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you to present. But please don’t keep them. They won’t work in any other type of device. There will be an expense to us if we don’t have them. I do want to welcome you to our third workshop, which is CDER and You: Keys to Effective Engagement. And really our goal in this meeting and workshop is to help you understand a little bit about the FDA and particularly about the Center for Drug Evaluation and Research. We affectionately call this our boot camp, which is how do you get some skills, how do you learn who to talk to, as well as how do you have a better understanding of what we can and cannot discuss. We’re going to talk a little bit about the circumstances under which we cannot disclose as much information as perhaps we would like.

We encourage all of you, whether you’re in the room or online, to ask questions. You can do that via the webcast and interact with our presenters. So we’re not going to interrupt the presenters, but we are allowing sufficient time for you ask questions, and we really encourage that.

The workshop is being recorded for archival purposes, but if for some reason you wanted to watch it again, you’re welcome to. We’ll provide copies of all the slides if you want to have them, and we’ll make them available on line as well as all of our contact information.

We’re going to start off with a welcome by our center director, Dr. Janet Woodcock. As many of you know, she’s the director of the Center for Drug Evaluation and Research here at the FDA. She has led many cross-cutting initiatives. While at the FDA, she introduced the concept of pharmaceutical risk management in 2000. As a new approach to drug safety, she’s led the pharmaceutical quality for the 21st Century Initiative since 2002. Prior to joining CDER, she was the director of Office of Therapeutics Research and Evaluation with CBER, which is the Center for Biologics Evaluation and Research, and during my four and a half years here at the FDA, she really has been the champion of patient engagement, and ever since I came here to FDA have talked about how
There is more transparency to the agency, but I remember when I was meeting with her prior to coming, she said it's almost like a huge ship, and if we can move this ship even a few degrees, we will have made progress. So I'm not sure how many degrees we have moved it, but I know there is some movement as we think about patient engagement.

So it's my pleasure to introduce Dr. Janet Woodcock.

(Applause.)

DR. WOODCOCK: Thanks very much, John, and I do think PASE has helped us with some of those degrees of movement of this ship. So I thank you and your staff because a lot of contributions have been made to get us more outward facing and more engaged with the public. The topic of this workshop today is engagement with FDA CDER, the drug regulators, and how might one do that.

First of all, I could say I understand how daunting a task this might be. Before I came to the FDA, which was long ago, I was a doctor taking care of patients, and I had a need to get one of my critically ill patients a drug called thalidomide. And little did I know what a history that had with the FDA and so forth.

So I attempted to locate the FDA. I called all these different people and everything, and I said I want to get this drug thalidomide, and they mentioned things like the Code of Federal Regulations. I said, "What's that?" I got all what I considered sort of bureaucratic speak and run around, and I tried and I tried. Finally somebody told me you have to find somebody who's making thalidomide. And of course then I found it wasn't really being imported into the United States, and that would be a huge problem, and it would be very difficult, and I couldn't find anybody to give it to me.

Hopefully, things have changed since then. There is more transparency to the agency, but I tell you this story to say at one point, you know a lot more about CDER right now than I did at that point. It was very daunting. It was kind of black box. There wasn't really open portals for which those citizens who wished to interact with the agency had an easy access to figure out what to do or whether something was possible.

Still today, I think we suffer. People, citizens, whether they're representing patient groups, or consumer groups, or a pharmaceutical developer, or some other stakeholder, you may get back a very polished lawyer that has answers that really are difficult to make heads or tails of what we're saying. And I think, John, what you were talking about, there's a lot of misunderstanding about what we can and cannot talk about.

We still struggle with plain, ordinary language and just telling people like it is -- here's the scoop -- so that you understand where you stand. I think one of the major issues is that people here are very busy, the scientists and doctors. They're often heads down working on the data and looking at evidence and judging evidence both in clinical trials. Is the trial safe to proceed? Are they doing the right designs? Are they studying the right endpoints and so forth? And then everybody looks at the marketing applications and did they demonstrate what they needed to? Is this drug going to be okay when it goes out on the market? And then we have millions of reports of adverse events all the time, and we have to sort through those and make sense of those.

So much of our large scientific staff is working on that. Last year we approved, for example, 1400, I think, generic drugs, and we processed many more than that, probably over -- we processed thousands of them and sent them back to the manufacturer. So much of our staff is engaged in doing that work on behalf of the public, surveilling the adverse events, surveilling the facilities that make drugs and so forth. So it's hard for them to switch gears from that extremely scientific activity that they do and then face outward and work with the general stakeholder.
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Communities.
So we have been trying for years to develop better interfaces so that anybody who comes to talk to us, whether they're small business, whether they're a patient or patient advocacy group, they know where to go and how to get entree into the agency, and then kind of how to work the lever so that somebody will hear their case or they have somebody to talk to get their questions answered. Our Division of Drug Information answers thousands of emails and calls every year, thousands and thousands, and yet there seems to be an insatiable appetite for information about drugs and health, and about the drug development process, and about whether generic drugs are good enough, and whether biosimilars are actually the same as other drugs; all these questions, and I had this side effect and could it be related to the drug I was taking, and so forth and so on.

At a higher, more integrated level, beyond personal questions, we have hundreds of stakeholders, why aren't you moving development for our orphan disease fast enough; why aren't you developing new endpoints for this very serious disease such as, say, Alzheimer's? A lot of people don't realize -- and partly because we need to effectively communicate with them, -- we're funded as a regulatory agency. We aren't funded to do research. A lot of Americans think we do all the clinical trials, for example, that the companies do and that NIH funds and so forth. They think we do them. So if anything goes wrong, they call us, like why didn't you do this trial about this drug? There is just a tremendous amount of misinformation. So many stakeholders, some of whom are highly sophisticated and many of whom really were like me with the thalidomide, never heard of the federal Code of Regulations or whatever and really don't understand the FDA whatsoever, except that we want something, and they're part of the potential solution.

In addition to setting up better transparency, one thing we try to do is put more information out, like the snapshots that PASE has done because people ask us everyday -- they were asking us why don't you put more women in trials, why don't you put more elderly in trials. So now we're putting out the numbers. First it's like, we don't do the trials but here are the people the trials had in them, and this has helped I think tremendously to elevate the level of dialogue about that particular issue.

So we're trying to have more transparency. But then we also are trying to have better avenues to work back and forth so that people can get their questions answered and also can interact with the appropriate part of CDER that they want to interact with.

Some things we will discuss today, how to set up a meeting with CDER, how to request a meeting outside of the drug development process, which we have very structured meetings for generic drugs and new drugs and so forth, but for other stakeholders, how do you do that? We're trying to get a system in place -- we have gotten a system in place, but we really need to make it work, how to get a meeting with the right people and make sure that if you meet with the center, that your agenda is fully vetted and the right people are at the table to engage with the issues.

So that's something we've done. We've tried to improve transparency. We've tried to improve our outreach and the amount of information that is available to people, and then we also need to improve this back and forth so that people can come in and get their questions answered, or advance their own agenda, or let us know what their agenda is. But for that to happen, we need to have at least semi-informed stakeholders.

The first five calls I made to the FDA back in the day of trying to get thalidomide, I was very ineffective because I didn't even know what an IND was, so I was starting at the most basic level of asking information. It's a very inefficient process, and at the time, I had to ask many people and learn many, many things to the point where -- and I had actually participated in
1 clinical trials. I had done a lot of things, but I
2 didn't know all this bureaucratic regulation
3 underlying all this.
4 One of the most important things is that we
5 help the level of engagement to the point that
6 we're dealing with informed stakeholders who at
7 least can formulate in their own minds what they
8 want from us so they can ask the question. We
9 almost need a leading edge of interaction that we
10 inform everybody about what we do, what we don't
11 do, what you can expect from us, and what actually
12 some other agency does or things we aren't actually
13 doing. No, we can't do a clinical trial in this
14 area. We don't do clinical trials. We're not
15 funded to do clinical trials.
16 FDA may fund a clinical trial very rarely on
17 some raging issue, but we don't get appropriations
18 to do that, so it's not our mission really. If
19 people can fix our role in their mind more firmly,
20 then we can have a better back and forth, or people
21 can go and make their point to an agency that does
22 do clinical trials and that would be appropriate to
1 talk to about that.
2 I think that's why this kind of meeting is
3 so important. You're already much more informed
4 stakeholders than our average stakeholder out
5 there. You knew about this meeting and would come
6 and interact with us. The more we can get an
7 informed level of stakeholders out there, then you
8 can help a lot of the other people, people you may
9 represent a part of your group or whatever; to
10 understand how to formulate their questions what
11 the agency can and can't do; what is appropriate to
12 pressure us to do in which it won't be useful
13 because we're not really in charge of that thing,
14 whatever it might be that people might want.
15 So I would encourage everybody today, ask
16 your questions. Get as much information as
17 possible. Reach the highest level of mutual
18 understanding as we possibly can because this will
19 only inform the dialogue over the next year. We
20 really do want to serve all of our stakeholders,
21 and we want to hear their voices, but we want to do
22 it in an effective way and hopefully a way that's
1 efficient both for you and for us so that you won't
2 be like me, or your various constituents won't be
3 like me, floundering around through a multiple,
4 multiple, multiple range of phone calls and
5 investigations trying to figure out something that
6 actually is very simple when somebody explains it
7 to you in plain language.
8 So I thank PASE for putting this on. I
9 thank all of you for showing up and the people
10 online, too. I think this will be a very useful
11 exchange, and we'll, again, tip the axis of that
12 ocean liner yet a little toward our ultimate goal
13 of really serving our stakeholders well. Thank
14 you. Thanks, John.

Min-U-Script® A Matter of Record (6) Pages 21 - 24
(301) 890-4188
1 Audience Response Questions - Noah Goetzel
2 MR. GOETZEL: Thank you, Dr. Whyte.
3 Hello, everybody. My name is Noah Goetzel.
4 I'm an ORISE fellow in Dr. Whyte's Office of
5 Professional Affairs and Stakeholder Engagement,
6 PASE. How is everybody doing today, first of all?
7 You guys doing all right? Everybody good? Find
8 this place okay?
9 (Audience responds.)
10 MR. GOETZEL: That's good to hear.
11 Are you excited to learn how to interact
12 with the regulatory scientists here at FDA Center
13 for Drug Evaluation and Research?
14 (Audience responds.)
15 MR. GOETZEL: Yes? You guys are excited.
16 That's good to hear. Well, we're delighted to have
17 you because we want to empower stakeholders like
18 you to share your unique perspectives. Whether
19 you're a patient, a caregiver, an academic
20 researcher, or a healthcare provider, or any other
21 type of representative here today, we're interested
22 in helping you guys share your voice with the FDA.

1 So before we get into our impressive slate
2 of presenters and presentations, I have a few
3 questions for you guys. I'm going to turn the
4 tables. You're going to answer these with your
5 clickers, which are in the center of the tables,
6 and we can go ahead and get started with the first
7 question.
8 Is this your first time attending a meeting
9 at the FDA? Click A if the answer's yes and B if
10 it's no. I'll give you a couple of seconds to send
11 in your answer choices.
12 (Audience responds.)
13 MR. GOETZEL: Let's check out the results.
14 For 80 percent of you, it's your first time here
15 coming to the FDA, so welcome, everyone, and only
16 20 percent have been here before. I apologize.
17 I'm sorry. Eighty percent of you have been here
18 before. Welcome back.
19 (Laughter.)
20 MR. GOETZEL: All right. Next question.
21 How confident are you in understanding the
22 different functions of the Center for Drug

1 Evaluation and Research? A, not at all confident;
2 B, somewhat confident; C, very confident. More
3 confident than I am in terms of reading answer
4 choices.
5 (Laughter.)
6 (Audience responds.)
7 MR. GOETZEL: Everyone send in your answer?
8 Let's see. So most of you are in the middle;
9 58 percent said somewhat confident, and then we
10 have 20 percent who said you're really not at all
11 confident right now, and 22 percent are very
12 confident. So hopefully by the end of this
13 presentation, you'll become a little bit more
14 confident in terms of what we do here at CDER.
15 I've got one more question for you guys.
16 Finally, how confident are you in your ability to
17 navigate through engaging with CDER? Same choices,
18 A, not at confident; B, somewhat confident; or C,
19 very confident.
20 (Audience responds.)
21 MR. GOETZEL: Let's check out the responses.
22 Okay. So once again, a lot of you are in the

1 middle. We have more people this round; 44 percent
2 saying that they're not at all confident in
3 engaging with CDER, and then 2 percent of you are
4 the experts, you're very confident, but most of
5 you, 53 percent, somewhat confident.
6 The goal again, after the presentation,
7 everyone's going to be very confident and engaging.
8 That will wrap up the ARS presentations. We're
9 going to keep going back to these clicker questions
10 throughout the day to keep you on your toes, and
11 the questions are going to get tougher. So this
12 was the easy round, and it's going to get more
13 difficult.
14 I want to give a quick shout out to our
15 social media staff here at CDER. We are tweeting
16 this event, so throughout the day, you'll see
17 information about the different speakers and
18 presentations. You can feel free to follow along
19 and join the conversation. Just follow the FDA
20 drug information account, that's FDA_drug_info, and
21 the hashtag for this public workshop is
22 #CDERandyouengagementworkshop.
So we look forward to hearing your responses and want to make sure that everyone has the opportunity to find out what happens and what we cover at our event today, even if you're not here in person or following along with our webcast.

Next up, we've got a video for you guys. During the ARS questions, I asked how confident you are in understanding the functions of CDER, and a lot of you said you're kind of somewhat confident. So I want to make sure that you have the opportunity to learn more about what we do in terms of the drug approval process, and you'll hear a ton of presentations today. My colleague in PASE, Lieutenant Commander Sadhna Khatri, will be leading our presentation on what CDER can and can't do.

This video, which you can search on the internet or on the Professional Affairs Stakeholder webpage, will tell you five things you need to know about the drug approval process. So you can go ahead and just Google if you're trying to find the FDA five things you need to know video, and you can enjoy this video that we have for you. It will be just two quick minutes.

MR. GOETZEL: There you have it. We're going to have a lot more presentations today about what CDER does here in the FDA and how to engage with us. Thanks very much. Enjoy the presentations.

DR. WHYTE: I want to thank Noah. Noah is what's called ORISE fellow. It's acronyms all over. I'm not even sure what ORISE stands for. But essentially, Noah is similar to an internship. It's time to come here and spend, and learn about the FDA. And I know he didn't know all those acronyms before he got here. He certainly didn't know CDER and IND and all of that. But I really want to thank him for the work that he's done, especially for this workshop. He's the youngest person on our team, so that's why it's always -- he's in charge of our social media and all the other efforts. But really, he's done an enormous amount of work helping to bring this together. So I wanted to thank Noah. And he has a podcast, too. I don't want to get in trouble like other people I mentioned, but he's big in basket ball. Go Villanova. Okay.

With that, I'm very pleased to talk about -- we have something new to announce at this meeting since many of you have been to previous meetings. And a challenge has been, over the years -- and Dr. Woodcock referenced it -- it's hard to figure out who do you meet with if you want to have a meeting. And it's hard to contact anybody here. How do you possibly find out? I mean, I don't know who answers the phone and directs you to the right number. And does anybody call anybody anymore anyway? You just kind of email. So how can you figure out who do you meet with and how do you get a meeting?

Recognizing that, we have created and just launched an online system for meeting requests for stakeholders outside of regulated industry, and we're affectionately calling it ESMR, the External Stakeholder Meeting Request. And if you have a better acronym for it, we're always willing to listen. This is an attempt really to make it easy for folks to request a meeting on drugs, and you don't even need to know who you need to meet with. And Chris Melton is going to come up and demonstrate it live, and hopefully it will work.

Do we have anyone here from Texas? (Applause.) DR. WHYTE: Okay. Chris' fun fact, so he says, is that he's from a small town in Texas called Jefferson. I don't know if it's Thomas Jefferson or Jefferson Davis. But he says it's the most haunted town in Texas.

Karen, is that true? I don't know, but we'll find out. But he's going to come up and demonstrate the ESMR system. Thank you. Presentation - Christopher Melton MR. MELTON: Thank you, Dr. Whyte. Good morning, everyone. I wanted to go back to Noah's fun fact. Were you able to keep the burrito down after you ran the lap? Great.

As Dr. Woodcock and Dr. Whyte alluded to,
1 the whole point of ESMR, it's a three-part
2 solution, and the system, and the part I'm going to
3 go over, is the website. That will be facing you.
4 As Dr. Whyte alluded to, I'm a health
5 communications specialist in the engagement staff,
6 and I want to go over the parts for the web page.
7 And as I transition through the workshop, I will
8 basically go through the live demo and show you
9 everything that will need to be seen. Before I get
10 into the demo, I will use about 5 to 7 minutes to
11 give you a brief live demo, and then from there, we
12 will move into the Q&A section.
13 Before I go to the live demo, just to give
14 you some background, parts of the 21st Century
15 Cures Act mandated integrating patient experience
16 into the drug development process, and that
17 increased patient centricity. CDER has increased
18 the number of interactions with external
19 stakeholders, specifically with patient advocacy
20 groups, with diverse needs and expectations in the
21 understanding about the drug development process.
22 The current environment at CDER was that

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<td>Moving back to Dr. Woodcock's vision, she</td>
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1 are the key essential information that we need to
2 run the meeting and get the key factors that are
3 needed.
4 Once that entered -- and again, I'll go to
5 my test -- you click the "submit" button, and then
6 that goes to our CDER PASE inbox. And you'll get a
7 response that within 7 business days, someone from
8 PASE will get in contact with you, and we will then
9 start triaging the meeting process.
10 With that, we will move to the question and
11 answer section, and if anyone has any questions,
12 please feel free to go to the microphones. And
13 we'll have a runner also bring the mic around so
14 you can ask any questions.
15 Questions and Answers
16 FEMALE AUDIENCE MEMBER: Hi. I just have a
17 question on the form. I didn't see like if you
18 already had a contact at FDA, is there a place in
19 that form that you can enter their name to help you
20 with your process of routing it to the right
21 division or person?
22 MR. MELTON: Good question. Currently right
23 now, there's not a section in the form where that
24 could be done, but what we will do when we call it
25 with the initial information, that's when we can
26 tease out that information for the people that you
27 currently have relationships with.
28 DR. WHYTE: I think the other point is we do
29 have a section on there about proposed attendees.
30 It's all free form as well, so I think you could
31 add in there the contact that you have. This is
32 meant to simplify a process as well, so we don't
33 want to duplicate anything.
34 The reality is, many folks, and probably
35 folks that are here, do have some organizational
36 awareness. So what we've talked to our colleagues
37 is for meetings that are already taking place.
38 There's already a set plan, and our friends and
39 colleagues in the Office of Hematology and Oncology
40 Products and oncology in general have very good
41 relationships with patient groups. We're just
42 ourselves going to put it into the system because
43 at the end of the year, the system will also allow
44 us count.
1 So it's important to understand, for the
2 center and for the senior leadership, how many
3 meetings have we had on patient engagement and what
4 are the patients and other groups -- this is also
5 for other groups other than regulated industry, so
6 it could be health professional, physician groups.
7 What are they coming to talk about? Are they
8 coming in to talk about neurologic diseases, or are
9 they coming in to talk about psychiatric
10 conditions? Is it dermatologic issues?
11 In the past, we had to do this all manually,
12 which is very hard to do. And again, this really
13 is a way to encourage dialogue, to encourage
14 engagement, which is two ways. So it's a good
15 point. We don't want to duplicate anything or
16 complicate anything. In many ways, with the
17 free-form text, people can just add that in. And
18 we do promise to get back to folks within
19 7 business days. That doesn't mean that we'll have
20 a meeting in 7 business days, but we're certainly
21 going to get back to you to start the process.
22 MR. MELTON: Any more questions? We have
23 another one.
24 MS. FOXWORTH: This is Phyllis Foxworth from
25 the Depression and Bipolar Support Alliance. Thank
26 you so much for being here. When I first started
27 engaging with the FDA, I certainly was not a
28 professional patient. I'm just a patient and a
29 caregiver of other patients. I still don't
30 consider myself a professional patient.
31 So my question is -- the form is great, and
32 maybe we'll cover this throughout the day. But the
33 real question is, why would I want to schedule a
34 meeting and what is the type of meeting I'd like to
35 schedule given that I'm not a professional patient?
36 MR. MELTON: With that, when you're asking
37 you're not a professional patient, there's a
38 progress. One reason would be for initial
39 information that you would need to access, we can
40 schedule a meeting and a fact-finding. Then it
41 also could be another meeting for whatever you
42 need. But the whole point is getting the
43 information of your main goal. Even though you're
44 not a "professional," quote, patient, whatever your
1 need would be, we would tease that out from
2 discussions with you, and then decide what would be
3 the best route forward with the meeting.

