FOOD AND DRUG ADMINISTRATION

PATIENT-FOCUSED DRUG DEVELOPMENT

PUBLIC MEETING

Morning Session

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Food and Drug Administration

White Oak Campus

10903 New Hampshire Avenue

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A P P E A R A N C E S

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C O N T E N T S

C O N T E N T S (Cont'd)

P R O C E E D I N G S

MS. GIAMBONE: All right. Well, let's go ahead and get started. Thank you all for being here. Good morning to everyone. My name is Soujanya Giambone. And I am with the FDA's Office of Strategic Programs. And I, along with my colleague, Sara Eggers, will be facilitating the meeting today. And on behalf of all of my FDA colleagues, I'd like to extend a very warm welcome and a very big thank-you to all of you for being here.

So what I'd like to do is go over the agenda and a few housekeeping items, and we'll get started. You should all have a copy of the agenda. If not, we have extra copies out on the registration desk.

So we're going to start today with some presentations from my FDA colleagues. They will provide some opening remarks, an overview of the Patient-Focused Drug Development Initiative, and background on Huntington's disease and treatment approaches.

And then I'll come back and go over the discussion format. We have two topics today, as you know. Topic 1 is on symptoms and impacts of Huntington's disease. And Topic 2 is on treatment approaches. So for both topics, we'll have a panel discussion followed by a group discussion.

So, we'll start with Topic 1. We'll do a panel discussion and a group discussion. We'll take a break, come back, do the same thing for Topic 2, so a panel discussion followed by a group discussion. And that will take us to the last half-hour of the day, which we reserve for Open Public Comment.

Open Public Comment is just a time that we reserve for anybody in the audience, not just patients or caregivers, but anybody that wants to share some thoughts or additional comments which are outside the scope of Topic 1 and Topic 2.

So if you're interested in speaking in Open Public Comment, you can register for it. The signup sheet is out on the registration table. And we'll take registration through break time.

We'll see how many people signed up and how much time each speaker will have.

And then we'll wrap up the day at 12:30 with closing remarks from FDA.

Just a few housekeeping items. Bathrooms are back out in the hallway into the foyer area. And if you make a right and go all the way down the hallway, you'll see the restrooms there. And we also have a kiosk, as you saw outside in the foyer area, that sells sandwiches, snacks, and drinks available for purchase. So you can please feel free to, if you want to go get something or if you need to take a stretch break, or anything you need, please feel comfortable to do so. We want you to be as comfortable as possible.

And just one more thought. If you want to preorder your lunch -- we have a pretty full house today. So if you want to preorder your lunch, you can go ahead and do that. Just let them know what you'd like to get, and they'll have it ready by the time it's lunchtime. Okay?

All right. So on that note, I'd like to turn it over to Dr. Billy Dunn for opening remarks.

DR. DUNN: Thanks a lot. Good morning. Hey, ya'll. Glad you're here. Thank you very much for coming. Welcome to this meeting on Patient-Focused Drug Development for Huntington's Disease. I'm Dr. Billy Dunn. I'm a neurologist. I'm the Director of the Division of Neurology Products here at the FDA. Our division reviews drugs for a wide variety of neurological diseases, including Huntington's disease.

We're very happy to see so many of you here today. I recognize that there's many faces in the audience. Many of ya'll are joining via the Web, as well. And I know that here up at the podium, I can't look at each one of you individually, but rest assured that I want to thank you personally, all of you, for coming here. I can't tell you how much we value the opportunity to participate in these meetings, to hear firsthand the experiences of people with the

disease, and to incorporate that into our thinking here at the agency.

As a testament to that, we have our entire professional team here at the meeting to listen carefully to what we discuss today, our entire team who works on Huntington's disease. These are all professionals in that area. And we really look forward, as I said, to incorporating what we learn today into our daily work.

Today's meeting is one in a series of these type of Patient-Focused Drug Development meetings. And I will let you know that Dr. Theresa Mullin will be discussing a little bit more about this initiative in just a few minutes.

As you know, Huntington's disease -- I don't need to tell you all this -- is a fatal genetic disorder that causes the progressive degeneration of nerve cells in the brain, resulting in many symptoms, including uncontrolled movements, loss of intellectual faculties, and emotional disturbance.

While physicians may prescribe medicines

in an attempt to help keep these clinical symptoms under control, there is no current treatment to stop or reverse the course of disease, which of course is what we ultimately want to do. We want to get all the way to a cure and fully prevent this disease, and that's what we're after. We're working assiduously on this with our colleagues in industry and scientific researchers. And I assure you that this is the long- term goal.

Dr. Dave Podskalny, our Team Leader for Huntington's disease, will provide a bit more background on Huntington's in just a few minutes.

This is a very important meeting, as I said. We fully understand that Huntington's is a serious condition and that there's a vast unmet medical need for patients with this condition. It is our responsibility here at the FDA to ensure that the benefits of a drug outweigh its risks.

Therefore, having this kind of dialog is extremely valuable for us. What we hear from you today can help us understand how patients view the benefits and risks of treatments for Huntington's

disease and, in so doing, help us with our risk/benefit considerations when we encounter a new drug that may be available for the disease.

We want to hear from you today about the different ways your symptoms and your experiences affect your daily life. This is what we want to improve with the treatments that will be available in the future, and we want to understand the best way to intervene in that regard.

It's also important to hear what you value in a treatment for Huntington's disease and what you would like to see in future treatments for you. It is also important to remember, as I'm sure you know, that FDA is just one part of the drug development process. We do not develop drugs, and we do not independently conduct clinical trials.

Drug companies, often working with scientific researchers or the patient communities, many of you who are here today are the ones who conduct the clinical trials and actually submit the applications for these new drugs to we who

work on them at FDA.

We work closely with these drug companies throughout the drug development process, from the point in time, scientifically, where they're just discovering new molecules, perhaps only exposed to animals, all the way up until they're ready to submit the application to us for marketing, and all the trials that happen in between.

If we do get a drug on the market, we continue to work carefully with them to conduct surveillance on these drugs and ensure that we've accurately described both their risks and benefits as additional scientific information becomes available.

I know there are a lot of representatives from industry, academia, and others in the room, and as I said, on the Web. Thank you all sincerely. I personally thank each and every one of you for being here and being a part of this meeting. This meeting will provide valuable input for you as well.

Again, welcome. I'd like to now turn it over to Dr. Theresa Mullin, who will talk about our broader efforts in patient-focused drug development.

