information? How can we get it and use it better  
13 to design clinical trials. Thank you.

14 MS. FURIA-HELMS: Thank you very much. This  
15 concludes the open public comment period, and we  
16 appreciate and thank everyone for participating today,  
17 not only in the OPC, but also today in the audience,  
18 especially our panel members, the FDA panelists, the  
19 folks online. Thank you so much for participating  
20 today. And I just want to briefly thank the Patient  
21 Affairs staff for all your hard work, along with the  
22 Office of Orphan Products staff that has been really



1 working really hard to get this meeting to be  
2 successful today, that I think we might have done a  
3 good job there. So, thank you all.

4 DR. MAYNARD: Thank you. So, I'm Janet  
5 Maynard. And on behalf of FDA, I'd like to thank all  
6 the speakers and everyone who attended today, both in  
7 the audience and on the web, for your participation.  
8 We greatly appreciate all that has been contributed to  
9 this meeting today.

10 This has been a very important meeting both  
11 for us at FDA, but for all of the stakeholders in drug  
12 development. I think for the patient advocacy groups,  
13 industry, for our research partners, I think we can  
14 all learn from what we have heard today.

15 So, recognizing that we do have differences,  
16 today I think we heard many commonalities, and I think  
17 where the commonalities that resonated most with me  
18 was the importance of using our voice. Also,  
19 something that resonated with me was something that  
20 Julie said about the global insult to the family, and  
21 really the significant impact that each rare disease  
22 has on both the patient and the family.

1           When we talked about some commonalities and  
2 symptoms, some of the commonalities I heard were  
3 related to fatigue, pain, communication impairment,  
4 difficulty with movement, sleep disorders, and  
5 seizures. These were some of the commonalities I  
6 heard. I also heard that life can be unpredictable  
7 and that it can be very difficult to plan when you  
8 have a rare disease and that that adds a lot of  
9 complexity to life in terms of thinking about how to  
10 get things done on a day-to-day basis, and saying true  
11 to the important things that you were trying to  
12 accomplish relating to school or work or family time.

13           Some commonalities I heard in terms of  
14 symptom management, I heard difficulties related to  
15 not having a treatment, when there's no treatment  
16 available at all for a rare disease, but I think we  
17 also heard the important balance that sometimes when  
18 there is a treatment available, that might not  
19 necessarily be the answer, either. That there may be  
20 significant side effects associated with that  
21 treatment that cause difficulties for patients and  
22 families.

1           For clinical trials, I really appreciated  
2 hearing about the complex decisions that these raise  
3 for patients and families. Also about the importance  
4 of data. I'm thinking about how we can use data to  
5 synergize rare disease product development.

6           This was a very informative meeting for all  
7 of us. I think we've heard today that the impact of  
8 rare diseases is enormous. The need for better  
9 products is really huge, also, and we look forward to  
10 incorporating what we have heard today as we continue  
11 to move forward with rare disease product development.

12           I wanted you to know that even though the  
13 meeting is over, that there is still opportunity to  
14 connect with FDA. So, you can see on the screen here  
15 that you can connect either with the Patient Affairs  
16 staff, or you can connect with the Office of Orphan  
17 Products Development, if you have any questions in  
18 follow-up from this meeting.

19           And as you have heard, we really encourage  
20 you to submit comments to the docket, which will  
21 remain open until May 30th. We appreciate all the  
22 feedback that we receive into the docket, especially

1 as follow-up today, as you maybe think about  
2 additional issues or considerations that we didn't  
3 have time to address today.

4 So, I think your voices were definitely heard  
5 today and the need for therapeutic options is very  
6 clear. And we look forward to all working together to  
7 improve medical product development for rare diseases.

8 A few housekeeping items. So, you should  
9 have had on your seat when you came in a survey. We  
10 want to continue to improve our public meetings, so if  
11 you could please complete that survey, we would be  
12 greatly appreciative. If for some reason a survey has  
13 gotten misplaced, no fears; we should have additional  
14 surveys at the registration table. So, if you don't  
15 mind taking some time to fill out the survey to give  
16 us feedback. You can give it back to any of the FDA  
17 staff who has one of the badges, or you can drop it  
18 off at the registration table. And for folks who are  
19 attending via the web, you will be emailed the same  
20 survey that's being completed in the room.

21 A transcript of this meeting should be  
22 available within 30 days. And as I mentioned at the

1 beginning, we will work on a meeting summary document,  
2 but we'll need to incorporate information from the  
3 docket, which will remain open until the end of May.

4 So, on that note, thank you again for  
5 everyone's participation today. We sincerely  
6 appreciate it, and we wish you safe travels. And on  
7 that note, I will close the meeting. Thank you.

8 [Applause.]

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