4 DR. WHYTE: Sorry. I keep jumping in. And,
5 Phyllis, it's nice to see you. I don't think we
6 view people as professional patients or not
7 professional patients. I'm not even sure
8 necessarily what that means. But what we hear from
9 the public is this need for information, this unmet
10 clinical need that they're concerned about drug
11 development in a certain space, and they may not
12 even use those terms; or they're concerned about
13 side effects; or they want to know why aren't there
14 drugs for lupus. Why aren't there drugs for a
15 certain neurologic disease? In many ways, we're
16 responding to folks, and there are many in the rare
17 disease community that have this desire to impact
18 the lives of their loved ones.

19 So there is no set agenda of what people
20 have to talk about to us, and in many ways, we
21 can't tell you what you should or shouldn't talk
22 about. We can tell you what we can't talk about;

1 for instance, if a drug's currently under review.
2 But we really want to encourage that dialogue.
3
4 When we talk about how do we measure what's
5 clinically meaningful to patients, it's hard to do
6 that unless you actually engage with patients. And
7 Dr. Woodcock often talks about that patients are
8 experts in their own disease, and you may have
9 heard her say that. So in order to do that, we
10 have to engage with patients. And I'm particularly
11 using that term "engage" because it's a two-way
12 communication. And historically, the information
13 on both sides has been pushed out. The agency
14 pushes out information that they want their -- and
15 advocacy groups push information back to us. And
16 we really have to have engagement and two-way
17 communication.

18 So part of that is -- you're right -- that
19 folks don't even know the FDA or think about the
20 FDA. And that's part of our goal, to try to
21 educate folks more about the drug development
22 process, and Dr. Woodcock talked about that. But
23 really, nothing's off the table in terms of what

1 you can ask to talk about.
2 MR. BARTEK: Guessing that there's probably
3 a full range of responses that one could expect
4 from such a request, might you give us an idea of
5 what that range might be. I doubt every request
6 gets a meeting.

7 DR. WHYTE: Sure. And he's from Texas as
8 well or he raised his hand. Part of it is to have
9 that phone call or to contact folks after the
10 meeting request. And I will tell you, just from
11 phone calls that I have made, people are always
12 surprised when they get a call from us. They're
13 shocked as if somehow we were unplanning to do
14 anything.

15 But our goal is to call people. Everything
16 doesn't always have to be these long emails. And
17 what we have in the agenda that we ask people, and
18 what I'm pushing folks on my team, is what is the
19 ask. So when we call you, I really want to know
20 what do you want to meet about, and we push even
21 more in terms of what do you want the FDA to do as
22 part of this meeting?

1 So that really dictates what kind of meeting
2 is it. Is it simply that you want to make us aware
3 of the severity of the disease and what's on
4 people's minds? Is it you don't feel there are
5 right endpoints; you're confused about certain
6 information? And I will tell you, all the requests
7 that have come in so far, our goal is really to
8 have a low bar for meetings, meaning we're trying
9 to encourage engagement.

10 So right now we are having a lot of
11 in-person meetings, but we're also thinking about
12 and interested in hearing people's viewpoints. And
13 it might be kind of a D.C. type of thing where
14 everyone likes to have a physical meeting, and in
15 many ways I think we can accomplish a lot by having
16 WebEx meetings or something of that nature, or
17 conference calls, because that also allows people
18 that may not have a lot of resources. Many of them
19 are caregivers and can't take two days of travel to
20 come to White Oak in Maryland. So we're really
21 trying to think through that as well.
22 But the default in a way is to have a
Meeting. What that means is open to what folks want to accomplish. I've been in the past on calls where -- Dr. Woodcock talks about people think we do clinical trials. I've been on calls where people think we make drugs, and we don't. So we also don't want to waste people's time to come in for a meeting on an area where we don't have regulatory authority. But our goal is to -- we're setting up a system to have meetings, so our goal is to honor those requests for meetings.

MR. MELTON: Okay. That will be our last question. What I'll do briefly is give the URL fda.gov/requestameetingondrugs. Thank you and have a good day.

MR. MELTON: So what I will do, it's fda.gov/requestameetingondrugs, that is progress, and you will be on your way to being an effective advocate, because part of the challenge is figuring out how do you meet with us and who do you meet with, and we're going to help you do that. So I really want you to remember that. I want you to promote that. I want you to talk about that to your friends and colleagues.

Now we're going to try to provide a little more about what we do here at CDER. And most of you have been at meetings, so you most likely know that CDER is the Center for Drug Evaluation and Research. So we work on drug issues. Our colleagues you'll hear from later might work on devices at the CDRH, or CBER, the Center for Biologics, and we'll talk a little bit about that. I'm going to introduce now Selena Daniels, and she's a team lead in the clinical outcome assessment staff in the Office of New Drugs. And she's going to talk a little bit about how do we incorporate patient experience data to inform the FDA. And it tells me that Selena is also a yoga instructor. And here is Selena. Namaste.

Presentation - Selena Daniels

DR. DANIELS: Thank you, Dr. Whyte, and I think the slides have transferred. Good morning, everyone. My name is Selena Daniels, and as Dr. Whyte mentioned, I am a team lead on the clinical outcome assessment staff here in CDER. And for those who aren't familiar with our role, we provide advice to the Office of New Drug review divisions in CDER in matters pertaining to the development of clinical outcome assessments and related endpoints. Today, I will be discussing how you can engage with FDA to collect patient experience data.

The patient perspective is an important part of the medical product development process, and FDA values the use of patient input to help foster the development and availability of safe and effective drugs. An article was published in JAMA in 2015 highlighting the importance of engaging patients across the spectrum of medical product development, and some of the key take-aways from this article was that capturing the patients' voice in medical product development is important. And if you can successfully capture it, it can transform the patients' experience of health care. One way to capture the patients' voice is to include clinical outcomes that are meaningful to patients from their perspective. I know I just threw out a couple of regulatory terms here, so I'm going to define them real quick.

Clinical benefit is a positive clinically meaningful effect on an intervention. In other words, it's a positive effect on how patients feel, function, or survive, and that can be how long a patient lives and how a patient feels or functions in daily life. This includes both improvement, but also may a delay in deterioration of a certain
1 symptom or aspect of that condition. Clinical
2 outcome is an outcome that describes or reflects
3 how an individual feels, functions, or survives,
4 and this can be assessed using clinical outcome
5 assessments. Typically, this could be a
6 questionnaire or this could be a task.
7 An important part of regulatory
8 decision-making is to carefully assess patients'
9 views on benefits and risks. For those who aren't
10 familiar with the 21st Century Cures Act, this is
11 an initiative to enhance the process of delivery
12 and development for disease treatments, and this
13 act now includes new statutory provisions for
14 patient-focused drug development. What this means
15 is that FDA is trying to incorporate the patient
16 perspective in a more systematic way for benefit-
17 risk assessment and taking into account patient
18 experience.
19 What is patient experience? The patient
20 experience in a medical product development context
21 essentially incorporates the patient's journey
22 throughout the course of their disease or

1 condition, which includes patient's views,
2 feelings, their needs, actions, preferences, and
3 interactions with respect to their disease and its
4 treatment.
5 Section 3002(c) of the 21st Century Cures
6 Act describes patient's experience data as data
7 collected by any persons that are intended to
8 provide information about patient's experience with
9 the disease or condition, and this can include
10 disease symptoms. This can include disease
11 impacts, experience with treatments, inputs which
12 outcomes are important to them, patient
13 preferences, or anything that's just an important
14 issue that's defined by patients.
15 So who should communicate patient experience
16 data? Of course, patients themselves. However,
17 there are instances where they may not be able to
18 communicate this, and in those instances, it can be
19 informed by input from patient partners and
20 clinicians. A patient partner may be an individual
21 patient, a caregiver, or a patient advocacy group
22 that engages other stakeholders to ensure that the

1 patients' wants, their needs, and their preferences
2 are represented in activities related to medical
3 product development and evaluation.
4 There are various different elements that
5 could comprise patient experience. Again, as I
6 mentioned, it could be disease symptoms. It can be
7 the burden of living with a disease, the burden of
8 managing the disease itself, impacts from the
9 disease or impacts from the treatment on activities
10 of daily living; patients' views on currently
11 available treatment options as well as unmet
12 medical needs; and again, patient preferences.
13 So how do you collect patient experience
14 data? FDA recommends using qualitative methods,
15 quantitative methods, or mixed methods to collect
16 robust and meaningful patient experience data.
17 This table provides a high level overview of these
18 different types of methods that can be used, the
19 first being qualitative methods.
20 Qualitative methods just includes the act of
21 just talking to patients. This could be using
22 direct communication to explore or confirm the

1 meaning of interpretation of a topic from the
2 participant's perspectives. An example of a
3 qualitative study could be having a focus group or
4 having interviews with patients where they're
5 describing their experience or their condition.
6 And the potential scientific objective for this
7 type of study would be related to experiencing or
8 exploring the most important aspects of that
9 disease.
10 Quantitative methods are characterized by
11 quantifying the data or using numbers, and this
12 generally entails statistical methods that are
13 summarizing this collected data. In regard to
14 collecting patient experience data, this could be
15 collected by the use of a tool such as a
16 questionnaire, and that questionnaire has a closed
17 set of questions where patients are selecting
18 response options most suitable for their response.
19 And it creates a score, which is numerical data.
To submit patient experience data, there are various pathways that exist. FDA will be issuing guidance on how to submit this data, so please stay tuned. Again, in regards to the content and the formats in terms of how to submit that data, it also depends on the purpose and the type of data that is being submitted. From a regulatory perspective, patient experience data is used to inform clinical trial design, clinical trial outcomes, trial endpoint development and selection, but it’s also used in our regulatory reviews, which includes benefit-risk assessments.

To summarize everything, patient engagement is critical throughout the medical product development process, and the way you can best help FDA is by using scientifically sound methods to collect robust, meaningful, sufficiently representative patient input to inform medical product development and regulatory decision-making.

That concludes my presentation, and I will open it up to questions and answers. There are mics, and I know there are runners around as well.

MR. BARTEK: Thank you, Selena. Can I ask about -- I would just say so many of our patient groups are now looking at new technologies for collecting patient data before and during clinical research. I’m wondering to what extent the developers of these new technologies -- I’m thinking of wearables and carriables and so forth -- are collaborating with you and your office, and others of your colleagues here at the FDA, in advance, maybe in the pre-competitive space so that when they develop these technologies and the way they’ll be used with our patients to see how they feel and function on a daily basis in their own environments -- so important to the endpoints that we’re trying to develop -- that they would provide technologies that would be useful for the regulator.

DR. DANIELS: No, most definitely. We are having engagements with some of the individuals that are using these technologies, and we are open to those novel methods. And we will be describing...
1 some of those novel methods in the guidance that comes forth for submitting patient experience data.
2 Are there any other questions?
3 Questions and Answers
4 FEMALE AUDIENCE MEMBER: Thank you so much.
5 That was very helpful. Do you have any published guidance that details for -- again, I don't want to use that word "non-professional," but I just think it's clear to make, that as patients, we're not scientists. So is there any guidance that's published that says what your criteria is as the definition of a scientific study as opposed to a non-scientific study?
6 DR. DANIELS: The guidance that's going to be put forth this June, the draft guidance on collecting patient experience data, is written using plain language, so it will go into the details of what are the best practices to do. But again, we also are encouraging if you are a patient or patient advocacy group to contact or collaborate with these subject matter experts because they do have the expertise to help you create these studies and use these methods to collect that data.
7 MALE AUDIENCE MEMBER: Is that the same guidance [inaudible - off mic]?
8 DR. DANIELS: Yes.
9 MR. BARTEK: Just a question. You're focusing on obviously clinical studies and stuff in your discussion. Will the guidance include, or the experience data that you're collecting include, things like how patients feel about size, shape, color, other kinds of physical attributes that sometimes can be a big issue from a patient perspective in terms of problems they have in taking the dose and that type of thing?
10 I know this is becoming a much bigger issue, and FDA has some guidances on physical design characteristics, et cetera. Will your data collection incorporate patient information on those kinds of things as well as more clinical type details?
11 DR. DANIELS: This first guidance on collecting patient experience data is going to be focused mainly on the methods that are being used to collect the data, but we do have a series of patient-focused drug development guidances that are going to be coming forth after this as well that will focus on how to elicit the most important concepts from patients. And it might touch upon in terms of some of those things, factors that you mentioned as well. But this guidance coming out in June, the draft guidance, will be focusing mainly on the methods and how to create representative inputs of talking to the right patients.
12 DR. HO: Thank you so much. This is Calvin Ho from the Tuberous Sclerosis Alliance. I was wondering if the June guidance will also be addressing using patient registries as a source for patient experience data.
13 DR. DANIELS: The draft guidance will be talking about different sources to use. Of course that could be focus groups; it could be registries. So it will touch upon that as well as what types of sources can be used to collect patient experience data.
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21 DR. DANIELS: The draft guidance will be talking about different sources to use. Of course that could be focus groups; it could be registries. So it will touch upon that as well as what types of sources can be used to collect patient experience data.
22 FEMALE AUDIENCE MEMBER: Hi there. I represent a patient community that is not yet a formal group. It's a rare, loosely defined group of individuals. Is that kind of non-starter in terms of creating or pulling together patient experience data or the non-professional aspect of their community, does that make a difference at all?
23 DR. DANIELS: No. Like I mentioned, anyone can collect data. I mean, we do encourage you to engage with us like if you want to know what the objective is and how to proceed, in that sense. You can do meeting requests as was mentioned in the previous session.
24 I think that was the last question. Thank you, guys. Have a great day.
25 (Applause.)
26 DR. WHYTE: Thank you.
27 One other point that Dr. Woodcock often talks about is when we're trying to collect information, there is a science to patient engagement. Sometimes there is this tension between the biologic sciences, which many of the
folks that FDA are trained in, and the social sciences, which is very much collection of data, but that can be just as rigorous as the biologic sciences. And sometimes when folks are thinking about a survey, they go on the process, which is well intention, but then there's not the science behind it. Someone might put it up on their website and have the first 30 people come, and that's useful information, but that may or may not be effective in a regulatory process. So that's why we want to have these meetings and early on have that discussion about what you're trying to do in terms of collecting information because there really is a science behind it. We do have on the website, fda.gov/requestameetingondrugs, those circumstances for which there are other types of meetings, such as the ones Selena talked about, but we can help facilitate that for you.

Now we're going to hear about the rare disease program because as I referenced early on, many of the folks that we hear from are in the rare disease community. I do want to recognize Larry Bauer in the back who really has been a champion of the rare disease program and rare disease advocacy. Larry, could you stand up and be recognized? I know many folks know you. You really have done a terrific job over many years in really addressing the issues of the rare disease community.

(Applause.) DR. WHYTE: We're going to hear about the rare disease program right now, and I'm delighted to introduce Dr. Lucas Kempf, who is the acting associate director of the rare disease program in the Office of New Drugs.

Presentation - Lucas Kempf DR. KEMPF: I am Lucas Kempf, the new acting director for the rare diseases program. My fun fact is I spent the last week in a car with my children driving cross-country, and I found out this morning that Kansas doesn't necessarily have the high speed internet to transfer my slides. So I'm not entirely sure which version of my slides are going to be in this talk, so please forgive me.

The challenges in drug development for rare diseases is that primarily since these affect very few people, a natural history is often barely understood or characterized. These diseases themselves tend to be serious, life threatening, and lacking approved therapy. For us at CDER this is important because there are very specific regulatory programs that Congress has passed to help develop drugs for serious life-threatening diseases, and especially rare diseases. The small population make it very difficult to recruit and design these drug trials. The disorders themselves may be diverse. And as we heard earlier, since there is very little known about these diseases, it's very hard to know what endpoints should be derived for these drug trials. What are meaningful clinical outcomes in these populations may not be well understood, and biomarkers for the improvement in these settings may often be lacking. It is frequently a lack of drugs that have been developed previously for these diseases, so we
1 don't know exactly how to do this. So frequently, these are completely novel programs, and nobody's entirely sure what the outcome should be as opposed to something for like major depression, where there are 16 or more different drugs that have already been approved. Also, about 50 percent of these disorders affect children, so there are special ethical considerations that you have to use when you're doing trials in children.

2 The rare diseases program, the reason we exist is to facilitate, support, and accelerate drug development for these rare disorders. The ways that we do this is a series of different kinds of responsibilities and programs that we enact. We help develop CDER policies and procedures through guidance developments and interactions with the senior staff.

3 We help develop good science in the areas of rare disease, so we develop a database of all the new drugs that are being developed to help inform the agency's understanding of what we need to do to help develop these sort of drugs. We also develop workshops to elicit external device to inform our internal thought processes about ways things should be done.

4 An example is this. We recently had a workshop on rare disease trial designs, which is fairly successful. Several hundred biostatisticians all showed up to discuss the ways that you could design trials when you only have a handful of patients and you can't necessarily use the standard statistics in that sort of fashion.

5 Internally what we do is we help educate our staff because a lot of the staff come in with not a lot of rare disease experience, and then suddenly they get an application that involves some small population of only a hundred people. How do they even approach that drug development program. So we run a 101 course for our new reviewers, and we also have an advanced study day-line course that we educate our staff yearly.

6 We also do external training. We give presentations at national and international meetings and interact with workshops. We do a lot of patient-focused drug development group meetings either here at the FDA or the ones that are being externally led to help our community develop meaningful data coming out of those meetings that will help inform the drug developers about the needs of the patient groups.

7 Internally, we also help work on one of these acronyms, the PRV program, priority review voucher, which is an incentive that Congress has given drug companies to develop drugs for rare pediatric diseases. We also are members sitting on the Rare Disease Council within the FDA, and we work with our external groups such as NORD. We have a cooperative agreement with them to help develop some of these registries that develop the information that we were discussing earlier.

8 We're also charged with working collaboratively with our stakeholders. We work with the NIH on a joint task force. We work with their rare disease annual meeting and work with TRNDs and NCATS to help them with their natural history study initiative. Also with the patient groups, we work in these face-to-face meetings that PASE, P-A-S or OHCA helped set up.

9 We give presentations to the stakeholder groups when they have meetings to help them go through some of the regulatory hurdles that they may be encountering as they partner with drug companies for drug development. We help National Organization for Rare Diseases have their annual meeting, review their program, and help set up their poster sessions.

10 So why is this all important? If you just look at drug development currently, this is a graph of new molecular entities. These are the brand new drugs. They've never been developed before, so there is this special category. And as you can see, year by year, they're kind of going up in the United States.