DR. MULLIN: Very good. Thank you, Billy. And good morning, everyone, and thank you so much for being here today. We're really thrilled that you're here. I have to say that, as part of the planning effort on this meeting, I'm also really glad we didn't schedule it for tomorrow or the day after that.

(Laughter.)

DR. MULLIN: Because I think it would have been a lot harder for all of us to get here. So that's one thing I'm really relieved about.

And I want to tell you a little bit more about this overall patient-focused initiative that we have underway. As Billy was saying, one of our most important jobs in the Center for Drugs is to evaluate the benefit versus the risk of a new drug.

And in that context, the patient's

perspective is, obviously, most critical, because the patient is the one that's going to be benefiting from the drug, any benefit there is to have, and experiencing any of the harms the drug may present. And so, understanding their perspective is especially critical.

We took the opportunity in the fifth reauthorization, or the fifth authorization, I should say, of the Prescription Drug User Fee Act, and that's what PDUFA stands for, to have a special initiative to try to focus on getting broader input. Before that, we were really only getting an individual patient representative speaking in the context of a particular drug, which means we had to do a lot of conflict-of-interest screening. And that's a very valuable perspective, but it's one.

And we wanted a much broader patient population perspective, and so, to really help us set the context for weighing benefit versus risk in those decisions that we make related to new drugs. This input is not only important at the

time of marketing preapproval decision, but really throughout the drug development process.

So these patient-focused meetings and this Huntington's disease meeting today is one of the small set that we focused on in PDUFA 5 to try to test out, how could we do better at trying to get more comprehensive input from patients related to the context of the disease and what they were experiencing?

So it's one of the 20 that we set up. Actually, we're going to get to 24 by the end of the five-year period. But these meetings are giving us a way to more systematically collect this kind of information.

We began this process in September of 2012, so about three years ago, announcing a set of diseases that our review division has identified as ones which they thought would really help them or where they really felt the need to get more information about what patients' perspectives would be.

We got about 4,500 comments on those

that we picked. We came up with a set of 16, based on that input, and going back to the review divisions. And in the last year, we've announced another eight meetings, so that we'll have -- this is our just quick showing you the diseases that we're covering as part of this initiative.

In today's, we've got two meetings. This morning we're spending on Huntington's disease; this afternoon, we're going to focus on Parkinson's disease. And there you can see the ones we have for the remainder of the year.

These meetings have been extremely helpful to us in terms of understanding better. And each one of them is focused, first of all, on two sets of questions that Soujanya mentioned earlier, one about how it is to live with the disease and another section devoted to what you're doing to treat your disease and how well that's working or not.

We also tailor other questions that the review division may be particularly interested in taking advantage of this opportunity to talk to

you, hear from you about other aspects of a disease or trials that they may want to take advantage of, this unique opportunity to ask you about. And so, we have those additional questions sometimes.

For example, when we had a meeting on HIV/AIDS, we asked about patients' willingness to participate in cure research, to get a better understanding of that and how the risk-versus-benefit tradeoffs, potential benefit tradeoffs for the patients.

And active involvement of the patients in your -- what you have to tell us today is just what this is all about, and that's the value of this meeting for us. And it's been tremendously valuable. We try to capture in very detailed notes, and using a transcript, just what you tell us today in the words that you tell us so that we can reflect that in a voice-of-the-patient report. Takes us awhile to put that report together because we collect information from our docket as well, from the electronic docket. And we leave

that open for a little while longer.

Those reports are very useful for later reference by the review divisions. What we've learned in these meetings have also given us a basis for building on, where do we go next with this? How can we further build on what we hear in these meetings to develop maybe more systematic ways to collect information about the impact of the disease and the impact of treatment in clinical trials and in other ways?

So we think this program, which has been exploratory in the last couple of years, is really giving us ideas for a longer-term approach here. So, thank you again so much for being here today. And with that, I'm going to turn it over to Dr. Podskalny.

DR. PODSKALNY: Again, thank you for everyone who came and attended this conference today in person and online.

I'm going to present some medical facts about Huntington's disease. But I don't want people to think that we've lost sight of the fact

that Huntington's affects people, families, and over multiple generations, and that every day we carry our experiences from clinic and caring for Huntington's patients to work with us. And we empathize with everyone who struggles to understand and cope with Huntington's disease every day.

Okay, the standard FDA disclaimer. But, okay, here we go.

Huntington's disease is an uncommon disease. And the number of people it affects varies by geography, from country to country. But somewhere between 2 and about 20 per 100,000. It's an autosomal dominant disease, which means that if one parent carries the gene, each child has about a 50 percent chance of inheriting the gene and the disease, in most cases.

DNA testing is available. And if you have what's called an expansion on your fourth chromosome, and it meets a certain threshold of 36 or more CAG repeats, you're likely to have symptomatic Huntington's disease.

The typical onset of symptoms are between age 30 and 50. When we talk about Huntington's disease, we've somewhat arbitrarily defined juvenile-onset Huntington's disease as beginning below the age of 20, and late-onset Huntington's disease beginning at age 60 or older. Although chorea, the movement disorder, is the most recognized feature of the disease, cognitive impairment, problems with thinking, reasoning, memory probably are the first symptoms of Huntington's disease and probably the most common.

Behavioral changes can reflect other changes that occur in Huntington's disease, those in memory, things like decline in judgment, being inflexible, and decreased awareness of self-care needs. Psychiatric symptoms often involve depression, anxiety, irritability, and apathy. Obsessive-compulsive symptoms are present, but generally are less common.

Swallowing difficulties can be a big issue as the disease advances, and weight loss -- weight loss that's not just explained by the

movement or increased activity or decreased appetite or food intake.

Current treatments for Huntington's disease really attempt to reduce the symptoms. Motor symptoms such as chorea, we have treatments such as tetrabenazine. Many of you may know of Xenazine. Neuroleptic drugs are used outside of their intended indication, and these are antipsychotic drugs, to help suppress chorea.

Antidepressants are frequently used, nutritional support; speech therapy to modify diet and to assess swallowing; occupational and physical therapy to maximize physical function; and psychological counseling.

There are family and caregiver support organizations, but also professional services that are available; genetic counseling. Most of you who have received a diagnosis or who have Huntington's disease in your family have probably spoken to a genetic counselor. Many of you may have sought psychological counseling. The benefits and support of advocacy groups and

support groups, respite care and family support, and good communication.

And that's all I have for you today. Thank you very much.

MS. GIAMBONE: All right. Well, thank you to my FDA colleagues for your presentations.

And I forgot to do one thing earlier when we started, which is I wanted to make sure we introduced ourselves. So, could my FDA colleagues please introduce yourselves? I know you've already met some of them.

DR. UNGER: Good morning, everyone. My name is Ellis Unger. I'm the Director of what we call Office of Drug Evaluation 1. Our office oversees the Division of Neurology Products.

DR. DUNN: As I mentioned earlier, I'm Dr. Billy Dunn. I'm the Director of the Division of Neurology Products.

DR. BASTINGS: Good morning. I'm Eric Bastings. I'm the Deputy Director of the Division of Neurology Products.

DR. PODSKALNY: I'm Dave Podskalny. I'm

the Clinical Team Leader, Division of Neurology Products.

DR. GOLDSTEIN: I'm Susanne Goldstein, Medical Reviewer on the Neurology Team.

DR. BERGMAN: I'm Ken Bergman, Neurology Reviewer.

DR. KAPCALA: I'm Len Kapcala. I'm Medical Officer on the Neurology Team.

DR. MULLIN: Good morning again. I'm Theresa Mullin. I direct the Office of Strategy Programs in the Center for Drugs.

DR. COMO: Good morning, and welcome. Peter Como. I'm a medical reviewer in the Center for Devices and Radiologic Health in the Division of Neurological and Physical Medicine Devices. We review all of the neurological devices for the treatment of neurological disorders.

DR. XU: Good morning. I'm Leu Xu. I'm the Medical Officer with Central Biologics and at the Office of Cell and Gene Tissue Therapy. I'm a neurologist-in-training.

MS. GIAMBONE: Thank you so much. And

we have a few colleagues here.

DR. CHALASANI: I'm Meghana Chalasani. I'm also with the Office of Strategic Programs.

MR. THOMPSON: Graham Thompson, same office.

MS. VAIDYA: Pujita Vaidya, Office of Strategic Programs.

DR. EGGERS: I'm Sara Eggers, in the Office of Strategic Programs. And I'll be helping with the facilitation.

MS. GIAMBONE: Great. Thank you.

So let's go ahead and give you an overview of the discussion format today. Okay. So as I mentioned earlier, we have two topic questions that we're going to be reviewing. Topic 1 is on the symptoms that matter most to you. So here, what we're listening for is, what are your most significant symptoms? And how do they impact your daily life? What are you able to do or not able to do as fully as you would like, because of your symptoms?

And also, talk to us about how your

symptoms have changed, how they have affected your social interactions and your mood, and really how you experience your symptoms on a day-to-day -- you know, during your day-to-day life.

So I know we have a lot of caregivers today. We have a lot of patients here today. So we're really excited about hearing all of your perspectives.

Topic 2 is on current approaches to treating Huntington's disease. And in this topic, what we're listening for is, what is your treatment regimen? What are you doing to treat your Huntington's disease, both prescription therapies and non-drug therapies? How well are these treatments treating your significant symptoms? And how do you know that it's working for you or not working for you? What are the biggest downsides to your treatments? And finally, what do you look for in an ideal treatment?

So we're going to start each discussion topic, hearing from our panel of patients. So in

just a short while, we'll hear from Panel 1. And the purpose of the panel statements are to really set a good foundation for our greater discussion today. So each panelist will have about five minutes to speak.

And I've had the honor and the pleasure of speaking to all of our panelists over the last week. And you're extraordinary, and we really appreciate that you've put these thoughts down and you're going to be sharing them with us.

So, our panel reflects a range of experiences with Huntington's disease. And as I mentioned, we have caregivers and patients here to share experiences today.

And then we'll broaden the dialog to include other patients and caregivers in the audience. So the purpose of this is to hear from more patients and caregivers in the audience, to build on what you've heard from the panel. So, we'll ask some questions along the way.

And if you're comfortable to do so, you can raise your hand. We'll have some microphone

runners here. Or you can just let us know that you're interested in sharing a comment. And we'll have a microphone runner come to you so we can hear from you also in the audience. And if you can, please state your name before answering.

Throughout the meeting, we'll have some polling questions. And the polling questions are not a scientific survey. They are meant to just aid the discussion. And it's completely voluntary. My colleagues will be passing out the clickers in just a little bit, and we'll test it out in just a short while to get you comfortable with how these work.

And we also, for those of you joining us on the Web, you can answer these questions through the Webcast. For the polling questions, we ask that only patients and caregivers please respond, please. And I just mentioned our Webcast participants. For those of you on the Web, we can't see you, but you are a very big part of our meeting today. Thank you for joining us. We are going to be turning to you throughout the meeting

to hear what you have to say.

We will also take some phone calls also later in the meeting. So, please continue to provide your thoughts through the Webcast. And although we can't read through all of them or summarize them all today, just know that all of your comments will be incorporated into our summary report that Theresa mentioned earlier. And as I mentioned, we'll occasionally go to the phones also.

The other way that you can contribute to this meeting, and that we strongly encourage you to contribute to this meeting, is to submit your comments through the public docket. The public docket will be opened for two months after the meeting. So it will be open until November 22nd. And it's really a way for you to continue the dialog and continue sending in your thoughts and comments. So please be sure to do that.

All of the comments that you submit to the docket will be summarized and incorporated into our summary report. And anybody is welcome

to comment, not just patients and caregivers and patient representatives.

Also, I want to share some resources that we have here at the FDA that you may have already interacted with. The FDA Office of Health and Constituent Affairs, and their contact information is here and also on the slide deck that is posted online. And the CDER, Office of Center Director, has a group within it, the Professional Affairs and Stakeholder Engagement Group, also known as PASE, which I know some of you have worked very closely with as we planned for this meeting.

So I want to go over a few ground rules for the day. And I know our panelists have heard me say these a few times, so I'm so sorry to repeat them again. But really, this is your day. Today is the day for the patients and caregivers to share your thoughts with us. So we encourage you to contribute to the dialog. Also looking to hear from patient representatives. But FDA is here to listen, as is industry and academia and

other government agencies.

So we know that this meeting will be very valuable to those of you that are here that are not patients or caregivers. But we ask that you stay in listening mode.

The discussion will stay on symptoms and treatments. So again, we're going to do our best to stay on topic and stay on time. Any other thoughts that you want to share outside of the scope of these topics should be reserved for open public comment or the public docket.

The views expressed today are personal opinions. And on that note, respect for one another is paramount. And finally, at the end of the day, we'll be passing out some evaluation forms. So please let us know how the meeting went for you today. Let us know what worked or didn't work, and we read those very carefully so we can improve upon these meetings.

Okay. And before we move on to the polling question, I do want to say that this meeting is being recorded and transcribed. And in

about a week's time or so, the meeting recording will be available in addition to the transcript. So a short while after the meeting is over.