11 Now, when you look at how many of these new drugs are for rare diseases, and you look at year to year, just looking at the last three years, about 40 percent of all new molecular entities that are being developed in the United States are for
rare diseases. This is a considerable part of
current drug development.
I'll just kind of skip by that. These
programs for rare diseases, when you look at that
40 percent -- these is our most recent updated
numbers -- about 56 percent of those are first in
class. These fast-track, breakthrough, and
priority reviews, those are accelerated programs
that involve more interaction with the staff. And
we're seeing that, at least in the United States,
72 percent in this last year were of rare disease
drug development was first done here in the United
States.
This is ever-increasing. In order to get a
rare disease drug designation, you send in an
application that tells the agency that you're
developing your drug for a rare disease, and we
chart this. As you can see, the number of
designations and requests that are coming in are
accelerating over the next couple of years. So
this is going to become a larger and larger portion
of the agency's drug portfolio. We're going to
skip by that. This is the rare disease priority
review determinations for the pediatrics, so that
also is going up.
Another important part of our program for
those of you who have been here before -- and it
seems 80 percent of you all have -- in the last
7 years, we've developed an EMA-FDA rare disease
cluster. This cluster is helping facilitate and
accelerate drug development due to the fact that we
recognize that rare disease drug development is an
international program. It's almost impossible to
do a drug development program just in the United
States. Therefore, we need to have greater
coordination between what you're being asked to do
in the EMA and the advice that you're getting of
what you should do in the FDA.
So what we've started up in the last year is
monthly meetings with the EMA to help coordinate
and collaborate with them in the advice that we're
giving in all phases of drug development, even in
the IND, in the early drug -- or even before some
drugs are being developed, we help coordinate. We
1 Questions and Answers

2 DR. HEALY: Thank you. Kevin Healy from Roivant Sciences. A lot of great information and a lot of work you're doing there, but certainly the rare diseases cut across CDER and CBER. I wonder if you could explain a little bit about your team -- you mentioned the placement within CDER -- and how that can apply for development of biologics, and even with the EMA-FDA cluster.

3 DR. KEMPF: So biologics themselves fall into both CDER and CBER. We throw around these terms. An NDA is for a new drug application. A BLA is for biological applications. A lot of the BLAs are actually done in CDER, so antibody products, small nucleotide RNAs, those sort of things, all fall in CDER. But we do coordinate with them.

4 CBER itself has a group of rare disease professionals. It's not quite as organized as a program itself like we are, but the council that we meet, CBER is on that. CDRH is on that. We're on the agenda with us.

5 That. So we all meet together to make sure that we coordinate. CBER is also part of our educational program, so when we develop our educational internal meetings, they have members to help define that agenda with us.

6 So while we have the defined program, we're always working collaboratively. Actually, some of the EMA cluster meetings, we bring in folks from CBER because the EMA doesn't necessarily break it up the same way. They just say we want to talk about this product, so we put it on the agenda.

7 Do I have any other questions?

8 DR. LUO: Hi, Dr. Kempf. My name is Michelle Luo. I'm from the Office of Women's Health. I think I have two questions. First, in the device review, they have HD, called humanitarian device exemption. Usually, the patient has less than 4,000, but that's before definition. So they're only looking for more safety, not the effectiveness or efficacy. I was wondering for the drug review, do you assess both safety and efficacy? That's my first question.

9 Did that answer all your question? Thank you.

10 Are there any other questions?

11 (No response.)

12 DR. KEMPF: Thank you.

13 (Applause.)

14 DR. WHYTE: Thank you. And now we're going to test your knowledge, so get out your clickers. And we're going to have Jamie Bishop, our program manager, come to the front. And her fun fact, which is very hurtful for someone who worked at Discovery Channel for a decade is she says she doesn't watch television.

15 DR. KEMPF: I think you're referring to the definition for humanitarian device exemption. That didn't change the definition for drugs. The drug's definition is the same. What did change recently is with the pediatric group review, is that it used to say the majority of your patients had to be 50 percent. There is some realization that that was leaving out some very important groups, so they changed the definition to say that the serious and life-threatening aspect of the disease has to primarily affect pediatrics because if you look epidemiologically, you could see a small population of patients who are children and may pass away, but then a less affected group may continue living. And then the general population would be higher, though the serious aspect that you're trying to develop your drug for could be affecting this small group, and then they wouldn't get the pediatric review voucher, which wasn't the intent of Congress initially.

16 Did that answer all your question? Thank you.

17 Are there any other questions?

18 (No response.)

19 DR. KEMPF: Thank you.

20 (Applause.)

21 DR. WHYTE: Thank you. And now we're going to test your knowledge, so get out your clickers. And we're going to have Jamie Bishop, our program manager, come to the front. And her fun fact, which is very hurtful for someone who worked at Discovery Channel for a decade is she says she doesn't watch television.

22 So let's hear your questions.
MS. BISHOP: Good morning, everyone. The first question is, who develops and tests new drug products before they reach the public, A, FDA; B, physicians and healthcare systems; C, pharmaceutical companies and other investigators; D, a consortium of international regulatory authorities, including the European Medicines Agency; and E, all of the above?

Please select the corresponding answer on your clicker.

(Audience responds.)

MS. BISHOP: The correct answer is C, and 54 percent of you picked C.

The next question is about the rare diseases program at the FDA. The rare diseases program within CDER, A, provides training to medical reviewers on rare disease drug development; B, collaborates with the National Institutes of Health to accelerate drug development; C, works interactively with rate disease stakeholder organizations; D, works to speed the review and approval of drugs that treat rare diseases; and E, all of the above.

(Audience responds.)

MS. BISHOP: The correct answer is E, all of the above.

The third question is, what initiatives did the FDA launch in 2013 to gain patient perspectives on specific diseases and their treatments through a series of patient meetings to better inform the drug review process? A, Clear Path Initiative; B, Pharm More Information Campaign; C, No Trial Left Behind; and D, the patient-focused drug development.

(Audience responds.)

MS. BISHOP: And the correct response is D.

My final question is, what can public stakeholders like you do to request to meet with the experts from the FDA Center for Drug Evaluation and Research? A, nothing, you're out of luck; B, stop by the White Oak campus uninvited and ask FDA security guards very nicely if any CDER division directors are free for lunch; C, visit fda.govrequestameetingondrugs and submit a simple meeting request form; D, submit a letter of intent to patientfocus@fda.hhs.gov that indicates your interest in conducting an externally-led, patient-focused drug development meeting; or E, both C and D.

(Audience responds.)

MS. BISHOP: The correct answer is E. Thank you.

DR. WHYTE: I am glad no one chose the No Trial Left Behind Act.

With that, we are running a little ahead of schedule, but we'll take a roughly 20-minute break. We'll definitely start promptly by 11. You saw the question about stop by and ask if anyone's free for lunch. I do want to remind people, if you want to eat lunch and you didn't pack your lunch, you should consider placing an order for lunch. I know many of you folks have been here before, but in theory, you cannot get to the cafeteria without an escort, so it may or may not happen; sometimes it does, sometimes it doesn't. I do not want anyone to get cranky at 2:00 because they haven't eaten.

So get a plan in place for the next 20 minutes. And after lunch, we are going to play Jeopardy, and we're going to divide into teams. So see you again in about 20 minutes. Thank you.

(Audience responds.)

Whereupon, at 10:37 a.m., a recess was taken.

DR. WHYTE: At this time, I'd like to welcome Dr. Elizabeth Hart up to the podium. Dr. Hart is a medical officer in the Division of Gastroenterology and Inborn Error Products in the Office of New Drugs, and she's going to provide insight into the needs of the CDER drug review divisions. And a fun fact about Elizabeth is that she has worked on four continents and traveled to six, and has competed in multiple triathlons.

More to hear on what you did on all those continents. Thank you. Antarctica?
DR. HART: No, that's the only one.

DR. WHYTE: All right. Thank you.

Elizabeth Hart

DR. HART: Thank you for having me. Good morning, and welcome to everyone. My name is Elizabeth Hart, and as he said, I am a medical officer in the Division of Gastroenterology and Inborn Error Products within the Office of New Drugs, within the Center for Drug Evaluation and Research. This morning I'm going to talk about the needs of the CDER review division, specifically a little bit about what we do, the regulations behind what we do, and then where are there opportunities for patients and patient advocates to get involved.

I have no disclosures.

The primary work of the CDER review divisions is to evaluate the efficacy and safety of new drug applications by sponsors. We don't determine the priorities. We don't determine which drugs are being evaluated for different diseases, but whatever comes in we evaluate. So we can't prioritize. That's up to sponsors, as you got in the questions.

The drug evaluation process is very important to our shared goals of having safe and effective new therapies for patients in need and to do that review as quickly as possible so that these new drugs can be marketed and available if they are determined to be safe and effective. In order to aid sponsors in the drug development process, we are involved, and are willing to be involved from the early stages of drug development, and continue to be involved postmarketing to further assess safety.

A little bit about the drug development process. Typically people think of it starting at the IND phase, which is when an investigational new drug is being evaluated for use in humans in the United States. However, there's actually a huge amount of work that happens before a drug ever reaches that point. There are issues with the discovery and the nonclinical research. But also, particularly for rare diseases -- and I will come back to this a little bit later -- are issues related to understanding the disease and how to measure important outcomes. And there's also a role for all of you there as well.

After a drug has an IND, it goes through the clinical development process. There are phase 1 studies to determine safety and tolerability; then there are phase 2 studies, which are dose ranging, proof of concept; and phase 3 studies are considered to be the pivotal safety and efficacy studies at which point an NDA for a new drug application or a BLA for a biologic license application can be submitted, is evaluated and reviewed, and then there is continued evaluation in the postmarketing setting.

That's the brief process. There are regulations that determine all of these. Specifically, the 1962 drug amendments to the Food, Drug, and Cosmetic Act requires the establishment of effectiveness of the drugs as a prerequisite to marketing approval. That effectiveness is further defined as substantial evidence, and substantial evidence consists of adequate and well-controlled investigations performed by qualified individuals.

And then the results have to be determined to evaluate the effectiveness of the drug involved on the basis of which it could fairly and responsibly be conducted, that such experts -- that the drug will have the effect it purports and is represented to have under the conditions of use prescribed, recommended, or suggested in the labeling or proposed labeling thereof. A lot of regulations.

I have not memorized them all.

Let me talk a little bit more about the adequate and well-controlled studies because these are the hallmark of moving a drug to market. It's important to distinguish the effect of a drug from other influences, including spontaneous change, placebo effect, and biased observations. An adequate and well-controlled study must have multiple characteristics. I want to highlight just a few of them.

One is that there are appropriate controls for valid comparisons -- it seems obvious but it's actually much more challenging in
1 practice -- appropriation selection of subjects;
2 and well defined and reliable methods of assessing
3 that response, as well as adequate measures to
4 minimize bias and perspective planned analysis
5 with rigor.
6 Once these studies are performed, how do we
7 determine clinical benefit? And that is also
8 defined for us. Treatment benefit occurs when a
9 drug positively affects how a patient feels,
10 functions, or survives, as discussed previously by
11 Dr. Daniels. But these are really important points
12 because it gets to things that are important to
13 patients. That clinical effect must be clinically
14 meaningful in the context of the given disease. So
15 we’re not talking about just statistical change.
16 We’re talking about clinically meaningful change to
17 outcomes that are important to patients.
18 This all sounds very straightforward on
19 paper, but the challenges come in practice. These
20 challenges are amplified, as Dr. Kempf said, when
21 it comes to rare diseases. First of all, with
22 rare diseases, we’re dealing with small

1 populations, which means even more so there are
2 limited opportunities for study and replication.
3 Every patient always counts, but especially in rare
4 diseases and in rare disease trials.
5 There’s an additional challenge of the
6 disease being heterogeneous, so these differences
7 can’t always be dealt with because of the small
8 samples based on statistical analysis. So you want
9 to make sure that results aren’t being driven by
10 outliers and that you understand, again, that
11 effect is coming from the drug versus it is based
12 on change in the population.
13 There are problems that sometimes we just
14 don’t even understand the disease manifestations,
15 so making sure that the drug is targeting something
16 that is meaningful and being able to distinguish
17 effects of the drug from effects of the disease.
18 With rare diseases, as we mentioned, there are a
19 whole variety of them, and, unfortunately, many of
20 them don’t have available drugs, which means that
21 there’s no precedent for drug development. That
22 means that endpoints, outcome measures, tools,

1 instruments, all need to be figured out for each of
2 these diseases.
3 It’s not easy, but it’s doable. And as you
4 can see from the previous examples, there are
5 multiple new molecular entities that have been
6 recently approved for rare diseases. But to
7 develop more of them, where do we start? We
8 actually start with the end in mind. This is a
9 picture from Namibia, one of those countries I
10 travel to, and it’s really important to recognize
11 that the path isn’t always smooth or easy, but it
12 is possible. So think about what is going to be
13 clinically meaningful and evidence of benefit, and
14 then how do you design an adequate and
15 well-controlled trial to measure that.
16 What can patient and patient advocates do to
17 facilitate drug development? There are certain
18 steps irrespective of what industry and different
19 sponsors are doing to develop drugs that apply to
disease-specific populations. This includes
20 understanding the disease by performing natural
21 history studies, which I will discuss in more
22 detail; provide the patient experience data, which
23 Dr. Daniels talked about earlier this morning; and
24 then to also, if possible, validate those
25 qualitative and quantitative assessment methods;
26 and when trials are being performed, encourage
27 participation.
28 A little bit about natural history studies.
29 These are comprehensive studies that are designed
30 to characterize the disease over time, starting
31 from the pre-symptomatic phase through the early
32 symptomatic, through the late symptomatic, and then
33 either to resolution of the disease to stable
34 disability or death.
35 It’s really important that these studies
36 capture as much of the population as possible and
37 identify variables that correlate with disease
38 progressions and outcomes in the absence of
39 experimental therapies; and as things move more
40 towards personalized medicine, understanding the
41 different features that impact the disease are
42 especially important. These studies are not the
43 same as registry studies, but they can be performed
1 prospectively or retrospectively.
2 So what do we do with these natural history
3 studies when they're available? They really
4 provide the scientific framework for rigorous
5 investigation that allow us to understand disease
6 outcomes and variability within disease
7 populations. This can inform trial design as far
8 as endpoints, determining a homogeneous population
9 to study, and then can also help to determine what
10 is a sample size to detect effect.
11 Rarely, but sometimes they can serve as
12 external controls for a pivotal study.
13 Particularly in rare diseases in which the disease
14 course is highly predictable, the endpoints are
15 objective, and there can be a dramatic treatment
16 difference, an external control can be used. But
17 in order for that to be realistic, the population
18 and the assessments in the treatment trial, the
19 experimental trial, and the natural history study
20 have to be equivalent and comparable.
21 I think that this provides a basis for
22 rethinking that progression from IND to BLA, and I

1 think it's really set on the foundation on planning
2 and natural history studies, and understanding the
disease, particularly in rare diseases, and what
4 effects and tools can be used. I offer this as a
5 new thought of how to think of the regulatory
6 framework of rare disease, starting early with
7 understanding the disease even before there is
8 potentially a specific compound for drug
9 development so that once there are potential
10 compounds available, the framework has been done
11 and a clinical trial can happen sooner and be
12 designed better.
13 The other thing, again, as Dr. Daniels
14 talked about this morning, is getting that patient
15 experience data to inform clinical endpoints to
16 ensure that it's the bothersome signs and symptoms
17 associated with the disease that are assessed
18 rather than symptoms that might not be as common or
19 as problematic, so that if a drug is effective, it
20 can be appropriately assessed on symptoms that
21 matter. Then along those lines also ensure that
22 the impact of the condition on functioning and

1 founder. I had a question. Our disease population has about eight patients across the world, so when you’re talking about a clinical trial for such a population, what would the control group be?

DR. HART: Yes. These are very much the challenges. Sometimes in that setting, we will use an external control group, so having that natural history, depending upon what it is and what the endpoints are, is a possibility. Sometimes there can be a delayed start. There can be a comparison within individual comparison. It really depends upon what the symptoms of the disease are, what the heterogeneity of the disease is, and really what is that natural history, because that can inform potential clinical trial designs. But it is possible.

MS. KRUSE: I'm Caroline Kruse. I'm from the Platelet Disorder Support Association. I am curious about biosimilars. It's my understanding that the FDA has approved 9 biosimilars, 7 in 2017. Are there any concerns about long-term data, interchangeability, labeling, and what role do you see biosimilars playing in the rare disease space?

Thank you.

DR. HART: Yes, biosimilars have a different pathway, as you alluded to, than new drugs. They are in between the ANDAs and the new drug. So there is a lot of potential and opportunity in that space. I am not the most appropriate person to speak about that space, though.

MS. FOXWORTH: Hi. It's Phyllis Foxworth again. Selena this morning shared that there would be guidance around the patient experience data, and I was just wondering if you can expand on how once that patient experience data is captured, what’s the process for getting it to the appropriate teams that do the drug reviews?

DR. HART: I wish Selena was still here to answer that, but there are a couple of different -- my understanding is that there are a couple of different pathways set up to do that, to make sure that that information is available, and there are different ways as far as sharing that data.

MS. KRUSE: Thank you. I'm just curious about biosimilars -- and they tend to be more of specialty physicians, rheumatology, GI -- or patient groups who might have a question as to what a biosimilar is and conflating biosimilar with generic. So I'm happy to follow up on that and specifically the issue of interchangeability.

In terms of also getting information to the review divisions, which is a great point, that's why we really need to have a coordinated process. And Selena and her team really are figuring out all those best strategies to get the information to the review division, but it also goes back to that point early on when I said to you we want to hear from you early on in terms of the drug development process.

Today we're focused on the FDA, but remember, there's really a continuum of drug development. And in many ways, the time for patients to engage on drug development is not only at the time when an NDA has been submitted and a decision has to be made whether to approve or not approve, it's very early on in the process, and...
1 talking to sponsors and working with sponsors in terms of what those endpoints might be, or working with the agency and trying to think through does there need to be guidance on what those endpoints are to foster drug development.

2 So that's why I encourage you to talk often and talk early to us, and we'll figure out for you how to get that information to the review division. And what I often like to say -- and Dr. Woodcock has joked the agency is full of introverts, and now we're trying to create a system that relies upon extroverts. And I guess I'm one of the few extroverts and I'm trying to hire extroverts.

3 But you also want to keep in mind the review divisions look at data, right? That's how they're going to make decisions. So how do we capture and package that experiential data in terms of a way that reviewers can use? How do we get it into that regulatory framework? Patient engagement really is an iterative process. I also want to say we're talking a lot about patients here, but remember, this is about all stakeholders who aren't sponsors.

4 At this time, I'm going to turn to my colleagues to find out whether we're going to do -- okay. We are going to do the clickers, and we're going to have Portia Seals, who's on our team at PASE, who tells me that she participated in the Olympic ceremonies at the Atlanta games. I'm just going to leave it at that. She told me not to expand on it.

5 MS. SEALS: The opening ceremony is not actually in the Olympics.

6 DR. WHYTE: Oh, participated in the opening ceremony. See I made it bigger; she participated in the Olympics. You kind of did; just the opening ceremonies, but that's still pretty good.

7 Audience Response Questions - Portia Seals

8 MS. SEALS: Question number 1, among the world's preeminent regulatory organizations, which approves new drugs the fastest? A, European Medicines Agency; B, U.S. Food and Drug Administration; C, Health Canada; D, Japan's Pharmaceutical and Medical Device Agency; or E, Australia's Therapeutic Goods Administration?

9 (Audience responds.)

10 MS. SEALS: Sixty-seven percent of the audience answered correctly with B, U.S. Food and Drug Administration.

11 Question 2, the FDA considers all of the following factors during the drug approval process except, A, biological markers; B, patient-reported outcomes; C, company stock prices; or D, clinical outcomes?

12 (Audience responds.)

13 MS. SEALS: And of course the correct answer is C, to keep ourselves out of trouble.

14 The next question, during a drug shortage, the FDA can, A, manufacture more drugs to meet demand; B, allow drugs to be imported from other countries; C, force a manufacturer to produce drugs; or D, none of the above?

15 (Audience responds.)

16 MS. SEALS: Wow. The actual correct answer is B, allow drugs to be imported from other countries. So there you learned something.

17 The last question is, the FDA determines the cost of drugs and whether insurance plans can cover these medicines, A, true; B, false.

18 (Audience responds.)

19 MS. SEALS: And the correct answer is B, false. Thank you.

20 DR. WHYTE: I'm going to be honest. I'm a little disappointed in those that did not say that the U.S. Food and Drug Administration approves drugs the fastest. And perhaps you're saying, "Well, John, you should have parsed it more carefully to say that, for the most part, the FDA approves drugs fastest in the world," because that is the truth, and we have lots of references to prove that, including New England Journal articles, if you need it. But again, today really is designed to help folks understand our processes and learn a few things about the FDA, and correct any misinformation that's out there.

21 One of the biggest challenges that we have
in talking to stakeholders -- and you're hopefully
probably also something you may not want to know,
getting the theme that we're very interested in
to really go into some of those issues around
our understanding on the issue is because there may
even tell you if we're planning regulatory action,
which can be very frustrating for people because
you often will come and have an excellent
presentation, and then you feel you're not really
going anything in return because we don't seem to
be responsive.
You shouldn't view that as we're not
interested, or that we don't care, but there are
circumstances where we cannot necessarily indicate
what is happening. And we do have to do a better
job of more effectively communicating that to
stakeholders so you don't leave with the impression
that we don't care, we're not interested, or we
don't agree.

Lieutenant Commander Sadhna Khatri, who
works with me and is a good friend and colleague,
is going to help tell you what CDER can and cannot
do by law; not what we don't want to do or want to
do, but again -- and this is a very important
conversation, and Dr. Woodcock alluded to it, that
we often don't explain to stakeholders that there
are these circumstances where we can't give you the
information perhaps that you would like.
Lieutenant Commander Khatri's fun fact is
that she participated at the White House with
Indian dance, as part of an Indian festival. So
it's with great pleasure that I call to this podium
Lieutenant Sadhna Khatri.
(Presentation - Sadhna Khatri
LCDR KHATRI: Dr. Whyte, thank you for the
introduction, and welcome to all of you who came to
attend in person at this beautiful White Oak
campus, and also to those on the Web. Welcome to
each and every one of you. My job this morning is
to tell you something that you want to know but
probably also something you may not want to know,
and to really go into some of those issues around
the limits of our ability to engage. So let's
start with CDER's mission.
CDER's mission is to promote and protect the
public health by ensuring that safe and effective
drugs are available to Americans. This is a very
succinct mission statement, but it encompasses a
lot of activities. CDER routinely consults with
American people in making its decisions about the
drugs that they use. It holds public meetings to
incorporate export and consumer input into its
decisions. The center also announces many of its
decisions in advance so that the members of the
public, academia, industry, trade associations,
consumer groups, and professional societies can
comment and make suggestions before decisions
become final.

In addition, CDER holds annual public
meetings with consumers and patient groups,
professional societies, and pharmaceutical trade
associations to obtain enhanced public input into
its planning and priority-setting practices. Over
the years, the policies have changed and laws have
become stronger, but the center's present and
future mission remains constant. That is to ensure
that the benefits of drug products made available
to the public outweigh all known risks.
Ultimately, patients are the focus of all CDER
activities, and we need to engage with them
First, let's start with some of the things
you really want to know, and that's where the
opportunities for engagement are. This has changed
over the last decade since I have been involved
with the drug development here at FDA. Patient
input is now playing an important and increasing
role in development and regulation of medical
products. A large number of patient activities are
in progress at CDER.

You can see on the slide the multiple
different opportunities for patient engagement with
FDA. You heard early this morning my colleague,
Chris Melton, mention about the external
stakeholder meeting request system, which the
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1 professional affairs and stakeholder engagement  
2 staff has launched recently. There exists your  
3 opportunity to request meetings with CDER.  
4 The next is the patient-focused drug  
5 development meetings. This is turning out to be  
6 perhaps the most effective and best way to bring to  
7 us patients' understanding and experiences of the  
8 disease. We have speakers on today's agenda who  
9 will be talking about the patient-focused drug  
10 development in detail, so I'm not going to go into  
11 detail with that.  
12 Next, we have the advisory committee  
13 meetings, and most of these advisory committee  
14 meetings do have a patient representative assigned  
15 to the AC meeting -- that's what we call it in  
16 short -- to present their point of view. Patient  
17 representatives are selected to participate in an  
18 AC meeting, and this is an opportunity for public  
19 dialogue. Patient representatives are considered  
20 government employees for the duration of the time  
21 they are serving on the AC committee.  
22 We also have public speaking sessions where  

1 many patients often take advantage and come and  
2 speak, but they often get about five minutes to  
3 make their point of view that is five minutes each.  
4 And if you have not witnessed or participated, or  
5 seen an AC committee meeting, there are a few  
6 recordings on our -- actually a lot of recordings  
7 on our website. I would highly recommend you to  
8 see that. It's a very neat process.  
9 Also, we often encounter patients at  
10 national meetings, such as NORD, the National  
11 Organization for the Rare Diseases, and we have  
12 lively conversations with patients, and we engage  
13 with them there. Sometimes patient advocacy groups  
14 also request to speak to us on an ad hoc basis, and  
15 we invite them here at FDA, and we schedule  
16 meetings with the review divisions where they come  
17 and express their point of view.  
18 Then there are citizens' petitions. Many  
19 patient advocacy organizations have the  
20 sophistication to submit to us a citizens’  
21 petition, which outlined a desired action that they  
22 would like us to consider on a point of view for us  

1 to consider. We carefully review those, and they  
2 often have a lot of legalistic aspects to them.  
3 Finally, we do put out notices in the  
4 Federal Register so that the public can be aware of  
5 some of the things we are doing such as the  
6 guidances. We do carefully review all the  
7 comments, sometimes thousands of comments, that  
8 come to us from the Federal Register notices often  
9 from patients and patient advocacy organizations.  
10 One of the most interesting developments for  
11 patient engagement has been in the development of  
12 guidances. The Duchenne muscular dystrophy  
13 community got together and put together a proposed  
14 guidance that they then submitted to the FDA, which  
15 we then reviewed and used as the basis of our own  
16 guidance on the development of drugs for Duchenne  
17 muscular dystrophy.  
18 We often receive lots of emails, letters,  
19 and phone calls. Sometimes the advocacy  
20 organizations seem to think that that is the most  
21 effective way, to bombard us with thousands of  
22 emails. While certainly it does get our attention,  

1 I can tell you that it's probably not the most  
2 effective way to be able to get your opinion across  
3 to us. And we are in the age of social media.  
4 FDA also has an FDA Facebook page where patients  
5 can engage with FDA and give their opinions.  
6 Earlier during the day, you heard Dr. Selena  
7 Daniels. She spoke about patient voice. Patient  
8 voice is important to us because patients bring  
9 insight to a disease. Patients provide insight on  
10 issues, problems, and/or questions that are  
11 important to patients and their family members. We  
12 also recognize not just one patient represents the  
13 entire patient community of that particular  
14 disease. Patients have a vested interest and  
15 diversity of opinions and varied perspectives both  
16 in terms of risk tolerance and potential benefits,  
17 so it's important for us to identify what matters  
18 and what is important for the patient. This will  
19 help us in the development of clinical trials that  
20 are meaningful and realistic, and will raise FDA's  
21 awareness.  
22 Dr. Whyte early on mentioned that the
FDA desires to be transparent, but often can’t because of the law. We do want to have a dialogue. We want to hear from you, but often when we are talking to you, we are constrained, and it’s very uncomfortable for us because we really want to be able to talk back, but we can’t, and that’s predominantly because of the law. I met a couple of you during the break, and we were talking about some things. I was restricted in giving my answers because of the law, again, and I did express that. We do operate under strict laws regarding confidentiality in regards to our knowledge, opinions, and what we are saying and discussing with sponsors during drug development and the review process because that’s a very confidential relationship that we have with the sponsor during that period of time.

This greatly restricts our ability to discuss or even mention the existence of specific products that are under review or development during that period of time. And I know sometimes it frustrates the patient community that we can’t directly tell them what we are thinking and what we think needs to happen next, or what we even think of what has happened so far, but the whole reason is that it’s really designed to protect the sponsor, and this is congressional action and law. It’s to protect their commercially sensitive information. Correct? But sometimes even within small disease groups, we find that there are patient advocates who have one strong view versus another group of patient advocates that have another strong view. So we have to be very careful in listening to both the views and try and incorporate those views into our thoughts. Finally, there is this area of bias. If we see the patients coming in who are paid and selected by the sponsors to present their point of view to us, believe me, we are aware of that. So we also carefully examine that into what we hear and what we know, being a very selected point of view that we may be hearing.

As much as we listen and as much as we try to incorporate -- and I think you will hear a lot more details when they discuss about the patient-focused drug development meetings, we really do value what we hear, but we can’t always follow what we hear, and we don’t always follow. We have to act still in an independent manner, and part of that can be due to the fact that the law
1 may not allow us to do what you are recommending us
to do.
2 You’ll be surprised that we even sometimes
to get phone calls from Congress sometimes telling us
to do things, and we say, hmmm, I don’t think
that’s legal, and we sometimes can’t always do
that. So we may also have a real difference of
opinion on the interpretation of the underlying
facts. You may or may not be aware, but in fact,
if you look at the medical and scientific published
literature, less than half of it can be reproduced,
so you can’t always believe everything you read,
even in a medical journal. It doesn’t always turn
out to be quite the truth.
3 FDA is the only regulatory organization in
the world that looks at the actual data. For
example, in Europe, they often will just look at
the summarizations that were given to them by the
sponsors. Here we say, in God we trust; everything
else, bring us the data, and we are going to take a
very good look at it. So we may have differences
and views on the practicality of the
recommendations that are made to us, or as I
mentioned before, conflict with the laws or
regulations, maybe not in a way that makes it
illegal, but what introduces a very different legal
risk.
4 Finally, the last two, there can be an
inconsistency with the recommendations in our
entire policy position or previous decisions. Now,
that doesn’t mean that we can’t change our policy.
5 It does not mean we can’t diverge from our previous
decisions, but we cannot do that lightly because
that would not be fair or consistent. So when we
do make a change, it has to be very, very carefully
considered and well supported.
6 With that, I’ll conclude my presentation,
and again, your recommendations, patients'
recommendations are very valuable to us, but we
always can’t follow or do what you’re recommending
us to do. Thank you very much.
7 (Applause.)
8 LCDR KHATRI: My email address is here.
9 Please feel free to emailed me if you have any

1 questions. We also have a mailbox for PASE, which
is cderpase@fda.hhs.gov. You can email us there.
2 I will be also here for the entire day, and if you
have any questions, please feel free to stop me.
3 Thank you.
4 Do you have any questions
5 Questions and Answers
6 MS. NIZAR: Thank you so much. All that
information was amazing. I just had a doubt.
7 We’re a rare disease organization, and as I
mentioned, a very small one. You mentioned the
ESMR. You mentioned the PFDD, the ad hoc, the FDA
meetings, the advisory committee, the patient rep
program. Now, basically my question is, is there
like a flowchart. Step 1, where do we go? Step 2,
where do we go?
8 LCDR KHATRI: No, there is no flowchart or
any sequence in which you should go step 1, step 2,
or step 3. Those are the different options for
patient engagement, depending upon what works best
for you. So as Chris mentioned earlier and showed
you, the external stakeholder meeting request
system is an online system, and you will be able to
request a meeting with FDA through that system.
2 It’s a centralized process. It’s a very noble
approach, and this is the first time PASE has done
it here at CDER. So if you request your meeting,
then we will be able to triage your request and
connect you with the right people.
3 MS. NIZAR: What’s the turnaround time for
replying?
4 LCDR KHATRI: Seven days we respond to your
request. It’s very easy form.
5 MS. NIZAR: Is it similar to the pre-IND
request?
6 LCDR KHATRI: I think our form is very
simple, and I can talk to you after the meeting or
doing lunch, and really walk you through the
process.
7 MS. NIZAR: Okay. Yes, I really appreciate
that. Also, I just wanted to mention, sometimes
it’s not always easy to attend these meetings. We
took like an hour and a half, maybe two, to
counter get here because the roads weren’t
accessible. We were literally on the road driving in a wheelchair because we couldn't find accessibility to get here. So it's not always very easy, so email is really our last resort. And sometimes it's not clear on your website either like who is the person that we need to contact, so we're sending like blind emails to people hoping against hope that someone will reply. Thank you.

LCDR KHATRI: First of all, I'm sorry that you had to go through that much trouble to travel to the White Oak campus, but this meeting is also on the Web, so it's easily -- people can attend through Web as well. And we do understand the traffic around the Silver Spring area and just coming to the White Oak campus. So we do understand all that.

Regarding the emails you mentioned, I gave you two emails, so if you have any questions, we have two emails. You can personally contact me, and I will also give you my card. But it is also on the slides, which will be posted on our website.

Also, PASE has a central email, which is monitored every single day. So every hour we would say -- and we are very quick in responding to any emails which we receive. MS. NIZAR: Thank you so much. I appreciate it.

LCDR KHATRI: You are welcome.

DR. WHYTE: I think your point is very good about people send emails blindly, and that was the whole impetus, in a way, to create this centralized process, that you don't need to know who you need to contact. You just go to fda.gov/requestameetingondrugs. So it's a fair point, and we acknowledge that it's not easy to navigate literally and figuratively the FDA.

So we still have work to do. It's iterative steps along the way. And I want to thank Lieutenant Khatri for that presentation in terms of sometimes it's hard for us to tell you when we can't say things. I liked your line when you said sometimes we'd like to talk back. She means in a good way, the talk back. Some other days may be different.

Are there any other questions that people have? And then we're going to break for lunch.

MS. KERKORIAN: You mentioned --

DR. WHYTE: I've been sitting next to you. You could have just leaned over. I know, leaned over.

MS. KERKORIAN: -- I know, leaned over.

You mentioned the turnaround time for responses to emails, but what is the turnaround time once you've identified the right person? Is there a turnaround time or a ballpark in terms of how quickly a meeting can be scheduled for planning purposes?

DR. WHYTE: We're really trying to accommodate stakeholders. What I focus on with my team is we're here for the stakeholders and how do we make it easy for stakeholders. It is kind of changing the mind-set. And for those of you that have been at meetings here at the FDA, we tend to travel in packs. So if you come to a meeting, we may often outnumber the number of attendees you have; that it's 20 people in the room. If

Dr. Woodcock comes, it's 50 people in the room, and that can be a challenge to schedule.

So we really do a couple of things. We want to ask the requesters -- sometimes people have a set time period that they want because they're going to be here in the D.C. area for other reasons, so we try to accommodate that as best as we can. And I will tell you that since we've launched, we've had some meetings that have been scheduled and already have taken place within two or three weeks. Other meetings are already set out for a couple of months, and there are a couple of reasons why that is.

Often folks want to assemble as many people as they can. And let's be realistic; everyone's not in the D.C. area, so folks have to fly in, and it can be expensive to make a flight at the last minute. And then depending upon the level of the meeting, it can be a challenge scheduling. But we're really committed to this idea of one or two months to really be able to get a meeting. I know that might seem long to some people,
and remember, this is an iterative process that we're trying to be as responsive as we can. We're really trying to explore the idea of WebEx and conference calls. I've talk to a couple of people at the break, and I mentioned it in my remarks. I just find there is this culture of meetings that people physically want to come and meet. And we're fine with that and embrace that, but sometimes that can be challenging to schedule everyone. WebEx can be productive, too, and conference calls. So it's just really trying to consider multiple approaches.

We have another question in the back, my biosimilars friend.

MS. KRUSE: I just wanted to say that our organization had a meeting back in November with OHOP, which is the Office of Hematology and Oncology Products. I don't mean to set the bar too high, but I had sent an email to Rea Blakey in PASE, and within 10 minutes, I received a response and worked directly with Lieutenant Khatri. And she was so wonderful and stayed in contact with me every step of the way. And if I didn't follow up, she sent me a reminder email saying "Hey, Caroline. You need to send those slides to me." But it was really a wonderful process to go through --

DR. WHYTE: That's great to hear.

MS. KRUSE: -- and was not complicated in any way, and just a very, very quick response on the part of the FDA. So thank you for that.

DR. WHYTE: And nobody paid you to say that, I feel like we have to disclose.

(Laughter.)

DR. WHYTE: That's great to hear.

MR. BARTEK: And this is another unpaid solicitation.

DR. WHYTE: Okay. Oh, whoa. Let's keep them coming.

MR. BARTEK: Just a quick comment. With all the discussion about how to request a meeting and how important it is to have meetings, one thing that hasn't been terribly emphasized is the importance of meeting with your sponsor, with the pharmaceutical company at meetings that they request and are on their schedules, like pre-INDs meetings, after phase 2 meetings, and so forth. And the FDA can't invite us as patient advocates or patients to those meetings.

The sponsor has to do that. But you can go to a pre-IND meeting to represent your patients, and you can become a very important aspect of the conversation with the review divisions at those and other scheduled meetings with sponsor.

DR. WHYTE: That's a very good point. And as you know from most of those meetings, the sponsor has to allow it, and the sponsor may or may not allow it. We have often stated, and Dr. Woodcock herself has stated, that there is nothing that precludes patients or other persons from attending these meetings, but they are the sponsor's meeting, so folks would have to be guests of the sponsor. We cannot include -- force that participation.

I think that's a very good point. And again, it also goes to the idea that patient engagement and stakeholder engagement is along the continuum of drug development. It's not just at that time when an NDA, a new drug application package, is before the agency. And I think that's a very important point, but it's also a good point to emphasize that it can often be hard for individual patients, for caregivers, to get through to a sponsor, to get to that point to say this is important to me, and we need to think through those strategies as well.

Any other questions?

(No response.)

DR. WHYTE: All right. So we're going to break for lunch. When we come back, we'll start promptly at 1:00. We are going to have a fun 30 minutes, not that these last few hours haven't been fun. But we're going to break up into four groups at the front, where we're going to play FDA Jeopardy, and we're going to test your knowledge.

There are no prizes other than your bragging rights.

Technically, I don't think I'm allowed to call it Jeopardy. I think it's licensed, but you
AFTERNOON SESSION

(1:01 p.m.)
CDER Jeopardy

DR. WHYTE: Are we ready to play some Jeopardy? Are we ready to play some Jeopardy?

Woo-hoo! Okay.

I still need two volunteers. Is that right, Noah? Two. I need two more volunteers. Don't make me choose you.

Let's get the teams ready. Am I reading these off, Noah, or you? Team 1, Katy Riddick; Kevin Healy; Hiren Gadiya; Janay Johnson; Jim Bender; Alana Broe. Where are you?

Team 1? Which one is Team 1 up here? The clicker has to be Team 1. One person will sit here, and the others will go around each team so you can work together to determine what answer you're going to pick.

Team 2 is Nadia; Neena; Dave; Calvin; Cara; and Anne Marie [ph].

Team 3 is Alysa [ph]; Leyla; Pam; Bill; Brigid; and one more option.

going to give you clues that you'll respond to, remember, in the form of a question. Remember that. You have to answer in the form of a question. There are 5 categories, 100 to 500 points, 25 questions in all. The winning team obviously will be the team that after the final Jeopardy has the most points. Remember you're penalized if you don't answer the question right.

Now, here's the important point. We've done this for a few years, and I say this. I say it like five times, and it still doesn't work. The way that we're going to play it is you have to let me read the whole question. So don't be doing it while I'm talking because it won't work.

(Laughter.)

DR. WHYTE: You have to wait until I've done the question, and then you click, because we have tested these. They all work, and I know everyone will want to say it doesn't work. It does. It's the FDA. It's like a device.

(Laughter.)