Okay. Perfect. So, let's try out the clickers and do our first polling question. All right. Where do you live? Press A for within Washington, D.C.

and metro area; or B, outside of the metro area. And again, those of you on the Web, please also participate.

(Pause.)

MS. GIAMBONE: Let's see here. Okay. And let's see what the results are, hopefully. Okay. Okay. That's okay. But I know from talking to many of you that a lot of you have traveled to come here. So thank you for doing that. Thank you for all of you that traveled from out of state to come here. But thank you also to all of the locals for being here.

Okay. You're doing it. Okay. Oh, well, look at that. Most of you came from outside the metro area. That's amazing. Okay.

Next. Have you ever been diagnosed as having Huntington's disease? Press A for yes, or B for no.

(Pause.)

MS. GIAMBONE: Okay. Eighty percent of you said no; twenty percent of you said yes.

Okay. Next. Are you male or female?

(Pause.)

MS. GIAMBONE: Okay. So we have 20 percent male. And it looks like the majority of you that are responding to the questions are female. Okay.

Now, there's a lot of choice here.

(Laughter.)

MS. GIAMBONE: For age, let's do A, younger than 20; B, 21 to 30; C, 31 to 40; D, 41 to 50; E, 51 to 60; F, 61 or greater; or G, not applicable.

(Pause.)

MS. GIAMBONE: I'm not sure why we have a "not applicable" here. That doesn't make any sense, does it?

(Laughter.)

MS. GIAMBONE: I'm sorry about that. I don't know why that's there. I'm so glad to see that nobody picked that one.

(Laughter.)

MS. GIAMBONE: Okay. So, it looks like the majority of you that are answering either as yourselves or on behalf of a loved one are in the age group of 61 or greater. And we also have 41 to 50, and 31 to 40. So it looks like we have a good mix of different age groups.

Okay. What is the length of time since your diagnosis? A, less than five years ago; B, five to ten years ago; C, ten to twenty years ago; D, more than twenty years ago; or E, I'm not sure.

(Pause.)

MS. GIAMBONE: Yes, please. Yep. So caregivers and patients, please respond to these questions.

(Pause.)

MS. GIAMBONE: Okay. So it looks like the majority of you responding are either speaking

on behalf of a loved one or yourselves, diagnosed five to ten years ago, followed by ten to twenty years ago. And then we also have a few folks that have been sort of newly diagnosed, less than five years ago. And it looks like we also have some more than 20 years ago. Okay.

Do you have a family history of Huntington's disease? A, yes; B, no; or C, I'm not sure.

(Pause.)

MS. GIAMBONE: Okay. So it looks like nearly 75 percent of you responding have a family history of Huntington's disease, followed by 25 percent that do not. Okay.

All right. And can we get a summary of what we heard on the Web?

(Pause.)

MR. THOMPSON: Results on the Web are pretty similar to what we had in the room.

MS. GIAMBONE: Okay. Great. Thank you.

All right. So on that note, we're going to begin with our panel discussion. So we have

our Panel 1 table right here. And may I ask that each -- right here, right here. Okay. Thank you. You know, I can't see the name, the card too well. So for some reason, I figured Topic 1 was over here. But thank you for reminding me.

So, we have our panels here today. And again, as I mentioned earlier, they've been working so hard over the last week-and-a-half to put their thoughts down and to share them with us. And they have been working -- you know, every day I've been talking with you and getting your feedback. And I know you've worked very hard on this. So, thank you so much. We appreciate it more than you know.

So, on that note, let's start with Colleen. Okay? And if you could please introduce yourselves and go ahead and read through your comments.

MS. WALSH-BARNES: I'm Colleen Walsh-Barnes.

(Pause.)

MS. GIAMBONE: Could you start over,

Colleen? We couldn't hear the beginning of it.

(Laughter.)

MS. WALSH-BARNES: Oh, well. And I'm an electrical engineer; that's embarrassing.

(Laughter.)

MS. WALSH-BARNES: I was caretaker for my husband and my two sons, Miles and Jason. Miles was diagnosed symptomatic at age 27, and Jason was 28. Miles died two years ago. He was 39. Jason, 40, is in a nursing home. He has lost all ability to control his body, and he wants to die.

Miles and Jay were complete opposites. Even a neurologist was surprised they were brothers. This is significant because HD wreaked the most havoc with their respective strengths. Miles as an introvert, gifted in academics and a good athlete. Jason was outgoing, extraordinarily athletic, and brilliant.

Miles's cognitive processing was the most impacted, while until the end his movements consisted of toe and finger twitching, facial

tics, and randomly touching his head. The first indication that Miles had HD occurred when he was 17. He had easily mastered all his honors math classes until he took calculus. Then he failed miserably. By the time he was diagnosed, he could not subtract. Early on, he could no longer plan an activity, anticipate an outcome, or reason logically.

He continued to ride his bicycle 20 or more miles a day, yet he would fail to stop at red lights, maintain a safe distance from cars and people, or ride where it was permitted.

As he began the end stage, he would wander. Even with keyed locks inside my doors, he once took my car keys, fell 12 feet out a window, and tried to drive. Because he no longer could process putting his foot on the brake while putting the car in gear, a tragedy was avoided. In the end, he could no longer retrieve words. So, any positive response became "Cool," and any negative response became "Fuck you."

Jason's motor control and his ability to

interact with others were the most impacted. He lost the ability to walk naturally, early on. His disturbed gait resulted in a clinical DUI from a New Jersey state trooper, even though four breathalyzer tests indicated he had not consumed alcohol. The deterioration of his motor control resulted in being taken to the police departments regularly.

Unlike Miles, he was cognizant of his declining abilities, so he surrendered his driver's license very early. His cigarettes would randomly fly out of his hands. When a police officer thought Jay had thrown a lit cigarette at him, he threw him to the ground, stood on him, and threatened to break his legs if he didn't stop moving, which of course he couldn't do.

Because he retained the ability to process reality, he became increasingly angry. He was fully aware of how people viewed him and what his future held. Given that people think HD victims do not understand their surroundings, he was harassed and subject to disparaging remarks.

This, combined with the loss of impulse control, resulted in many psych ward unit stays.

As a result, he could not be around most people. However, until very recently, when he stopped speaking, being able to speak, he was often asked to troubleshoot HVAC systems, which he successfully did.

Contributing to his frustration, depression, and anger was not knowing when an ability would be lost. One Memorial Day, Jason could swim fabulously. By Labor Day, he jumped in a pool and could not even tread water.