DR. WHYTE: Are we operational? It is
randomly determined who will go first. But here are the categories: Acronym Soup; Drugs and Biologics; Play It Safe; Trials and Tribulations; and the Advocacy Cheat Sheet. So randomly selected is Team 4. This is why you need to be near each other. Team 4, Play It Safe for?

TEAM 4: Five hundred.

DR. WHYTE: Going big. All right. Play It Safe. Remember, wait until I finish reading it. "The FDA can require manufacturers provide the safety strategy to manage serious known or potential risks associated with medicines and manage their use so that patients can continue using them."

Now let's see how smart you all are. Team 1, in the form of a question.

TEAM 1: What is risk evaluation and mitigation strategy?

DR. WHYTE: What is risk evaluation and mitigation strategy? That is correct, REMS.

TEAM 1 choose again.

TEAM 1: We would like to Play It Safe for 300.

DR. WHYTE: Play It Safe for 300. This phase of the regulatory process occurs after the FDA has approved a drug or biologic product for marketing in the U.S. The FDA monitors these products to detect serious, unexpected adverse events and take action when necessary. Team 4?

TEAM 4: What is postmarket surveillance?

DR. WHYTE: What is postmarket surveillance. I'll accept that. Yes, that's correct or it could be phase 4.

TEAM 3, choose again. I'm sorry. That was Team 4 I apologize.

TEAM 4: Acronyms for 200.


TEAM 1: Acronym Soup for 200. Team 1?

DR. WHYTE: That is correct. What is new molecular entity? Choose again.

TEAM 1: Acronym Soup for 300.

DR. WHYTE: Acronym Soup for 300. PDUFA?

Team 2 is on the board, maybe.

TEAM 2: Prescription drug user fee.

DR. WHYTE: In the form of a question. TEAM 2: What is prescription drug user fee?

DR. WHYTE: That's correct. Remember, we're rules followers here. Okay, Team 2, choose again.

TEAM 2: Advocacy Cheat Sheet for 200.

DR. WHYTE: Advocacy Cheat Sheet for 200. These three public seminars welcome patients, caregivers, and other members of the public to present data, information, or viewpoints on issues pending before the FDA committee. Team 4?

TEAM 4: What are advisory committee meetings?

DR. WHYTE: No -- you know, what I'm going to allow it. Let's give it to them. It's really, "What are FDA sponsored public meetings." Advisory committees are one of those types of meetings, so we're going to be lenient.

Team 4, choose again. Team 3, you've got to get ready with your clicker.

TEAM 4: Acronym Soup for 100.

DR. WHYTE: Acronym Soup, playing it safe,
<table>
<thead>
<tr>
<th>Page 133</th>
<th>Page 135</th>
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<tbody>
<tr>
<td>1 DR. WHYTE: What is the Office of New Drugs?</td>
<td>1 of biomedical research and may be used for conditions that lack other available treatments.</td>
</tr>
<tr>
<td>2 That is correct.</td>
<td>2</td>
</tr>
<tr>
<td>3 Okay. Team 1 is in the lead with 1300; followed by Team 2, 700; Team 4, 500; and Team 3 is getting ready. You’re getting ready. I don’t know if it’s the device or the users.</td>
<td>3 It says Team 4.</td>
</tr>
<tr>
<td>4 TEAM 1: Drugs and Biologics for 300.</td>
<td>4 TEAM 3: What is biologics?</td>
</tr>
<tr>
<td>5 DR. WHYTE: Drug and Biologics for 300. I work on drug issues. These types of drugs fill most of the prescriptions in the United States.</td>
<td>5 DR. WHYTE: You’re Team 3.</td>
</tr>
<tr>
<td>6 Although they typically cost less than their brand name counterparts, they’re equivalent in terms -- wait till I finish -- in terms of quality, performance, strength, and safety.</td>
<td>6 (Laughter.)</td>
</tr>
<tr>
<td>7 Team 3? No, that’s not right. Which team is it?</td>
<td>7 DR. WHYTE: No, you’re Team 2. It’s Team 4.</td>
</tr>
<tr>
<td>8 TEAM 4: What are biologics?</td>
<td>8 TEAM 4: What are biologics?</td>
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<td>9 DR. WHYTE: What are biologics is correct.</td>
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<td>10 I know, I thought it did, too, but I have to do what the computer says.</td>
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<td>11 Okay. Team 1 is in the lead with 1300; followed by Team 2, 700; Team 4, 500; and Team 3 is getting ready. You’re getting ready. I don’t know if it’s the device or the users.</td>
<td>11 what the computer says.</td>
</tr>
<tr>
<td>12 TEAM 1: Drugs and Biologics for 400, please.</td>
<td>12 Okay. Team 4?</td>
</tr>
<tr>
<td>13 DR. WHYTE: Also known as the prescribing information or package insert, this informative communication provides healthcare professionals the necessary information to appropriately prescribe drugs for safe and effective use. Team 1?</td>
<td>13 TEAM 4: Drugs and Biologics for 200.</td>
</tr>
<tr>
<td>14 TEAM 1: What are generic drugs?</td>
<td>14 DR. WHYTE: These drug products are safe and effective for consumers to use without a doctor’s prescription. Team 3? See, it works.</td>
</tr>
<tr>
<td>15 DR. WHYTE: What are generic drugs. Okay.</td>
<td>15 TEAM 3: What is over-the-counter drugs?</td>
</tr>
<tr>
<td>16 You’ve got to wait until I finish talking. I was watching. I was hopeful. What are generic drugs?</td>
<td>16 DR. WHYTE: What are over-the-counter drugs?</td>
</tr>
<tr>
<td>17 TEAM 1: Drugs and Biologics for 400, please.</td>
<td>17 That’s correct. Okay!</td>
</tr>
<tr>
<td>18 TEAM 1: Drugs and Biologics for 400, please.</td>
<td>18 (Applause.)</td>
</tr>
<tr>
<td>19 DR. WHYTE: Let's do Drugs and Biologics for 100, please.</td>
<td>19 DR. WHYTE: Woooo! Now we've got a game going. Come on, Michigan! Trials and Tribulations for 300. This entity seeking to market a drug is responsible for its development and proving it's safe and effective. Team 2?</td>
</tr>
<tr>
<td>21 We’ll give it to you. It's usually what is the drug label or what is the packaging prescriber information. But we'll give that to them. What is the prescription drug labeling information? It goes by a couple different terms.</td>
<td>21 DR. WHYTE: What's a drug?</td>
</tr>
<tr>
<td>22 TEAM 1: Drugs and Biologics for 500.</td>
<td>22 DR. WHYTE: What is a drug? That's correct.</td>
</tr>
<tr>
<td>23 DR. WHYTE: These products include vaccines; human blood and blood components; human cells; gene therapy; and tissues. Gene based and cellular products within this category are at the forefront</td>
<td>23 Okay. Choose again.</td>
</tr>
<tr>
<td>24 TEAM 1: Drugs and Biologics for 500.</td>
<td>24 TEAM 3: Let's go Advocacy Cheat Sheet for 300.</td>
</tr>
<tr>
<td>25 DR. WHYTE: These products include vaccines; human blood and blood components; human cells; gene therapy; and tissues. Gene based and cellular products within this category are at the forefront</td>
<td>25 This FY 2013 to 2017 initiative seeks to gather patient perspectives on their conditions and available treatment therapies in a more systematic way to better inform drug development and</td>
</tr>
</tbody>
</table>
1 evaluation process.

2 Team 4?

3 TEAM 4: What are patient-focused drug developments?


5 TEAM 4: Advocacy Cheat Sheet for 500.

6 DR. WHYTE: Advocacy Cheat Sheet for 500.

7 Wooooo! Okay. You could either take the lead or end up in last place. How much you want to bet?

8 MALE PARTICIPANT: Bet it all!

9 DR. WHYTE: You're going to bet it all?

10 TEAM 4: Everything?

11 DR. WHYTE: Bet it all, 1300, the whole thing. Wow! High risk, high reward.

12 This organization engages with stakeholders, including patients, advocates, and healthcare professionals, to improve their understanding in how the FDA approves and regulates drugs.

13 TEAM 4: What is the P-A-S-E?

14 DR. WHYTE: What does it stand for?

15 DR. WHYTE: What are what?

16 (Laughter.)


18 All right. You're in the lead. Wow!

19 TEAM 4: Advocacy Cheat Sheet, 400.

20 DR. WHYTE: Okay. This program helps consumers and healthcare professionals better understand who takes part in clinical trials by providing them with demographic data on the trial participants for FDA-approved new molecular entities.

21 TEAM 3? Dr. Woodcock mentioned it.

22 TEAM 3: What are [inaudible - off mic]?

23 DR. WHYTE: I'm going to give it to you. It's what are drug trial snapshots? But close enough. Okay. Choose again. Anybody could win it.

24 TEAM 3: I'm still going through a hell of a trial. Trials and Tribulations for 500.

25 DR. WHYTE: Okay. Trials and Tribulations for 500. Also known as compassionate use, this practice refers to the use of an unapproved investigational medical product outside of a clinical trial.

26 Team 1?

27 TEAM 1: What is expanded access?


29 TEAM 1: We're going to Play It Safe for 200, please.


31 These entities are required to report adverse drug events to the FDA.

32 TEAM 1? It's multiple answers.

33 TEAM 1: What are PADARS [ph]?

34 DR. WHYTE: What are what?

35 TEAM 4: Advocacy Cheat Sheet, 400.

36 DR. WHYTE: That's better. Okay.

37 (Laughter.)

38 DR. WHYTE: What's MedWatch? Where did you pull that out of all of a sudden? That's correct. We'll accept it. I think you were thinking FAERS.

39 All right. Choose again. You're back in the lead.

40 TEAM 1: Trials and Tribulations for 200.

41 DR. WHYTE: This phase of clinical trials is typically the final phase before approval and involves human subjects to establish the safety and effectiveness of a drug. I love how I say you cannot click beforehand, and while I'm reading, it's clicking.

42 (Laughter.)


44 TEAM 4: What is phase 3?

45 DR. WHYTE: What is phase 3? That's correct.
1. Let's just go over, Team 1, 2900; Team 4, 2800; Team 1 [sic], 2000; and Team 3, 900. Anybody in theory could win, in theory. Okay. Let's go.

2. TEAM 1: Participation in advisory committee

3. DR. WHYTE: Participation in advisory committees. That's one of the answers. That's correct. What is participation in advisory committees?

4. MALE PARTICIPANT: Do we get credit [inaudible - off mic]?

5. DR. WHYTE: Participation in advisory committees.

6. TEAM 4: Trials and Tribulations, 400.

7. DR. WHYTE: Trials and Tribulations, 400. That's correct. What is participation in advisory committees?

8. MALE PARTICIPANT: Do we get credit [inaudible - off mic]?

9. DR. WHYTE: Trials and Tribulations, 400. That's one of the answers. That's correct. What is participation in advisory committees?

10. TEAM 1: Participation in advisory committee meetings.

11. DR. WHYTE: Participation in advisory committee meetings. That's one of the answers. That's correct. What is participation in advisory committee meetings?

12. TEAM 1: Participation in advisory committee

13. DR. WHYTE: Participation in advisory committees. That's one of the answers. That's correct. What is participation in advisory committees?

14. TEAM 1: Participation in advisory committee meetings.

15. DR. WHYTE: Participation in advisory committee meetings. That's one of the answers. That's correct. What is participation in advisory committee meetings?

16. TEAM 1: Participation in advisory committee meetings.

17. DR. WHYTE: Participation in advisory committee meetings. That's one of the answers. That's correct. What is participation in advisory committee meetings?

18. TEAM 1: Participation in advisory committee meetings.

19. DR. WHYTE: Participation in advisory committee meetings. That's one of the answers. That's correct. What is participation in advisory committee meetings?
DR. WHYTE: This is exhausting.

All right. Time up. Team 1? Don't show the answer yet. We're doing it like the real Jeopardy.

(Mr. Goetzel explains procedure.)

DR. WHYTE: Okay. We'll start with Team 3?

All right.

Team 3, what's your wager? Oh, you wagered -- what's your answer?

TEAM 3: Sixty-seven percent.

DR. WHYTE: I'm going to let us see all the answers, and then we'll do it that way. Because otherwise, then the others won't know. Okay, 67.

Team 4, what's your answer? Sixty.

Team 2?

TEAM 2: A little different, 5 percent.

DR. WHYTE: Five? No, that's not right.

(Laughter.)

DR. WHYTE: You guys have a lot to learn.

TEAM 1?

DR. WHYTE: Is that what you wrote down?

Okay, 44.

And the answer?

MR. GOETZEL: So what did Team 3 -- did they get it right?

DR. WHYTE: They said 67. Oh, we don't have the slide with the answer? We're not showing them the answer? I want to look at my notes. The answer is -- and someone is going to win by only off by 1 percent -- 61 percent.

(Applause.)

DR. WHYTE: And I believe Team 4 said 60.

TEAM 4: Yes.

DR. WHYTE: All right. Team 4 is the winner. Congratulations. Let's give them all a round of applause.

(Applause.)

DR. WHYTE: Team 3's still saying those didn't work or something. But good job, everyone.

Good job.

With that, we're going to talk about -- we have numerous committees and panels to obtain independent expert advice, and if you attended some of these meetings or you look online, a lot of people talk about the docket. And you might be thinking what's a docket, where is the docket, what does that mean? And we often say submit your comments to the docket.

We're going to hear about how do you rock the docket from John Wright -- not John Whyte, John Wright -- from the Division of Dockets Management.

We actually have a docket's management division in the commissioner's office. And a fun fact about John is that he's been in the coldest -- the Alaskan interior in February -- and the hottest -- Death Valley, California in July; it really should be the opposite, John -- in one year's time. So we will hear all you need to know about submitting comments to the docket.

Presentation - John Wright

MR. WRIGHT: Well, good afternoon, everyone.

I don't know how much you know about music history, but this is kind of like the Monkeys trying to follow Hendrix. So we'll see what we can do. Oh, yeah, the temperature thing. That was about 20 years ago. I was in the military. The cold part was because they made me. The hot part was my choice.

So what do we do at dockets management? I'm going to go through these and answer as many of your questions as possible. But the biggest thing to remember is we do everything. If there's an administrative question for the FDA, it comes to us, and we read it and we determine where it goes.

That includes everything from tin cans, to laser beams, to drugs. In fact, I have spent a great deal of time in the past week on the standard of identity for tuna fish believe it or not.

We are made up of three teams. First of all, the acronym's at the top. The OCOES, that's Office of the Commissioner, Office of the Executive Secretary, Division of Dockets Management. I hope you know what FDA means. DDM is three teams. We've got the D&D team. That's Dockets and Documents. They handle mostly tobacco dockets, which include things like civil money penalties for selling to underage people, and the Public Reading
Room. The Public Reading Room does a live comment management, and they handle walk-in visitors. All of you are welcome to be a walk-in visitor should you need to submit anything to Dockets. But the most important team is mine just because we do most of the stuff that requires contact with people outside of our office. So that's the biggest thing to remember.

This is a small list of things we do. Here it says, "Petitions to the government." Now that can take many forms. Typically, what we see from your community are petitions related to drugs or abbreviated applications for drugs and things of that nature. Sometimes we see advisory petitions. For example, I got one from the country of Spain asking about the identity of a cheese and wanting our advice and things like that.

How many of you read the Federal Register regularly? Good. We're responsible for getting the things from the FDA to the Federal Register. For example, if you look in the FR and you get an invitation to come visit the FDA for an advisory committee meeting, that will have been submitted to the Office of Federal Record by us.

If you want to ask the FDA to do something, and it is administrative, what that means is the answer for your question or the action the FDA takes is already decided by the law. Then you can send us a petition, which will compel the FDA or ask the FDA, depending upon the law you are citing, to do or not do something about a drug.

The most common types of petitions that I see are for suitability petitions. For example, somebody will want to market a generic drug, and they won't want to go through the whole, long process. So they'll find another drug that's really similar, and they'll say, "Hey. Let me use this drug," and they'll petition us for that sort of thing.

We also keep records of all these things going back until I think 1957, and many of these are still active. In fact, some CDER dockets dating back to 1973 are still open. And those things will forever be open because the particular ones I'm thinking of involve drugs that are recognized as generally safe and not going to bother anybody. Well, every time that gets challenged or questioned, something gets added to that docket, so they will never close.

Also, we do information requests. Right here, it says "FOIA requests," but not all of our requests are FOIA. Some of them are far less laborious. For example, if you just walk into the office, you can take care of a FOIA request in 20 minutes instead of 20 days because we are pretty responsive, and of course we handle comments. Now, when I say "comments," I'm talking about every single comment for every single activity the FDA might do on a given day. Now, if you can imagine what CDER, just CDER, does in a day, it's quite a bit. There are 13 centers like CDER, and they all do a lot. An example is I think Friday -- no, it was last week, the docket for flavoring in tobacco was opened, and it garnered 3,000 comments in two days, and there are two people that manage those. So you can imagine we've got a lot of things that we have to do, but that said, we are still extremely responsive.

Now, petitions, this is what I was just discussing with you here. Some of you may want to do this; some of you won't. But if you do want to submit a petition, you can call us first. Before you go to all the work, if you've never done a petition before, please just call us. A lot of your entities are very small and you can't necessarily afford a regulatory counsel or an attorney, we will help you.

For example, we will give you copies of these regulations. 1020, that's generally administrative regulations, what has to be on a piece of paper for the FDA to read it. Right there where it says, "content," that's what everybody looks at, at a citizen petition, the first time it comes to our office. They do not care what it's about. It can be, hey, this petition is going to save the world. All we care about is that right there.

So you need to call us, and we will help you.
set it up so that it gets accepted without any
difficulties. That includes more complex things.
For example, if you want to tell the FDA to stop
doing something, it requires additional steps that
we will walk you through.

Now, back to comments here, you may be very
interested in comments once you open a docket. A
lot of times a special interest group, for example,
may have an issue for which they've submitted a
petition, and that issue may garner a lot of public
interest that that group may not be aware of. So
it's an opportunity for them to gauge public
interest, public opinion, and things of that
nature, so the comments are very, very important to
us.

Furthermore, we actually do read them all.
The comments are collected, deduplicated,
categorized, and sent directly to the human beings
who actually make the decisions, so they're not
wasted. And that includes comments that may be
submitted electronically or submitted by very
concerned citizens who do it 10, 15 times a day.

We still read them all. And believe me, there are
many, many citizens who are very passionate about
their voice being heard.

Also important to note, if you submit a
comment, it will be public. The only time we do
not post comments publicly is if they are
specifically stated as confidential. That includes
everything that you might send to Dockets
Management. It will be posted in public. If you
want it to be otherwise, again, please call, and we
will make arrangements for you to be able to do
that. That is very important because stuff will
just get posted automatically otherwise.

This is the very, very important screen
right here just because I like it, and it's made
out of -- I think they're Morgen. Anyhow, this is
where I want you all to get your pens and papers
out because you actually get our phone number and
email, and I want you to use them. The main phone
number, the 402-7500 number, that is a number to
the public reading room, and they can direct you to
pretty much anywhere in the FDA. They're very,
very helpful for a non-public affairs office. That
said, they cannot speak for the FDA. They will
route you to the right person. A couple of people
in my office are me -- and that's me -- and of
course, Dynna Bigby. We are always available. We
check our emails compulsively, things like that.

Do you have any questions? I'm going to
back it up here in case you need to make notes.

Any questions?

MR. WRIGHT: Wow! I must have been
through. That's excellent. Well, thank you very
much. Again, if you do have any questions or if
anything comes up and you can think of a way that
we might be able to assist you, please just let me
know.

(Applause.)

DR. WHYTE: Well, thank you. Now we're
going to hear from a panel of my FDA colleagues,
and Rea Blakey is going to introduce them. Rea is
the communications policy strategist and engagement
team lead at PASE. And a fun fact about Rea is
that her name -- R-E-A -- was once used as the
answer in a New York Times crossword puzzle. The
clue was CNN medical correspondent, and I'll allow
Rea to introduce my colleagues. And I'm just going
to say, the last time I saw two of my colleagues
was at a snowstorm in Philadelphia, and they left
me there. I had to come back the next day. So
nice to see you again, Andrea and Pujita.

Discussion Panel - Rea Blakey

MS. BLAKEY: Oooh. I think the young kids
call that a burn. Yikes!

(Laughter.)

MS. BLAKEY: Well, let's not be so formal,
ladies. Come on up, and I'll introduce you once
you get up here. There are four panelists. We
have one other member who is right now involved in
a conversation with the commissioner. She will be
joining us, hopefully before we all conclude. But
I think you're really going to enjoy the
presentations from these first three ladies.

We tend to work a lot together because we
all work generally in the same kind of space that
1 has to do with patient engagement. I'm in
2 Professional Affairs and Stakeholder Engagement,
3 but of course across the FDA are other offices and
4 agencies that work in the same general space and
5 then have some other offshoots of things that they
6 do.
7 So we're going to discuss some of that today
8 and also talk about what we hope will be an
9 interesting future in regards to patient engagement
10 in general. Obviously, transparency is a major
11 issue for us not only here at CDER but across the
12 FDA, and really if you think about it, throughout
13 the entire government. The public deserves to know
14 what's going on. We really try to address that.
15 Certainly at PASE, you've heard about the
16 request a meeting on drugs opportunity that you
17 have. If you send in your requests, they will come
18 to my office, and we will triage them, and we will
19 do our best to make sure that you get your voice
20 heard. But just in case, there are other avenues,
21 and that's really what this panel discussion is
22 about today, the other avenues that could be

1 available for you to get your voice heard because,
2 again, we want to hear from you. We want you to
3 have an informed opportunity to inform our process
4 because, ultimately, we work for you.
5 So thank you again for coming. I probably
6 should have said that first because it's important
7 that you're in the room with us to know that we're
8 working on your behalf.
9 I will introduce each of our panelists just
10 as they're about to give their presentations. I
11 will start with Pujita, who I have to say I'm
12 curious about the snowstorm story, but if you don't
13 have time, we'll let it go, but maybe you could
14 fill us in a little bit. Pujita is the acting
15 director of Decision Support and Analysis Team, and
16 that's in the Office of Strategic Programs.
17 Pujita, welcome.
18 Presentation - Pujita Vaidya
19 MS. VAIDYA: Hi, Rea. Thank you so much.
20 As Rea mentioned, I'm in the Office of
21 Strategic Programs. And I know I forgot to send
22 you a fun fact, but I've come with one. So a few

1 months ago, I was actually in Switzerland and went
2 to the top of Mount Titlis in the Swiss Alps. And
3 there, actually they have the highest elevation
4 suspension bridge in Europe, so it's over 10,000
5 feet up there. So I walked across that.
6 Definitely, it was a breathtaking view, but my
7 heart was pounding. And I am afraid of heights as
8 well, so that makes it even worse, but it was
9 great.
10 I'll be talking to you about FDA's
11 externally-led, patient-focused drug development
12 meetings and the opportunity for stakeholders.
13 (Brief pause.)
14 MS. BLAKEY: In a previous life, I would
15 have said that happens on live TV, however I hope I
16 put the batteries in the right way. I feel a buzz.
17 I think it's happening. Let's test it out. Sorry
18 about that.
20 Before I get started and jump into our
21 initiative, I just want to talk about let's define
22 what patient-focused drug development is. We're

1 not thinking about FDA's initiative, but in
2 general.
3 Patient-focused drug development, as we
4 think about it here and as we define it, is a
5 systematic approach to help ensure that patients'
6 experiences, perspectives, needs, and priorities
7 are captured and meaningfully incorporated into
8 drug development and evaluation. This is a
9 definition we're really moving forward with. It's
10 a definition that we've included in a glossary that
11 we're coming out with in June. It's going to be
12 part of that glossary, and really, this is the
13 essence of it.
14 So keeping that definition in mind, I want
15 to talk really briefly about FDA's patient-focused
16 drug development, how it came about, and then jump
17 into that externally-led piece. Back in 2012, we
18 in the FDA recognized the need to systematically
19 collect the patient's perspective. Patients are
20 experts in their disease, and they have a very
21 unique opportunity and way to provide their input
22 that could inform drug development.
So keeping that in mind, from 2012 to 2017, what we did was we kicked off the Patient-Focused Drug Development Initiative where we had 24 disease-specific meetings to really provide a platform for patients and caregivers and other patient advocates to come to the FDA and tell us how it feels to live with their condition. What happened during that time is that around 2015, we really started seeing and growing external interest and expanding the efforts. As I mentioned, as part of the commitment, we only had 24 meetings. That's what we could commit to with our resources. But honestly, there are so many diseases out there, so what we started to do is actually welcome patient organizations to identify and organize their own patient-focused collaborations to generate the similar type of inputs that we were doing here at our FDA-led, patient-focused meetings.

These meetings are truly just to provide an important opportunity for patients, caregivers, and other patient representatives to come and talk about subjects that matter most to them, impact on daily life, current treatment options, and talk about the treatment burden.

With that, what we did was we opened this up to the external groups. And while FDA -- definitely we attend these external meetings that are hosted by the patient organization, so it's truly your meetings. Any deliverable that comes out of it or the meeting itself we really say is not considered FDA's sponsored or indoor, so it's truly just your meeting.

Now I'd like to go to let's think about planning for this meeting. What are some things to keep in mind? As I mentioned, the key participants and the folks that we really want to hear from at these meetings, whether it's led by FDA or it's led by patient groups, is really the patients, patient representatives, and patient advocates. So that being said, all of the other folks, such as regulatory and federal agencies, including FDA, medical product developers, researchers, and healthcare professionals that are out there, we want them to be in the audience, but they are really typically in listening mode because it is really giving a platform to the patients and caregivers.

One thing I always like to say is the FDA-led meetings, we did 24 meetings with a group of only five of us, so we understand that it can be very resource intensive. And we just want folks to think about if you decide to do one of these meetings, it doesn't necessarily always have to be a stand-alone meeting. There are several groups that have annual conferences, or there may be a scientific workshop that's being planned. So it could be part of those conferences as a session maybe. It doesn't even have to be a full-day meeting. Maybe it's something that you have two hours where you engage with the patients. So there are various options for those. And what we recommend is actually that we have the FDA-led meetings, and that can really serve as a model for you as you're thinking about identifying the disease area that you want to have a meeting about. We have some criteria laid out that's on our website, thinking about the discussion topics to focus on and what we've typically focused on, the types of questions we've asked, the format that we use. We always use this unique format that you wouldn't typically see here at the FDA where it's a facilitator-led large group discussion. It's very much interactive. We go into the audience and try to get more perspectives from folks in the audience.

There's a polling similar to what you've seen today and an interactive webcast. And what we like to see from these are really meeting deliverables such as actual Web recordings, transcripts, and really some type of summary report, let's say, for folks in the future. If they want to refer back to the meeting, the summary reports can really serve as a really good resource for us here at the FDA and for other stakeholders as well.

Some key considerations when thinking about this, we do have a letter of intent process, so we
1. ask that you submit a letter of intent to CDER's Office of Strategic Programs. That's our office that I'm in. Our team is really here to serve as a resource for you, answer any questions, and help you as you start planning the meetings, so we really are here to help.

2. As I mentioned earlier, we understand it is a resource-intensive effort, but sometimes you may actually have the people that you need within your organizations. A meeting planner may not always be necessary or full on-conference organizers because we realize that does cost a lot of money there.

3. And honestly, at the end of the day, active community outreach is very important for these meetings because you want to be able to get the patients and caregivers to ensure a representative group. So we really rely on patient groups and organizations that are out there to get patients in the room for our FDA-led meetings and for your own meetings. Obviously, you have more of the contacts with these groups, so it's even better.

4. At the end of the day, we do want to be respectful of the time of patients and their caregivers, so we really need to think about is it really necessary. Is this the right time to have this meeting? What are we going to do with this information. So taking those into consideration is very important.

5. Just final thoughts, these meetings really do strengthen the understanding of the disease and treatment burden, what we call the therapeutic context. This input from these meetings can support FDA staff as they're thinking about conducting their benefit-risk assessments for products under review or even while advising drug sponsors on their drug development programs. But more broadly, it can support drug development as well by thinking about identifying areas of unmet medical need in the population, identify and develop tools to assess the benefit of potential therapies, and raise awareness and channel engagement within the community.

6. I do want to mention that the reports that I mentioned earlier, the deliverables from these meetings, if you do conduct an externally-led meeting and you have a summary report, we recently in January launched an external resources page where we're actually housing those reports. So we ask that you house it on your website, and we're linking to those reports so it can be available on our page as well so that we're also sharing the information and making it available for folks. This is just a glimpse of that. If you search "external resources" or "information," you can get more information on this.

7. With that, I will turn it over to Andrea. Thank you.

8. And honestly, at the end of the day, active community outreach is very important for these meetings because you want to be able to get the patients and caregivers to ensure a representative group. So we really rely on patient groups and organizations that are out there to get patients in the room for our FDA-led meetings and for your own meetings. Obviously, you have more of the contacts with these groups, so it's even better.

9. But because I'm being diplomatic, I'm going to let Andrea because she has an interesting fun fact. And I apologize for my voice. I have a little something going on, and we'll figure out what that is after this is over. My name is Andrea Furia-Helms, and I'm with the patient affairs staff, newly established. My fun fact is in November, I went to Ireland with my husband on vacation, and we took archery lessons. The final challenge was to hit a balloon on the target, and my husband, who is an ex-Marine, missed, and I got it directly center. And the
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As I mentioned, I'm from the patient affairs staff. We're in our infancy stages. We were just developed in December of 2017, and we work closely with the medical products centers. What we do is work on cross-cutting issues. Each of the individual centers have their own patient engagement activities such as the patient-focused drug development meetings that are focused on drugs and biologics and Center for Devices, that are also focused on devices. But we work on cross-cutting issues, and we help coordinate and complement and enhance those types of patient-engagement activities where more than one medical product center might be involved. We report into the principal deputy commissioner for medical products and tobacco, which is part of the immediate office of the commissioner.

I'm going to talk to you a little bit about the first initiative that came out of patient affairs, and that's the Patient Engagement Collaborative. This is a collaborative forum with the Clinical Trials Transformation Initiative, and we're establishing an external group to come to FDA and meet with us regularly to talk about patient engagement and how we can enhance those experiences, engage better with you and you engage better with us, and to better learn the regulatory processes. Why are we establishing a patient engagement collaborative? There was an impetus for this. First and foremost, we listened. There was a docket that was opened, and there were several comments from stakeholders saying that we'd like to meet with FDA regularly, which is understandable. Sometimes we are in reactive move where there's something that comes up and we need to hear from you immediately. But it's really important that we hear from you as stakeholders on a regular basis.

So we heard loud and clear we need to establish some kind of forum to meet with you regularly and have conversations ongoing, and the laws also. In the recent laws, the 21st Century Cures Act and FDARA, there's a lot about fostering patient participation and incorporating patient experiences in the process. And we had a model. I had the great opportunity to spend a fellowship a couple weeks at EMA, the European Medicines Agency, and they've had the Patient and Consumers' Working Party for the last 10 years, where they have organization representatives meet with the EMA regularly to talk about regulatory discussions and patient engagement. Another model is NIH's COPR, the Council of Public Representatives, similarly meeting with the community and understanding their needs and how they can participate more in biomedical research.

The membership criteria, obviously patients who have a personal disease experience, caregivers who support patients could be parents of children, a partner, spouse, family member, or friend who serve in a primary caregiving role, and then also representatives from groups that have either direct or indirect experience with diseases. In December we opened the Request for Nominations, and on January 29th, it closed, and we received 200 nominations, more than expected but pleasantly surprised. So currently we are reviewing and looking at which members are meeting the criteria. So we have a selection committee right now reviewing those nominations. We hope to schedule the first inaugural meeting in late summer, early fall. And even though the comment period has closed and the nomination period has closed, that doesn't mean you won't have an opportunity. The membership will be two to three years staggered appointments, and we are looking for diverse perspectives to come in after the two or three-year appointments to come in and share their perspectives as well. We want to make sure we're including a broad perspective that's not always the same voices every time we have the patient engagement collaborative nominations. If you want more information, there has been a voice blog that was issued on December 20th on the patient engagement collaborative.

Another initiative that we're launching is...
listening sessions, and this is to better understand the patient experience around diseases, especially in rare diseases. So we created a memorandum of understanding with the National Organization of Rare Disorders. These are pilot listening sessions, so just a little bit of background. Medical officers during their review work, they will sometimes say I don't really understand this particular disease and can you connect me with patients and caregivers so I can better understand the disease, around disease burden, treatment burden? What kind of activities are they limited to due to their disease and how can they improve their quality of life if a treatment were to be developed?

So we would have these teleconferences and gather patients and caregivers to share their experiences, and the review divisions find it very valuable and useful. So we're going to pilot listening sessions with NORD, and we're going to develop these in a certain therapeutic area. Really, the goal is to demonstrate added value so we can hopefully expand to other therapeutic areas.

Right now we're in the process of deciding on what the pilot therapeutic area is, and again, it would be a cross-cutting area so that it would include all the medical product centers and the review divisions specific to that therapeutic area. We're in the process of developing a process with NORD for the listening sessions, and we're thinking about including an educational component as well. I think it's important to have some kind of basic understanding in the regulatory process, and I think workshops like this or even webinars and things, that might be helpful as a precursor for joining a listening session. And then we're going to evaluate internal and external feedback, and then develop recommendations on how we will move forward.

So that's what I'm sharing with you today. I'm happy to take questions after all the presentations. Thank you.

MS. BLAKEY: Thank you.

Salina Miller is our next speaker. She's a health programs coordinator, however, her office has recently changed, maybe in the last 24 hours or so, Salina. Maybe you could update us on that and share a fun fact.

Presentation - Salina Miller

MS. MILLER: I can't necessarily follow Wonder Woman here, but I did try to pool my family members for a fun fact because I couldn't think of anything myself. I'm full of fun, by the way. Some of them I can share with you, but one I can't share with you. One I will share is that my dad told me that he had applied for me to be the Indian Gerber baby back in -- well, should I tell you back when? But I'm still waiting on the response.

Yeah, so that's the fun fact.

As far as the office, I work within the Office of Health and Constituent Affairs, which is within the Office of the Commissioner. Yesterday, we announced that the patient representative program will be piloted within the advisory committee oversight and management staff. So that is starting as of yesterday for about four months, and we're going to be working together to kind of leverage off of each other and learn more about recruitment efforts, streamlines and things. So it should be exciting, so I'm looking forward to that.

The FDA patient representative program, it really began in the early or late '80s, early '90s, soon after the HIV epidemic evolved from that. It did roll into including cancer patient representatives, so oncology was a big part of the program as well. And now it really is the flagship program for patient representatives, which are considered special government employees to engage with the agency in a formal process.

It's really a mechanism that provides pathways for patients and caregivers to be an active participant in what we do, provide a voice, that important voice, voices that we want to hear from in whatever decisions we make regulatory-wise, and it really furthers the understanding of who we are, and that's such an important part, and provides a presence at the table for patients and caregivers.
We have about 200 FDA patient representatives in the program constantly recruiting. These patient representatives collectively represent anywhere from 300 to 500 diseases, conditions, or device experiences. I've listed just a few of these on the slide. We are continuing to recruit. We have areas in terms of opioids, opioid use, naloxone use. Pain is always something we're recruiting for; COPD. There's a host of these that are listed online on the For Patients website of the FDA page.

So what do we look for in becoming a patient representative? First, I should really, really emphasize that the agency looks to recruit based on need. That's a very important thing to understand. We are constantly communicating with patient groups, with patients and caregivers directly. And during those conversations, we are learning about what's in their communities, what's in their pipeline; internal conversations with the divisions and the reviewers of what they're seeing; how can we forecast what's really needed in terms of experiences so that we can start the recruitment process as early as possible.

In terms of being a patient rep, we look for certain things, first and foremost, the personal experience with the disease or condition. It doesn't necessarily have to be a patient. We also understand there are certain situations we have to ask for a caregiver to represent, maybe a minor or someone who's unable to represent themselves.

Community awareness, it's significant; advocacy experience that is relevant to not just their own experiences that they can share, but also those of their community. That's a very important aspect that we look for, someone who's objective, absolutely; analytical, preferred but not a must. Some of our patient reps do like to delve into the science of what we do, and it's just helpful but it doesn't necessarily have to be the case.

Of course conflict of interest. This is an area that is growing. We know that patients are much more engaged in their communities. We also know that there are areas of conflict of interest that can be an issue, so we try to have that conversation with them early on to really probe and see what are their activities about and is there something that we can do, or a mechanism, or a waiver that we can use to make sure that they can be in the program.

Great communication skills. I've had many conversations with patient rep candidates who are so excited to serve but yet when it comes to the communications or if they are gun-shy speaking in front of people, it can be a learning curve for them. But of course commitment to serve, it is really important to emphasize the importance of how we rely on our patient reps to serve on committees and to recognize that assignment as an important aspect of serving.

Generally, patient reps intersect with us, both in the drug and biologic development phase early on in the process, as early as when we receive an application. But really it's up to -- as Andrea says, it's up to the conversation with the medical person or the reviewer really, who can give us kind of a clue into learning more about a disease or condition so they can have these consultations with patient representatives early on throughout the process and get a sense of targets, or benefits and risks, or things that the patient is interested in sharing. That is the opportunity.

There is also another area which would be the advisory committees. That stage is really an important and significant part of the patient representative program, and it provides the patient representatives at the table during the advisory committee meeting surveying with other scientific members.

The advisory committee meetings, our patient reps are generally considered as temporary voting members. For each assignment, they are screened for conflicts of interest for each assignment. The disciplines, as I said, are other scientific members, and these committees are across all the medical product centers. On average, we have anywhere from about 35, 40, to about 60 assignments per year.
1. Some of the other ways they can serve, they can serve as consultants, as I mentioned, connecting with the divisions directly, having a telecon with them, sharing their personal experiences and actually being privy to confidential information. Workshops and symposiums also are the growing activities for the patient representatives. It's a little outside of their role as a patient representative, but they certainly are very effective in those areas, and we are continuing to use them. So once they become a special government employee, what happens? We have patient reps who have really no idea who we are, how we're structured, and what are some of the activities. So it is our job in this office to really get them ready to serve. We do a very personalized FDA 101, providing them background on the agency. We have them engage with other more seasoned patient reps that are in the program. We describe how the scenarios are for serving on an advisory committee meeting, for example. We provide regular training webinars, where they're able to engage with specialists here internally and can ask real questions of them. So it's a closed webinar, and they feel comfortable to ask whatever questions they feel necessary. But they do have that resource, and we do have resources online where we can provide patient reps with information firsthand. And also, every year we have an annual workshop, and at the workshop we have folks who come within the agency with their expertise and are there typically for a day and a half, and they can engage with them. So that's a real significant way that we engage with the patient reps, particularly within the new recruits. Here's just a snapshot of last year's workshop. It kind of gives you an idea of who's all there. There are folks here from the agency and also some of the new recruits from the FDA patient rep workshop. I did not include an email address, so it's fdapatientrepprogram@fda.hhs.gov in case you are interested in becoming a patient rep.

2. MS. BLAKEY: Thank you, Salina.
3. MS. MILLER: Sure.
4. MS. BLAKEY: Diane Maloney has pulled herself away from very important duties at the commissioner's office to join us today. And Diane actually represents the Center for Biologics, so she has a slightly different perspective but no less interesting than the other speakers. So welcome, Diane, and thank you for joining.