Woven through the incidences that I have shared are obvious examples of psychiatric problems. The psychiatric problems stole their lives. Both lost their wives early because of the inability to contribute to or to understand the relationship. Their behavior became too difficult for their wives. Sadly, Miles's son was kept from him for three years after his marriage ended.

Both began having random gay encounters, which their psychiatrist attributed to HD.

Jason's lack of impulse control created bad situations, but his psychiatric issues caused violent reactions. He could not be reasoned with.

Miles's perseveration kept him locked on one thing for days, like telling me every 15 minutes for 30 hours straight that he couldn't have an orgasm. Miles's inability to reason caused him to integrate the outside world into his reality. When hospitalized with a pedophile, he started calling the police to tell them he was a pedophile so he would not hurt another child.

Jason reacted to any perceived slight with violence. Most difficult for Jason was the inability to work. He had five years with perfect attendance and rave reviews. But after that, things fell apart. His movements made him a safety hazard, but his impulses resulted in unacceptable behavior, like leaving inappropriate messages on the CEO's voicemail.

The disease inhibited them from the very beginning to be themselves or a functioning man. Until the end, in which there are no good days, a

good day would be one that didn't include an outburst, or one where they fell three times instead of six, or they were able to eat, or were not committed or arrested.

The disease symptoms do not come and go. Abilities will vary on a day-to-day basis. But once a particular ability, like walking, is gone, it never comes back. A bad day would be caused by not eating 5,000 calories a day, stress, or lack of sleep. When the disease became obvious, strangers avoided them on the street. As it progressed, most friends and family stayed away.

(Pause.)

MS. WALSH-BARNES: Once in a nursing home, they lost all contact with everyone other than me, their sister, and her family. However, to their credit, Jason's AA group continued to stay connected. Thank you.

MS. GIAMBONE: Thank you so much, Colleen.

We have Marie next.

MS. CLAY: Hello. My name is Marie

Clay. I'm from Rome, New York. And this is my daughter, Lori.

(Pause.)

MS. CLAY: On behalf of my daughter Lori, she would want you to know that her dreams at the time of her diagnosis 16 years ago were to be a hands-on devoted mother and to follow her dream as a chemical engineer.

As I begin to tell you her story, I want you to imagine an ice cube melting, just like my daughter's precious life. I was Lori's caregiver until a couple of years ago. She decided she wanted the closest thing she could have to a normal life. She moved in with her fiancof 19 years and their son. Today she can't even write a grocery list, handle a monetary transaction, or help her son with homework.

Lori was a very meticulous person. She took pride in her appearance. But today she needs assistance with brushing her hair, teeth, bathing, dressing, having her food cut up, pouring a cup of coffee, reaching for a plastic glass or a plastic

dish in the cupboard, and making decisions -- everything that we don't give a second thought to.

Education was extremely important to Lori. Lori cannot even focus to read a newspaper today. This devastating diagnosis of inheriting her father's defective gene was only made worse by knowing her son may inherit this deadly gene. He watched his grandfather decline slowly until his death in 2006. My grandson has to live with the knowledge that he may end up like his grandfather and mother.

He hasn't had a typical kid's life. No trips to the park with friends, movies, or spending the night with friends; no after-school activities, because he has to be with his mom when his father goes to work. He's hesitant to have friends over because of his mother's condition.

I think with him being almost 18 now, he handles things differently. He holds his mother's hand when they go places. How many kids at this age hold their mother's hand? I admire my grandson. I've never seen him cry, ever.

Over the years, I have used Lori's birth date, August 6th, as a guide to her progression. Every August 6th, I can't help but look back over the past year and reflect on her progression. Her balance declines more and more. She has fallen several times and needed staples in her head each time. Lori's speech is slurred and getting worse.

I know the day will come when I will no longer hear her sweet, delicate voice. At times on the telephone, I need my grandson to interpret what she is saying, and that is frustrating for her and stressful. The constant chorea movement wears her out to the point that it's an effort for her to even speak.

I refrain from informing her on family issues. She doesn't need the stress to compound her issues, and she can't do anything about it anyway. We try to keep conversations positive and calm surroundings. Friends have faded away. People are uncomfortable seeing her like this. It's not the friend they remember.

There are very few outsiders in Lori's

life. There's more and more apathy with Lori, and one day runs into another, it seems, with her. Lori and I have always did mother-daughter activities over the years, and they have dwindled. The biggest activity we've done in the last year was sitting with her to put photos in an album. Lori watched as I put them in the album, trying to at least laugh at the photos.

Spontaneity no longer exists. Either we have to plan ahead or days before, depending on the activity. Last-minute activities cause stress. Her poor balance plays an important part in what she can take part in. Crowded venues do not work. She falls into people. People stare and make remarks.

Lori has been unable to go out on her own for many years. It isn't safe for her, and it's depressing to lose that independence. Her delay in processing thoughts, verbalizing them with slow speech, is frustrating for her. When we have a conversation with her, we have learned to use short, precise sentences, how to give her time

to absorb the information, time to process and verbalize her answer. A conversation that takes you and I 30 seconds takes my daughter several minutes.

Remember that ice cube? It's halfway gone from melting. So is my daughter. Lori also suffers from dystonia, which between the muscle spasms that can last for days, poor balance makes for a very rough, uncomfortable day and night for her. Lori is never home alone. It's too risky.

The HD symptoms get her frustrated, so that makes for a very bad day. When she can't explain something to someone or accomplish the simplest of tasks, it's another frustrating day for her. Watching her favorite baseball team, the Baltimore Orioles, keeps her spirits up.

My daughter is fortunate to have a loving fiancof 19 years and their son, who is 17. Lori's son has grown up to be a caregiver. She is totally reliant on them. And she's reliant on God. She has great faith. And so many with HD do not have that support.

My daughter deserves the treatment and medication to give her a better quality of life. Lori doesn't have the option of chemo or insulin. As of today, she can't even hope to make the claim that she's a survivor. My daughter is that melting ice cube. Thank you very much.

MS. GIAMBONE: Thank you so much, Marie.

Julie.

MS. ROSLING: My name's Julie Rosling. I'm from Orange, California. And we did not have any knowledge of previous generations having had Huntington's. And so, when this all of a sudden came forth, none of us knew what it was all about.

And I went till 2009 when I was very symptomatic at that point, and I started falling down in the pharmacy where I worked. And somebody looked at me then and diagnosed me as having Huntington's. I was told that I was certainly disabled and could not work any longer as a pharmacist. And it really upset me.