5. MS. MALONEY: Thanks so much, Rea. And I'd like to first say thanks to CDER generally, and Rea as well, for including CBER in this workshop today. Sp I wanted to just give you a very high-level introduction to CBER, Center for Biologics Evaluation and Research, and the work that we do involving patient engagement.
6. Oh, I did have a fun fact.
7. MS. BLAKEY: Please.
8. MS. MALONEY: I told Rea my fun fact is actually somewhat of a costly fact. I have three daughters, and all three of them got married within eight months of each other, but it was fun, and memorable.
9. MS. BLAKEY: And costly.
10. MS. MALONEY: And they were all very special and unique, as are my children.
11. CBER and patient engagement, we do need to hear from patients. It's very important. I'll tell you a little bit about the Center for Biologics. This is a picture that was taken in December of some of the folks in CBER. We are at the far end of the campus in one of the newest buildings, and we have actually quite a lovely atrium. So there we are, at least a lot of us, taking a picture of many of the employees in CBER. I just wanted to let you know a message that I think you've probably heard a lot today, that we FDA and CBER really do listen to patients. We recognize the important voice that you have and the unique voice that you have; and that, really, it is a critical one in the regulatory decisions that we
1 make. And we very much value engaging with
2 patients and all that people do to contribute to,
3 in our center, the development of biological
4 products in particular.
5 Within CBER, we actually have a number of
6 activities that we do with regard to patient
7 engagement. One of the things is increasing
8 awareness within our center. We have a number of
9 groups that we've formed to pull people from all
10 the various offices that we have. We have a
11 patient engagement working group, and we have a
12 rare disease working group. All of our offices are
13 represented. We share information. We talk about
14 outreach opportunities and what's going on with the
15 other centers and the commissioner's office as
16 well, in patient engagement in general and rare
17 diseases as well. That would be within the center.
18 In addition, we work very closely -- I work
19 with all my colleagues here in the commissioner's
20 office, Center for Drugs, as well as the Center for
21 Devices, on cross-cutting patient engagement
22 issues, and then of course external work as well

1 with patient groups.
2 Again, I think you all recognize that
3 patient-focused product development and drug
4 development is evolving over time, and it's been
5 going in since as early as the 1980s, maybe before
6 then I think with -- I was actually here in the
7 late '80s with the AIDS patients. I think we
8 learned a lot hearing from them and the value and
9 seeing things from all the various perspectives,
10 and then continuous as part of the Cures Act and
11 some of the provisions that I think people have
12 presented today.
13 I just wanted to talk a little bit so you
14 know -- we work, as I said, closely with CDER and,
15 CDRH as well, and the commissioner's office. A
16 number of patient groups are here not because they
17 are looking at a particular product but because
18 they care about a particular disease. And the
19 disease isn't necessarily -- there may be many
20 different therapies or diagnostics that would be
21 appropriate for that particular disease. And often
22 when a patient group might want to meet with the

1 agency, it might be more appropriate to meet not
2 just with one center but to meet with another
3 center or all three medical product centers
4 together for that particular disease. So I just
5 wanted to underscore that.
6 Some of the products that we regulate within
7 the Center for Biologics are on this slide. We
8 regulate vaccines, including preventive vaccines,
9 childhood vaccines, as well as some therapeutic
10 vaccines. There are some cancer vaccines,
11 allergenic products. We regulate live
12 biotherapeutic products, or some people refer to
13 them as probiotics. We have many blood products,
14 for instance, for a lot of bleeding disorders.
15 We actually within our center regulate some
16 devices, so we regulate some devices that are used
17 to screen blood donors for infectious diseases.
18 You wouldn't want to take blood from someone who
19 might be infected with a disease, a virus, that
20 could be transmitted through blood. We also
21 regulate tissues, so for instance, skin and bone
22 and cornea, as well as cellular products,

1 xenotransplantation products, and gene therapy. So
2 it's quite a range of products that we regulate.
3 Now I will give you a high level of some of
4 the types of meetings that we have had that have
5 involved patients. Salina has actually touched on
6 a lot of these. We have meetings where patients
7 have been involved for specific products. So they
8 might come in -- especially in the
9 instance -- Salina talked about they might be
10 special government employees, so they could have
11 access to confidential information. And they might
12 meet with one of our product offices at the
13 investigational new drug level, where the sponsor
14 is there as well. In addition, they might sit on
15 an advisory committee on specific issues that we're
16 dealing with, with regard to the particular
17 product, or as we're reviewing a biologic's license
18 application. So that would be the application for
19 a product approval.
20 In addition to product-specific meetings, we
21 sometimes have issue-specific meetings or
22 disease-specific meetings. For instance, we might
1 have an advisory committee meeting on a particular issue. For instance, one area we were dealing with a couple years ago had to do with a risk assessment we did with regard to variant CJ, Creutzfeldt-Jakob disease, and what risk, if any, there was to patients who received blood products. And we engaged with some of the patients in terms of how best to communicate that risk in a way that was understandable and clear to folks. We also engage with patients at a variety of public meetings and workshops, some of which we would sponsor, and then others that others sponsor and invite us to. In addition, we will meet with patient organizations, similar to John's meetings that they hold, and again, which can be with our center as well. We sometimes have meetings where we meet, just our center, with various patient groups.

You've heard Pujita talk about the patient-focused drug development meetings. CBER has been very involved in those as well. CBER led the vast majority of them. We led three of them I think but participated in many more of the internal ones. And in addition, we also have attended many of the externally-led, patient-focused drug development meetings and very much appreciate the invitations to do that and all that we've learned from all the patients.

Those are just examples of the patient engagement that we have had, and this is just our contact information on this slide should you want it. Thank you very much.

MS. BLAKEY: Thank you very much. We can live it up for a moment.

I just want to get a gauge because I want to be mindful of the time, how many of you actually have questions that you cannot leave here today without asking of any of these panelists? Because I can condense our discussion, being mindful of the time.

(No response.)

MS. BLAKEY: No one absolutely, positively has to ask a question before they leave here today? You're thinking about it. You're processing it.

Okay. Another question for you. How many of you have considered having an externally-led PFDD meeting?

(Hands raised.)

MS. BLAKEY: Okay, getting a little bit more response there. That's good, important. How many of you have interacted already with the patient affairs staff; for example, the PECs?

(Hand raised.)

MS. BLAKEY: Okay, a few more there. We like to know who's in the audience. Anybody here a patient rep, has already worked with OHCA as a patient rep?

(Hands raised.)

MS. BLAKEY: Okay, a couple. Anybody had meetings already with CBER?

(A few hands raised.)

MS. BLAKEY: We're going to work on that for you, Diane. We're going to get you a few more folks.

One of the things that we discussed as a center as well. We sometimes have meetings where we meet, just our center, with various patient groups.

You've heard Pujita talk about the patient-focused drug development meetings. CBER has been very involved in those as well. CBER led the vast majority of them. We led three of them I
and the complexities of the conversation are changing. Patients are recognizing who we are more and are able to ask really poignant questions. So we have to be on top of our game and being a resource for them.

I'd like to see that we are able to enhance our current strong platform when it comes to patient engagement with the program, particularly, and that we're able to think of novel ways that the patients can -- particularly the ones in the program are able to engage with the agency, and we're seeing that trend already. Adcons and homework assignments with divisions are key, but also thinking outside of that and having their perspectives come in different ways I think is one that I'd like to see over five years.

MS. BLAKEY: Thank you.

Pujita, you're actually involved in writing some of the guidance for these things. What's on your plate for the next five years?

MS. VAIDYA: If the past is any indication, if you think back to five years ago, as I said, in 2012 when we started thinking about how do we get the patient's voice into this, how do we collect this, we've come a very long way in the past five years. There's been so much that has happened. Now we know, we've learned that patients really want to be active. They want to be at the table, and really, a lot of folks have been given that opportunity. As you hear all of us talk about, we want more of you to come take initiatives. There are several opportunities out there. There are areas where you're experts, where we may not have the expertise. So there's definitely a lot going on.

Rea just mentioned the guidances. In the slide that Diane put up in the 21st Century Cures, we have a series of guidances that we're going to be putting out in the next five years. So it's really hoping that -- and this is to help guide the methodological way of collecting this type of patient input and for us to be able to take that and incorporate that so that it can inform regulatory decision-making and drug development; so

not just what we do, but the whole development process.

So in five years, that's when the plan is to have all of the guidances out, and we hope that we've given out all the information, that folks have to take that and be able to collect robust data and patient experience data, as we call it, and either submit it to us -- in some cases we may be the end user, but in a lot of cases it may be other folks, the industry counterparts that we have, or other folks who can take that data and do something with it. I'm hopeful for the next five years because I think our plan is to put a lot of guidance out there, and hopefully that will be informative for everyone. Thanks.

MS. BLAKEY: Diane, did you want to weigh in on that?

MS. MALONEY: Sure. I'll add as well. We certainly won't be moving backwards. I think that you'll see more and more FDA folks that are having direct contact with patients. I know just myself, I've had a lot more and learned so much just in the last two years or so in terms of the involvement.

And I agree with Pujita. Congress now has asked us to do some things, and we will do them. But I think we were committed to doing a lot of engaging with patients as well.

So I think it's a journey. We've begun it, and I think it will continue. And in five years, it will be interesting to look back to see what we've achieved and then what more we have to do.

Thank you.

MS. BLAKEY: Andrea?

MS. FURIA-HELMS: I think there is an opportunity for being a new office or a new staff. The opportunities are actually quite endless. You're starting from scratch and really understanding what the needs of the patient communities are, and that's what we're trying to do. We're trying to reach out and understand what their needs are and where can we enhance patient engagement across the medical product centers. Although opportunities are endless, resources aren't always, but we're trying to do our best in
incorporating as much of your voices as possible where we can, and I think part of it comes from collaborating with the other centers.

One of the things I do think that I hope to see in the next five years is the evolving of the patient experience data, the science around it. I think it's starting, and it's really not ripe yet, but I think it's developing. And I think with the guidances that Pujita's office is working on and a lot of this whole new science that's being worked on outside of FDA, I think there may be opportunity that in the future we can say that a listening session or a patient-focused drug development meeting actually informed a regulatory decision, and we can actually correlate the two. So that's something I'm hoping for.

Questions and Answers

MS. BLAKEY: Ditto. Second that.

We just have a couple minutes. If you have questions, now would be the time. We do have a couple of people who can walk around with a microphone. Christine is there at the ready. If you've written anything and maybe you don't want to be the person to read it, you could hand it over to Christine.

Yes, please?

MS. KERKORIAN: I'm still not totally clear on the difference between the listening sessions and the patient representative program and what the goals or objectives are of those two initiatives.

MS. MILLER: The patient representative program, we have to do some recruitment for the program to make them special government employees. It is a four-year term, and they're able to serve in a different capacity. So they are really on standby at any point that we want to have a conversation with them that may involve some confidential information. There is reimbursement and some compensation for certain activities. They can also participate in a listening session, but I believe the listening sessions are outside of that. It could be the general public.

That's really the primary distinction that I can think of, right?

MS. FURIA-HELMS: Yes. The listening sessions really are driven by review divisions wanting and have an interest in understanding typically a rare disease. They haven't had experience with it, and they might have this need to better understand it in their review work.

They are also quickly turned around. Just recently, we got one done within four weeks, so it's something that's a teleconference. We can reach out to advocacy organizations to help identify exactly the patients we need to hear from that patient community, and have those teleconferences with specific questions that are coming from the review division so they can better understand disease burden, quality-of-life issues, and just how the disease impacts them on a daily basis. And even, because it's typically around the area of rare disease, how are they managing their symptoms without any products on the market.

MS. KERKORIAN: That's always initiated by [inaudible - off mic].

you've written anything and maybe you don't want to be the person to read it, you could hand it over to Christine.

Yes, please?

MS. KERKORIAN: I'm still not totally clear on the difference between the listening sessions and the patient representative program and what the goals or objectives are of those two initiatives.

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That's really the primary distinction that I can think of, right?

MS. FURIA-HELMS: Correct.

MS. BLAKEY: If I might add, the listening sessions in particular, that word is key because, really, we're listening to hear what you have to say, but typically we're not necessarily responding. You'll recall Sadhna's talk earlier about what FDA can and can't do. And there are times when we want to glean information from you, but we can't necessarily tell you why, or what it is, or where some product might be in the review process. So listening is key. It's typically more of a little bit of a one-way type conversation, though, we want the information so that we can apply it in some form or fashion.

I do believe someone back here has a question, and then, ma'am, you'll be next.

FEMALE AUDIENCE MEMBER: Our organization would like to plan a PFDD meeting as part of our annual patient conference, so I have two questions. How much lead time do we need to plan this sort of meeting, and do members of CDER travel, or do we need to hold the meeting within the DC, Virginia,
Maryland district?

MS. VAIDYA: Thank you for your question. To answer your first question, we do have a letter of intent process, and in the guidelines that we have set forth for that, we do ask that at least a one year’s time headway would be nice. If you’re planning a meeting right now let’s say for April 2019, or thinking about or considering something there, we ask that you actually start thinking about submitting your letter of intent around this time, this time of year.

Typically, the planning itself, I would say from the experience that we have, it takes at least six months to plan one of these meetings to really get it solid. So it’s planning for that, and we want to make sure that CDER, CBER, CDRH, all of our colleagues are aware of it so that they have enough time to actually plan to attend these meetings as well.

So your question about traveling, one of the other points that we have in our guidelines is that it will be much easier for FDA folks to attend the programs, and how are the roles different?

MS. FURIA-HELMS: Sorry if I wasn't clear on that. The patient engagement collaborative is a forum to talk generally about patient engagement, and understanding your experiences, and engaging with FDA, and understanding our experiences so that we can better enhance our engagements with patient community stakeholders, so general discussions.

With the patient rep program, as Salina will probably tell you, it's specific to a medical product. They have to go through conflict of interest to review that confidential information.

With the patient engagement collaborative members, they're not screened. They're not special government employees. Conflict of interest is considered during the selection process but not rigorously like becoming a special government employee. So anyone can be as long as you meet the criteria, which is similar to the patient rep program. As long as you meet the criteria, you can nominate yourself or be nominated by someone else.

MS. BLAKEY: If I could, just because we are meeting if it is in the DC, Maryland, Virginia area. That is ideal. However, if you have a very good webcast option or something, that will definitely help. But to try to get folks to actually be there in person, we do try to encourage planning in this area. Thank you.

MS. BLAKEY: I apologize. I’m going to have to make this the last question or comment just because I promised I would be mindful of time. Whoops. Okay. We'll have two.

You tell me, Christine. I'm sorry. I meant the lady right here with the red jacket. I apologize. If you really do want to ask a question, we'll try to squeeze that in.

FEMALE AUDIENCE MEMBER: Similar to the first question, I was a little confused on the difference between the patient engagement collaborative and the application process you're currently going through with the 200 applications and the patient representative program where people have a separate application. What kind of candidates are you looking for both of those programs, and how are the roles different?

FEMALE AUDIENCE MEMBER: My question is whether the general public can attend the advisory committee meeting. If we can, what's the process to apply to attend such a meeting? Thank you.

MS. MILLER: Generally, advisory committee meetings are public. There's no necessary registration per se. We've had patient groups that have had registration for applications just to bring them to the meeting, but for the most part, the agency is open to the public.

MS. BLAKEY: So we hope you'll come. We hope you'll all be there. Thank you all very much for your attention. How about a round of applause for our panel?

(Applause.)

MS. BLAKEY: Thank you, ladies. Very informative. Thank you all. Appreciate it.

Audience Response Questions - Christopher Melton

MR. MELTON: Now it’s audience response questions again, so if everybody could please grab
1 their clickers, I'll be going through the audience
2 response questions. I'll be reading four questions
3 for everyone.
4 Please evaluate the following sentence.
5 "Following the how to get your voice heard
6 discussion panel, I feel that I have the necessary
7 information and resources to request a meeting with
8 the FDA." Your choices are A, strongly agree; B,
9 somewhat agree; C, neutral; D, somewhat agree; and
10 then E, strong. So take a second and put your
11 answers, and we will tally the responses soon.
12 (Audience responds.)
13 MR. MELTON: We have A, 63 percent. All
14 right, great. Now we'll move forward over to
15 question number 2. "How long does a new drug
16 application take, also known as NDA, the approval
17 process typically take?" A, less than 6 months; B,
18 approximately 6 to 10 months; C, approximately 1 to
19 4 years; D, an average of 12 years; or E, none of
20 the above.
21 (Audience responds.)
22 MR. MELTON: The correct response is B,

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1 approximately 6 to 10 months, and we have
2 42 percent. So we've got that marked for next
3 year, and we'll know.
4 Question number 3, "The FDA can publicly
5 disclose the status of a drug product currently
6 under review." Answer true or false, A being true;
7 B being false. This is an easy one.
8 (Audience responds.)
9 MR. MELTON: Or maybe not. The correct
10 answer is B, false. Now we're going to transition
11 to question number 4. Drug manufacturers are
12 required to report adverse events from a drug to
13 the FDA, A being true; B being false.
14 (Audience responds.)
15 MR. MELTON: The correct answer is A, true.
16 We've got 9 percent that will get it the next time,
17 right? Thank you.
18 DR. WHYTE: Okay. That's not the right time
19 now, but we are in the home stretch. And I will
20 point out we have been very close to time. We've
21 spent a lot of time telling you the FDA
22 perspective, and myself and my colleagues thought

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1 it was very important for you to hear from others
2 and what their experience has been, the good and
3 the bad.
4 So I'm delighted to invite Alexandra Kruse
5 and Phyllis Foxworth to come to the podium. We
6 asked them to talk about their experience in terms
7 of interacting with the agency. Alexandra is the
8 research coordinator for the Platelet Disorder
9 Support Association, and Phyllis is the vice
10 president of the Advocacy, Depression, and Bipolar
11 Support Alliance.
12 Perhaps we'll start with Alexandra, and you
13 both come to the table -- really, the time is yours
14 to talk about your experience; we did not give any
15 prepared remarks to them -- and then allow them to
16 ask questions directly of you. But I really wanted
17 the time to be yours, and I appreciate you coming
18 and sharing your experience, the good and the bad.
19 Thank you.
20 Presentation - Alexandra Kruse
21 MS. KRUSE: Thank you to the FDA and
22 especially to PASE for inviting me here to speak

1 today on behalf of the Platelet Disorder Support
2 Association. For 20 years, PDSA has been
3 empowering patients with immune thrombocytopenia,
4 or ITP, a rare autoimmune bleeding disorder that
5 affects 9 out of 100,000 people around the world.
6 Through education, advocacy, research, and support,
7 the FDA has really encouraged rare disease advocacy
8 organizations to make their voices heard, as
9 95 percent of rare diseases don't have an approved
10 treatment and there are no cures, making the work
11 that advocacy organizations do that much more
12 important and improving a patient's journey towards
13 better health.
14 Furthermore, many rare disease organizations
15 have an average staff of three people. Often they
16 are caregivers of patients or they are patients
17 themselves, making it difficult to prioritize
18 initiatives on behalf of their patient population.
19 PDSA has a staff of five full-time employees, and
20 I'm excited to share that engaging with the FDA has
21 been so much easier and more accessible thanks to
22 public workshops like these, in addition to
Registries are great for recruitment and provide data retrospectively and prospectively. History studies, as they're a golden opportunity to engage with the agency.

First I'd like to share with you a little bit about PDSA's journey in engaging with the FDA. PDSA was founded 20 years ago by ITP patient Joan Young. At that time, there were few therapies available to treat ITP, and the main treatment choices were either really high doses of steroids or surgical removal of the spleen, so neither a fun option.

Joan started the organization like many other advocacy organizations by empowering ITP patients through medical education and providing support forums for patients to share their stories. Ten years later -- so we're at 2008 -- Joan testified before FDA's oncology drug approval committee, or ODAC, a group of outside scientists, clinicians, and laypeople charged with making recommendations to the FDA on various treatments. This was our first interaction with the FDA, and unfortunately it would be our only interaction until eight years later.