Now, I see I'm 73 years old, and I have all the symptoms that are out there, yet have

nothing to do with chorea. This has been the biggest thing is that there are so many different types of symptoms that are out there that are not currently being addressed. And I think this is where you guys are going to help us immensely, by getting the right stuff next time.

Now, the symptoms that I have, because I don't have chorea, I still have a lot of imbalance and uncoordination. I fall all the time. I can't go up or down stairs. And most of the times that I can, I'm hanging onto the handrails that are at my house to keep from falling. I can't drive anymore. I spend about 90 percent of my time during the day just sitting so I can do anything at all.

My fear of choking now has gone up considerably. And I have to have water with me, always water, or I'd choke, I'm sure. My mouth is dry. My dental health has suffered. I often bite inside of my mouth. I have little lesions where I've bit them.

The thing that's the most important

thing to me is that my symptoms are affecting every system in my body. We're no longer talking just about CMS or brain disease or anything like that. It's talking about systemic things now, with GI, and GU, urgency, frequency, insomnia, where we can't sleep at night due to the leg and arm movements and the restlessness that go with that.

Some of my behavior things were piano lessons, oil painting, and playing with my cat, Portia. Now I can't play Chopin on the piano anymore. I can't walk to the corner and back. And my long list of very, very favorite activities is like gone, this due to this devastating disease.

I don't have best days anymore. It's really hard to say that, but it is true. I don't have best days anymore. On the worst days, I have days where I don't get to bed on time, wake up during the night and can't go back to sleep. Falling on the bed or tripping in the dark. You spill food while you're sitting at the table, or

your plastic drink cup, I have to have a special lid on it so I don't spill stuff.

My hearing is going, and I don't remember who else has said that, but it does make conversations difficult. And people do misunderstand me. This -- there's nothing I can do. Nothing I can do or hope for in terms of medication, balance issues, or losing more memory than I have already.

My condition and symptoms have changed over time. Starting asymptomatically, like I did, and not having chorea, but ending up with systemic issues instead, this is where we have to have some new definition of the symptoms that are being -- that are evolving out of this disease.

There are no days that my symptoms stop. It's been an ongoing situation. My symptoms have never gotten better; they're getting worse every single day.

Huntington's has affected my social interactions because the issue seems to be due to slurring of words and because I also can't hear

very well, too. And so, there's misunderstanding all the time we're going along, when your thinking no longer corresponds with my speech. As a pharmacist, this is very, very distressful, to know that this has been taken away from me by something which I didn't know was there.

I am a living example of what this disease is all about. No one wants to be reminded of their own mortality by seeing me. Huntington's has significantly affected my mood. It's variable. My depression is worse, knowing that I will never get better.

Somewhere along the line, my gene-positive Huntington's situation changed my demeanor and behavior. I start losing control of the situations and talking loudly. Now I lose patience with people who say that they're going to do something for me and don't. I also get angry with people that don't tell the truth, and I become stubborn if I feel I'm correct about something and somebody else is wrong.

I don't know what to say, other than all

of these outside stresses cause me to be more depressed, distressful, upset. Why is it our fault when it's the genes have made us do this? Thank you.

MS. GIAMBONE: Thank you, Julie.

Next, we have Denise.

MS. HUDGELL: Good morning. My name is Denise Hudgell. And I'm from Council Bluffs, Iowa. I'd like to thank you for this opportunity to share a story. A little bit about myself. I've been an RN for 18 years, and most of my career spent in psychiatric nursing and home health. But most importantly, I'm the mother of three children.

My son, Aden, just celebrated his 10th birthday this past Saturday. He's your typical 10- year-old boy that loves Hot Wheels, classic cars, big machinery, animals, and he loves to sing everywhere to every song that he knows. I was looking at pictures from his birthday party last year and this year. And there are so many changes. He's skinnier. He's pale. He has dark

circles under his eyes, and he's tired, often the ravages of the awful juvenile Huntington's disease.

Aden has been symptomatic since the age of four. He started with behavior issues, anxiety, walking on his toes, stiffness in his arms, and changes in his handwriting, which he had just begun to be able to do. After two years of trying to get him seen by a doctor and countless pieces of information to support the need for an HD test, at the age of six, we got the devastating news that Aden had a CAG repeat of 102 -- 102, a number that I will remember forever, a number that changed our lives.

There are things that Aden loves to do, but can't anymore due to his symptoms, such as ride his bicycle, play on a soccer league, play on the playground with his classmates, slide down the slide, and just ride in a car. A few-minute car ride is challenging for us due to his neuropathy. It causes so much pain and the pins-and-needles feeling that he gets all over his body.

In fact, Aden expends much of his energy throughout the day standing for everything he does. He stands to watch TV, to eat, in the classroom. And if he's not standing, he's pacing the floor. His neuropathy is partially kept at bay if he stands or walks.

Because of all the energy he uses during the day, he becomes easily fatigued and has a shortened day of school. He's very isolative at times and doesn't like to leave the house if he doesn't have to, since the car rides are too difficult for him. His 44-year- old dad is in a nursing home in end-stage HD. And the nursing home is over an hour away. So Aden has not been able to see his dad in six months due to the car ride.

I've spoken with many other JHD families, and their children have the same issue with the neuropathy and the painful car rides.

Aden has OCD, gets frustrated easily, agitated, irritability, anxiety. He perseverates and tells me every day, "Mommy, I hate JHD," and

becomes very tearful -- very difficult as his mom to hear because it makes me feel helpless.

You would think after all the energy he uses during the day, he would be tired and would crash at night into a deep sleep, but that's not the case. When it's time for Aden to lay down, that is when his neuropathy is at its worse. He violently thrashes around on the floor, trying to itch while he is trying to go to sleep.

He hasn't slept in a bed in over two years because the bed is too soft. He said the hard surface for the itching makes it better, but the itching is still horrible.

On average, we get about three to four hours of sleep at night, but there are many nights we don't sleep any, and we've been up for 36 hours at a time before he crashes to get some sleep. Last week, we slept a total of 25 hours.

Once Aden falls asleep, he continues to toss and turn all night, wakes up several times, and gets up at his usual time in the morning. So he's tired all day. After several different

doctors and meds, including diphenhydramine, antiepileptics, melatonin, Neurontin, and trazodone, none of them have worked.

And when we don't get sleep, I still go to work the next day and Aden still has to go to school. We don't receive any respite care because he's been on a Medicaid waiver waiting list for two-and-a-half years and we still remain number 605 on that waiting list, probably making it another two years until we come off the list.

Aden has been having an increase in issues with balance and gait. He falls all the time. His elbows take the brunt of the injury due to his dystonia and the way he holds his arms. He's had to go to the ER on a few occasions to get staples in his head. His right knee and ankle are collapsing. His knees rub together when he walks. So he has the beginning of friction ulcers. He also has the beginning of a pressure ulcer on the top of his foot where his opposite heel rests at night.

His back now has some scoliosis,

secondary to his dystonia in a few areas, and his ribs are pulling away from his sternum, so he has a rather large indentation in his chest.

When I return home, Aden will be scheduled to have a peg tube placed to assist in receiving the nutrients that he is lacking due to the increase in his chewing and swallowing difficulties. He recently was diagnosed with a dilated esophagus, and I've had to do the Heimlich maneuver on him on three separate occasions. The fear that I saw in my son's face was unbearable.

All of these symptoms, as well as seizures and countless others, have been progressing quickly over the last two years, especially the last eight months. It is scary to see the changes in your child, and you can see the worry and fear on Aden's face when he talks about JHD.

But I'm not only here for our family. I'm here for all the other families that have children fighting this wretched disease. I'm here for McKenna, Aden's half-sister, who is

wheelchair-bound and fighting seizure activity all day, every day. For Jacey, who has given a voice to these kids for the past several years, and her sister Erica; for Meg, who has been in the hospital for eight weeks because her sleep and itching problems. And now her mom is fighting to get the help she needs to care for her when she is sent home.

For Cameron, Ethan, Gabe, Luke, Corey, Sara, Chris, and too many others to name -- our children deserve to have a long, happy, pain-free life. They deserve to make a lifetime of memories. They deserve to go to prom, learn to drive, graduate, go to college, get married, and have children. They deserve to live.

Please help me save my son and all the other children with JHD.

MS. GIAMBONE: Thank you so much, Denise.

We have Katie next.

MS. JACKSON: My name is Katie Jackson, and my husband was diagnosed with Huntington's

disease when he was 27 years old. I don't know what has been more terrifying for me, watching Huntington's disease take everything away from my husband or knowing that our three children have a 50 percent chance of inheriting their father's same fate.

I speak for many families, including my own, when I say we fear the most depression, anxiety, irritability, aggression, all of the psychiatric symptoms, all of which wreak havoc on our loved ones and our family members. There's also apathy, loss of judgment, loss of memory. The significant symptoms associated with Huntington's disease, the list is long and devastating.

Before Huntington's disease entered our life, my husband was an outgoing, amazing husband, a very active, loving father, and a sheriff's deputy. I'll never forget the day the department placed a badge on my husband's chest. He was so proud to live a life of service protecting the innocent.

Mike has always been the fun guy. We had parties at our house almost every weekend. Mike loved being around people, and people loved being around Mike. Fast-forward to today. My husband is 35 years old. My husband most days barely gets off the couch. He suffers from chorea. He has trouble with his gait. His speech is so slurred, oftentimes you can't even understand him. He chokes every time he drinks water. He has to eat very slowly so he doesn't choke on his food.

We get stared at every time we go in public. People constantly make comments at my husband about how he shouldn't be drunk out in public with our children. My husband hasn't had a drink of alcohol in over five years.

One time, we took our kids on vacation. We picked up our kids from the camp at the resort. A security guard walked up to us in front of our children and a ton of people and told my husband he had had enough for one night and he needs to get back to his room. It was so embarrassing for my children and my husband.

The stares and comments and judgments constantly given to us by the public is something me, my children, and my husband have had to get used to. As you can see, the social impact on our family is great.

A couple of weeks ago, my husband decided he would go to my daughter's soccer game. Right away, my daughter scored a goal. My husband was so excited, his movements intensified. I looked over at the opposing side of the field to see one parent mimicking my husband's movements. Another parent was pointing and glaring at my husband. This happened right in front of my 12-year-old daughter. Talk about an exciting moment taken away from a child due to ignorance and lack of compassion.

These are just a couple of stories of the hundreds that I could tell you, but I only have five minutes. So let me get back to the significant symptoms of Huntington's disease in my mind.

The most significant symptoms of

Huntington's disease are the ones you cannot see. These are also the ones that are often overlooked because you physically can't see them. But Huntington's disease families, we experience and feel these symptoms every single day.

I am talking about the psychiatric symptoms associated with Huntington's disease. To see my husband, once so social, happy, full of life, sitting on a chair staring at a TV all day long is heartbreaking. To see my husband battle with depression and severe anxiety is so hard on our whole family. My husband, once a fun guy, now often refuses to ever leave the house because it sends him into a panic, the thought of it.

The anger and irritability and behaviors is so hard on our children and our family as a whole. We desperately need help with the psychiatric and cognitive symptoms associated with Huntington's disease. Fortunately, in my case, these features have never been so bad that I fear for mine or my children's safety, but I know many families who live in fear daily.

My husband asked me before I came here, "Why is there still nothing they can do for us?" I told him, "The science we've been hearing about has to, you know, prove safety so it doesn't hurt you." He looked at me and said, "Katie, I am dying, and there's nothing anyone can do for me. I'm not going to be around to walk our daughters down the aisle when they get married, and I'm going to be lucky if I get to go to one of their high school graduations.

"The worst part, Katie, is the kids may have to go through this, too." As he told me this, his eyes filled up with tears. Whenever we talk about the children, he gets very upset. He said, "I wish I could try all these new therapies. I want to fight for my children."

My husband has participated in five clinical trials and studies. I know he isn't only participating for himself; he is participating for our children.

Our loved ones are suffering so bad and losing every bit of their quality of life. Time

is something we simply don't have. The suffering is great, and the Huntington's disease genetic fate haunts us generation after generation. I cannot protect my children from the genetic fate of Huntington's disease, but what I can do is fight for them. I'm a mother fighting for her children. And I'm sitting here in a desperate plea for you all to help us.

I was there when my babies took their first breath into this world, and I'm going to fight to not be there when they take their last because of Huntington's disease.

MS. GIAMBONE: Thank you so much, Katie.

So, what I'd like to do is give our panel a surround of applause.

(Applause.)

MS. GIAMBONE: I think you're all so courageous. And this goes to our Topic 2, panelists 2, you'll be speaking in just a short while. But you are incredibly courageous for coming here today and sharing these thoughts with us. So, thank you for doing that.

So, what I'd like to do now is to just ask the audience here today and the other patients and caregivers in the audience, what you heard in the panel, does that resonate with you, too? Does that sound like your experiences also? I see a lot of head nods. So it sounds like there's a lot of shared experiences here.