PDSA is really grateful to have a really strong relationship with healthcare professionals, medical institutions, researchers, the pharmaceutical industry, and other patient advocacy groups, but we have not been involved much in the regulatory process since Joan's meeting. As one of the four pillars of PDSA's mission is advocacy, we knew that we needed to change, and we decided to engage with the agency.

It's important to note that in early 2016, PDSA received a grant from the FDA and the National Organization for Rare Disorders to begin a natural history study patient registry to collect patient experience data. Commissioner Gottlieb and others at the FDA have stressed the importance of natural history studies, as they're a golden opportunity to provide data retrospectively and prospectively. Registries are great for recruitment and

actively including the patient voice and regulatory decisions, and for allowing us to share our stories with you and answer our questions.

At the end of 2017, our executive director emailed PASE to set up an ad hoc meeting to educate the FDA on the ITP patient experience. Now, actually you can go to fda.gov/requestameetingondrugs – I've gone this morning -- but back then, we received a meeting request within a couple of hours, and we had a meeting set up with the Office of Hematology and Oncology Products in PASE, and I'll talk about the details of our meeting in a bit.

Looking ahead past 2018, we've learned through these public workshops, and especially today, that there are a number of ways to begin help advance the regulatory process for ITP patients to give them access to establish the new drugs and improve the ITP treatment paradigm. The list on the far right with the boxes is definitely not exhaustive, but we plan on conducting an externally-led, patient-focused drug development
meeting next year, submitting preliminary registry data to the agency for use in clinical trials; perhaps provide additional patient testimonies for new ITP therapies if they’re needed; and submit comments on FDA draft guidances. All of these activities help the FDA in providing more experienced data, which ultimately helps the patient.

Now I’m going to talk about our 2017 meeting with OHOP and PASE, and what we did to plan our meeting and some of the key things that I’d like all of you as patient groups to take away from what we learned. There are four key things that I would say are important in planning a meeting between the FDA and a patient group.

First, it’s important to involve key leaders. Our meeting in November included our executive director, who spoke about PDSA’s initiatives to help ITP patients and our goals for the meeting. A patient representative, Barbara Pruitt, who is a fierce advocacy for improving the lives of ITP patients, shared her 50-year journey of living with ITP; one of our medical advisors, Dr. James Bussel from Weill Cornell Medical Center, who discussed the unmet scientific need of ITP physicians and researchers; and myself, who as research coordinator shared patient experience data from our registry.

Second, our goals were to educate the FDA on the most significant symptoms of ITP, current treatment side effects, burden of disease, and impact of condition on quality of life; to ensure that the ITP patient voice is included in providing guidance and advancing science; and to serve as a comprehensive resource on the patient experience and provide input and guidance in new drug development research moving forward. Most importantly, we asked the FDA to prioritize the unmet needs of our patients. The ITP community needs more efficient diagnostic tests. We need treatments that last and better quality of life. We need increased awareness in public and professional health communities and comprehensive treatment centers to improve current outcomes, and we need increased research and federal funding opportunities. These are lessons we’ve learned from our patients, their caregivers, our medical advisors, and clearly demonstrated to the FDA that we realize what’s missing in the ITP paradigm, and identify for them the unmet need of our patient community. Finally, the last thing we learned is that you must be able to back up your asks with quantitative or qualitative data, as was mentioned multiple times throughout the workshop today.

PDSA’s registry with NORD and the FDA attempts to fill some of the gaps and evidence in the scientific need of our research community. The registry establishes baseline information, logs longitudinal disease progression, and identifies patient-reported outcomes. Its goal is to characterize and describe the ITP population as a whole; assist the community with the development of recommendations for standards of care; assist researchers studying the pathophysiology of ITP and interventional outcomes; and support the design of clinical trials for new treatments. The impact of registries are monumental. Providing patient experience data will in turn be able to help regulators make informed decisions about new therapies for ITP and inform trials.

Thank you to NORD and to the FDA for supporting the rare disease program. We were really encouraged during our meeting, by the way, that the meeting was actually a discussion and not necessarily a presentation from either side. The agency’s prioritization of patient involvement ensures that feedback from patients on endpoints and methodologies, as well as benefits and risks, are integrated into the drug approval and development process. This meeting was beneficial both to PDSA and to the FDA in beginning a fruitful collaboration and open line of communication.

What are some of the take-aways? I think there used to be this idea that patients and the medical community and regulators used to be the silos and didn’t really work together, but I think...
1 it's really vital for everybody to work together to
2 improve how patients feel and function. So it's
3 really important for that collaboration to be
4 occurring, and the FDA really encourages that,
5 which in turn is really encouraging to our patient
6 community as well as to our medical advisors in the
7 scientific community.
8 As I mentioned, the FDA wants to include the
9 patient perspective, so when planning a meeting in
10 whatever form that might take, you need to help
11 them to help you. You need to know as a patient
12 advocacy organization what you bring to the table,
13 which is valuable experience information.
14 Another take-away is to have the right
15 people in the room and ask the right questions.
16 You should have an agenda prepared and make sure
17 you have a variety of disease experts convened to
18 share their experiences. And of course as we've
19 learned today, there are a number of ways that the
20 FDA can help you plan your meeting so you can help
21 them.
22 For us it was really encouraging to our ITP

1 community that we are collaborating with the
2 agency. It really goes beyond facilitating
3 interaction. Working with the FDA raises awareness
4 and gets you one step closer to addressing the
5 unmet needs of your patient population. As I
6 mentioned earlier, patients are able to express
7 what matters most to them and take charge of their
8 own health, which is so important. Working with
9 the FDA empowers patients and helps them feel in
10 control of their healthcare experience.
11 Maybe most important, follow up with the
12 FDA, engage with them early, and engage with them
13 often. This really creates a strong bond between
14 advocacy organizations in the agency and keeps both
15 parties in the loop. This is really the next step
16 in patient advocacy, and it's really exciting.
17 Working with the agency allows regulators to listen
18 to patients regarding the benefits and harms of
19 treatments, as at times their chief complaints may
20 not be factored explicitly into drug development.
21 PDSA was really honored to be given the
22 opportunity to advance the science of patient input

1 and provide guidance to the FDA. PDSA's focus
2 remains the clear and significant medical need of
3 our patient population, and we look forward to
4 collaborating with the FDA in the future. Thank
5 you for this opportunity.
6 (Applause.)
7 Presentation - Phyllis Foxworth
8 MS. FOXWORTH: Hi. I'm Phyllis Foxworth.
9 I'm with the Depression and Bipolar Support
10 Alliance. DBSA is the leading peer-directed
11 organization for individuals living with mood
12 disorders. We were founded over 30 years ago. I
13 like to tell people that it was well before there
14 was Facebook and the internet, but there were
15 several small pockets of support groups around the
16 country in major markets that were holding these
17 support groups. They somehow discovered each other
18 without Facebook or social media, and they came to
19 Chicago about 32 years ago and got together and
20 founded DBSA.
21 From there, we've grown to over 250
22 affiliates around the country that provide over 600

1 support group meetings in their community. I'm
2 with the national organization, and our focus is on
3 providing education, hope, and inspiration for
4 individuals living with mood disorders, that they
5 can and should expect to lead quality, productive
6 lives, as well as participate in advocacy to make
7 that world happen. That takes us to where I became
8 involved with the FDA about three years ago.
9 I'm not going to go into much detail as I
10 often did, but kind of give you an overview of what
11 our campaign with the FDA has been all about. As I
12 said, we became engaged with the FDA about three
13 years ago. We responded to the docket that we
14 learned about today, where they had listed the
15 diseases and disorders that they were considering
16 for FDA-led, patient-focused drug development
17 meetings.
18 So we responded to that docket, and that
19 really forced us to start coalescing around the
20 idea of what is the unmet need and quite frankly,
21 that was the easy part of the whole process. There
22 are about 16 million people living with major
One-third to two-thirds of those people are not getting any benefit from current medical, therapeutic, and pharmacological interventions. Furthermore, people living with depression are at a high risk of suicide. People are dying daily, and there is this idea -- I think I even heard someone say this morning something about depression, "Well, they've got all those compounds out there." Well, the truth of the matter is, two-thirds of the people are getting no benefit from them. People who live with depression, major depressive disorder, are at high risk of suicide. Death by suicide is the 10th leading cause of death in the United States. The sad news is that there has not been any breakthroughs in major depressive disorder in over three decades. Thirty years ago, there were some major breakthroughs with antidepressants and antipsychotics, but there has been nothing since then. And I keep going back to that fact that one-third to two-thirds of individuals who have access to that medication are getting no benefit.

In addition, those that are, are at very serious risk of relapse, again raising the possibility of suicide for them as well. Additionally, people living with mood disorders die 25 years sooner than the average person, 25 years. And that's not because of the suicide; that's because of all the other physical conditions associated with depression.

So we didn't have a difficult time understanding what the unmet need was. There's clearly an unmet need. We recognize that we need to advance the science from 30 years ago, and that's where we certainly want to collaborate with the FDA on understanding where there are new opportunities to look at new science around those disorders. But we also recognize that for patients, current clinical trials are primarily focused on symptom relief, and that's not what patients are interested in. With that hurdle, it becomes difficult for there to be new drug development, so our collaborating with the FDA is, to try to move the needle on both the science and to start looking at what are the outcomes that patients want from treatment that are not symptom based.

Going through over the campaign here, we utilized the resources at our disposal. I will share with you that we certainly didn't know what we didn't know when we embarked on this journey. It's been a great learning process for me. But we were able to use resources at the FDA, particularly PASE. They have been so helpful and valuable. When we started out on this journey, we did what they said not to do. We started dropping emails, and we would have meetings with people. And they would say, "Oh, you need to talk to somebody else," so we would schedule a meeting with somebody else. And we'd go to that meeting, and they'd say, "Oh, you need to have a meeting with somebody else." But I will say that at the end of all those meetings, the person who said you need to have that meeting with somebody else always followed up, and they would email me back, and they would copy that person and say, "You need to be meeting with these people from DBSA." So eventually, that got us to PASE. I remember having a meeting with Dr. Whyte and Rea. They were in the room. And Dr. Whyte said, "Why are you guys here?" And quite frankly, I didn't know why we were here. I just knew that we had this unmet need, and that I knew that there were other patient advocacy organizations that were using the FDA to help them find a solution to their unmet need. So I didn't have an ask when I went in, and that's where PASE was so helpful, is that they were really able to help us. They listened carefully to our unmet need and helped us develop a path forward.

One of the things that I did do after that conversation with them was I wrote a white paper that really helped me coalesce around the idea of what is the unmet need and what is a pathway
I had a lot of mentors, people who were always willing to help me, and they were always willing to share their ideas. I would call them up and drop them an email, and they'd say, "You're doing the right thing. You're on the right path." So I would encourage you to use your mentors out there.

Then we developed some very meaningful input. As I said, when we had that first meeting with PASE, Dr. Whyte said, "Why are you here? What do you want?"

DR. WHYTE: [Inaudible - off mic].

MS. FOXWORTH: He was very friendly, but -- he was very friendly. I do not mean that as a criticism. It was really probing as to what do you want; why are you here? And that's what we needed to hear. And he suggested that we -- he said in this organic meeting, "Hey, it sounds like you guys need a scientific workshop." I took that challenge. He laid down that guideline, and I took that challenge. Within one year, we had a scientific workshop last November where we convened all the major stakeholders, that being patients themselves; caregivers; clinicians; our industry, the people who are responsible for drug development.

I remember Dr. Whyte kept saying that, "Well, you need to be talking to the people who are developing the drugs." The FDA was there. But we began the journey. It was a full-day meeting. It was very small, very intimate, of about 35 people, academics who are responsible for creating those tools to measure, where we started the conversation about what is it that patients want and how do we get to the place where we can start measuring what patients want.

Based on that scientific workshop, I walked out of there. Again, I continued to say that I put myself in these positions where I don't know what I don't know. And that's a message that I will leave you with; it's don't be intimidated by that. That's what's always propelled me forward is I don't know what I don't know, but I know that mentors and people will help me find that answer, and I'm not afraid of it.

That output from the scientific meeting really helped us develop a strategy for the patient-focused drug development meeting. We submitted our LOI last November, shortly after the scientific meeting, and we now have scheduled -- our externally-led, patient-focused drug development meeting is scheduled for November 16th.

So we continue to be on this path. I just want to share that it's a collaborative strategy. As I said before, we knew what the unmet need was, but we didn't know what to do about that. We also knew that other patient advocacy organizations were working with the FDA, but we didn't know what that meant. And the collaborative effort that we've had between the FDA and us has been invaluable. They've been able to help us understand what our ask is. They've been able to help us develop a strategy for moving forward.

I just want to close with what is our strategy for moving forward. As I said, we opened up that -- what we recognize is that, currently, clinical trials for the past 30 years or longer have been based on symptom mitigation, and we know that's not what patients in our space are looking for. But what I realize from that scientific meeting was that we're really not that far apart, that we have FDA language and we have academic language that talks about homogeneous dimensions and domains and validated skills, which mean nothing to patients.

Patients have their own language, and that's about what's working in my life and what's not working. They have heterogeneous life circumstances. If I wanted to scream how many times I've heard at that scientific workshop, "homogeneous, homogeneous, homogeneous," but I realize that we really aren't that far apart, that we have more similarities than we think. It's just that we're all speaking the different language. So that's where we're going with our patient-focused drug development meeting. We are
looking to spend some time in some focus groups
with our patients to understand what it is that's
important in their life; what are they looking for
within treatment outcomes. We are developing
panels that will be able to share the burden
perspective with the FDA. We will then be able to
share some of the qualitative and quantitative
surveys that we're doing over the next six months
with the FDA.
Most importantly is that I have a monthly
meeting with the FDA to help me, and I did not know
that was going to happen. I thought I was on my
own. I thought I was going to have to just pull
together this patient-focused drug development
meeting, again, not knowing what I don't know. And
when they accepted the LOI, they reached out and
they said now is our time for our monthly meetings,
and that has been so invaluable.
So that's the mantra that I will leave you
with. It's okay that you don't know what you don't
know; that the FDA is here to help.
(Ms. FOXWORTH: We submitted our LOI last
November, and it was reviewed, and we received word
in March that it had been accepted. So between
March and November is about -- I can do my math.
MR. ACCETTURA: Well, why did it take until
March then to be accepted? Is that part of the--
MS. FOXWORTH: It goes through the review
process.
MR. ACCETTURA: So it's really closer to
12 months than 6 months?
DR. WHYTE: I think part of it is on our end
das well, that internally there is a bunch of folks
that we want to be involved in it. Our focus
is -- I know everyone, often time frames are
different, but it's also expectations where we can
have discussions on. These are hard to do well,
and what we want is the interested parties to come
together and really think through the process. And
then we want to be able to respond to what the
groups are thinking, and sometimes those interests
are different, and that's a good thing, but then it
takes time to work it out. I know everyone is

Questions and Answers

DR. WHYTE: I think we have time for a
couple of questions, and we'll be wrapping it up
very soon. I will say while you come to the mic,
my big point to people has always been make an ask;
what's your ask? And I think that's part of the
challenge, that folks often get so excited just to
come in and tell their story. It's also important,
what are you asking us to do. And that kind of was
my point, like why are you here?
MR. ACCETTURA: Carl Accettura. I'm with
PharmoRx Therapeutics. I came today because I saw
Phyllis was on the agenda and I wanted to get to
understand what DBSA was doing. And I was more
delighted because I've made connections with
rare-disease-side people. This has been a great
meeting, so I thank FDA for holding this.
My one question for Phyllis was why did it
take all the way to November? Because we heard
earlier maybe six months to put together a
patient-focused drug development meeting. Was
there anything unusual that occurred?

MS. FOXWORTH: And I just want to add that
we received a word, which I think was very timely.
I submitted the LOI November 30, and then somewhere
the 1st of March, we received notice that it had
been accepted. And I need all that time to
prepare. I don't want to just slap something
together that's not of value to the patients nor
the FDA. I really need that time to pull together
a quality meeting.
DR. WHYTE: Any other questions? I know
folks are getting tired.
DR. WHYTE: Well, I want to thank both of you for coming and sharing your perspective, and thanks for your kind words. It’s an iterative process. We want to get better, and hearing from everyone helps us to do that. I want to thank all of you for coming today. I know it can be challenging to get here. We’ve spent many hours here, and I hope it’s been valuable to you. I’ve had the fortune of being up here, and being visible, and getting to interact with you, but as you can see, there are a host of folks that have been involved.

I want to thank my folks and colleagues from the Division of Learning, Chad and Derek [ph]. I want to thank our friends at OCOM and DDI, Zac; Raj; and Sharon; and certainly all the folks on our team, Noah; Rea; Chris; Sadhna; Malena [ph]; Scott; Jungha; Derek; Diane; Mary; Hala; Rhonda; Shawn; Dave; David; Christine; and Chris. You never expect all these folks are necessary to make this type of meeting happen, but it is, and I want to recognize their hard work as well.

We have a couple of final questions, and I’m going to let Noah Goetzel -- as he says, like pretzel -- do the final audience response questions because, again, Noah has done an enormous amount of work bringing this together along with Rea and the rest of the team.

Final Poll Questions - Noah Goetzel

MR. GOETZEL: Thank you very much, Dr. Whyte.

I’m back, everyone. So in the morning -- I have results for you. The first question that I asked, that one hasn’t changed. Still 80 percent of you have been here before.

For understanding the function of CDER, for that one, 20 percent of you said you guys are not at all confident in understanding CDER’s functions; 57 percent said you’re somewhat confident; and 22 percent said very confident. So I’m going to go ahead and ask that question again. Pick up your voting little gadget things, the clickers, and A for very confident; B for somewhat confident; and C for not at all confident.

Go ahead and submit your votes. Thanks.

(Audience responds.)

MR. GOETZEL: All right. We have our results. We had a big jump. Twenty-two percent, very confident, went up to 66 percent, and a few of you say you’re somewhat confident, and amazingly zero percent say you’re now not confident whatsoever. So everybody’s at least a little bit confident in understanding what CDER does, and that’s good news.

On to the next question. In the morning, the question was how confident are you in your ability to navigate through engaging with CDER. In the morning, we had 44 percent said not at all confident; 53 percent said somewhat confident; and 2 percent, one person, said they were very confident.

So now the polls are open. You can vote again, your confidence level with navigating through and engaging with CDER, choice A is very confident; B, somewhat; and C, not at all confident.

Thank you very much, We’ve got one more question for you before the final words of wisdom by Dr. Whyte, and then we’ll be all set. This last question is how would you rate your overall satisfaction with the information presented today during our CDER and You Public Workshop? A, very satisfied; B, somewhat satisfied; C, neutral; D, somewhat dissatisfied; and finally E, very dissatisfied.

Go ahead and vote with your clicker the last time for the day.

(Audience responds.)

MR. GOETZEL: Okay. That’s great news. We have 68 percent who said they were very satisfied and 29 percent who said they were somewhat satisfied; 3 percent are neutral; and nobody said
1 that they are dissatisfied with today's
2 presentation. So that's great to hear. Thank you
3 guys very much.
4 Closing Remarks - John Whyte
5 DR. WHYTE: Well, thank you, Noah, and thank
6 you all for sticking with us. I guess my final
7 words of wisdom would be that we're open for
8 business. We want to hear from you. Check out
9 fda.gov/requestameetingondrugs. Hopefully, it
10 won't crash, and we look forward to engaging with
11 all of you. Safe travels this afternoon. Thank
12 you.
13 (Applause.)
14 (Whereupon, at 3:07 p.m., the meeting was
15 adjourned.)
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**A Matter of Record**

(301) 890-4188
Food and Drug Administration - Public Workshop
CDER and You: Keys to Effective Engagement
April 3, 2018

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