What we'd like to do next is another polling question. So if you could get your clickers out. And just a reminder that, you know, we only want patients and caregivers to respond to these questions. And if the patient represented -- if the patient is responding to the question, then the caregiver doesn't have to so we avoid double-counting. However, if the patient -- or if you need to help and do the clicker, please feel free to, for the caregiver, you can go ahead and respond on behalf of the patient.

So let's start with our first polling question. Of all the symptoms you have experienced because of Huntington's disease, which do you consider to have the most significant

impact on your daily life?

And you can choose up to three: A, cognitive impairment, such as difficulty concentrating or difficulty with complex tasks; B, chorea; C, fatigue; D, unsteady gait, difficulty walking; E, depression or anxiety; F, slurred speech; G, weight loss; H, difficulty swallowing; or I, other symptoms not mentioned on this list.

(Pause.)

MS. GIAMBONE: Okay? Okay. So, it looks like cognitive impairment, nearly two-thirds of you answered that that's the most significant symptom, followed by depression or anxiety. Again, we also have unsteady gait or difficulty walking. We have chorea and other symptoms not mentioned. So we'll be sure to come back and hear about that also.

Okay. So, what do we have on the Web?

MR. THOMPSON: On the Web, similar numbers. We have 80 percent saying cognitive impairment; 56, depression or anxiety; 43, unsteady gait; 31 percent say difficulty

swallowing; and then everything else is 20 percent or less.

MS. GIAMBONE: Okay. Great. Okay. So, what I'd like to do now is spend some time on some of these symptoms that you've highlighted and hear from those of you in the audience, and also any panel member that wants to participate also.

And can you talk to us a little bit and tell us how you experience the cognitive impairment? Describe that symptom for us. Is there anybody that would like to -- okay.

(Pause.)

MR. NIERENBERG: Good morning, and thanks for this panel. My name is Roy Neirenberg. I was a software developer in Washington -- in San Francisco. Before that, in the '70s, I was a regulatory lawyer in Washington. So, being at a regulatory agency is kind of fun.

I thought I had a perfect job and it would last forever. But when I was diagnosed with Huntington's disease, we talked to a cognitive neuropsych, who said we'd better bring someone

else in the family to run the business because I wasn't balancing checkbooks -- I was balancing checks in my business. I was making bad decisions. I hired someone from Yahoo who I thought could take over the company, and I couldn't -- when he didn't -- when he didn't perform, I couldn't fire him.

My daughter came into the business. And after awhile, when she wanted to move to smaller quarters, my -- there was no desk for me there. That's how I retired from my software company, which I founded 30 years before.

MS. GIAMBONE: Thank you, Roy. Thank you.

Yes.

MS. JENNINGS: Thank you for meeting with us today. My name is Arlene Jennings. I'm here with Charlie. Next week, we will have been married 21 years. Happy anniversary, Charlie.

His main issue is his inability to concentrate. Charlie is one of six siblings. Four have had Huntington's. Two of those have

passed away. One took her own life because she couldn't stand the idea that she might have it. And the sixth person has not been tested.

Charlie was a land surveyor in his career. And being a land surveyor, you have to be very precise and clear in your thinking and your writing. And Charlie is now not able to balance his checkbook or count change in a timely manner.

There are no meds for this part, for these symptoms. But Charlie does take meds for anxiety. And we both are very glad that there are meds for anxiety because that does make life easier for us. Thank you.

MS. GIAMBONE: Thank you so much.

Is there anybody else that would like to talk about how the cognitive impairment is bothersome and how it's impacting your life?

MS. SALDANA: Hi. My name is Frances Saldana. I'm President of HD CARE. It's a support organization under the UCI MIND. And the father of my children had Huntington's. All three of my children inherited juvenile onset. And what I

heard from the panelists is everything that my late husband and my three children experienced in so many different ways.

Whereas my youngest daughter, who passed away five years ago, was just a sweetheart, she suffered tremendously from the late onset -- or the late stages when she was having seizures so severe that when her jaws clenched, her gums would bleed. Terrible infections. She could not resist any kind of infection at that late stage.

My son, however, was -- it was totally different. He's in the late stages of Huntington's right now. He probably has probably a year left, I would say. He's been in a care home for eight years. Very combative, aggressive. You just couldn't stop him. What he wanted to do was what he was going to do. In fact, Dr. LaVonne Goodman actually rescued him in Seattle one time when he just ran away -- ran away to Paris, France, at the age of 16, without me knowing about it.

And that's not even the worst part with

when they get this kind of behavior. It's when I have heard other stories from other family members where they will actually, you know, burn the house down or kill somebody else in their family.

So, as tragic and as horrible as Huntington's is with the symptoms and everything they suffer -- not being able to walk, not being able to swallow -- the psychotic part of it is the most traumatic to the family. This is what causes divorces. And when that happens, the HD person will not be able to be taken care of the way they should be taken care of, because care homes don't take care of them that way.

And that's really tragic. That's what happened with my son. When he was still ambulatory, no care home in Orange County would take him, and he wound up in Watts for five years. Fortunately, he cannot walk anymore, so he's back in Orange County, where I can see him every day and have a private caregiver for him in the care home.

MS. GIAMBONE: Thank you, Frances.

Thank you.

And we'll take one -- okay, we'll take two more comments here.

MS. SMALL: My name is Nancy Small. I'm from Upper Black Eddy, Pennsylvania. And my relationship to Huntington's disease is as a gene-negative family member, who -- and I'm here really representing my grandfather, my mother, my brother, my niece, and my great-nieces, who all of them except the great nieces have had Huntington's disease or have it.

But I think of Huntington's disease as like the epitome of the family disease, because it will be a defining part of my life from birth to, hopefully not until I pass, but potentially till then. And of all the symptoms that I think have had the most significant impact on my life, I think they are the behavioral and psychiatric symptoms, the ones that are less evident and less able to be quantified. And they're the ones that are most misunderstood or certainly have been in my 58 years.

My short story is that I had a very unsettling relationship with my mother during my teenage years. I hadn't known my grandfather because he had what was called Montgomery nerves and had been closeted away from everyone, including the family. I received -- my high school received a call a couple of days before my graduation to tell them to get me home. I arrived home to find ambulances and police cars, and my father passed away. He was not Huntington's affected. He had committed suicide.

My mother was hospitalized because of smoke inhalation at the time of her husband's death. It was at his funeral that I learned from a family member, my mother's brother, that there was this thing called Montgomery nerves, also known as Huntington's chorea at the time, and that my brother and I were at risk.

Years later, after the marker, and then the gene identification, I was tested because at the time I was a single parent with three children at risk. And my brother was beginning to show

some signs of the disease. He ultimately was tested.

His reaction to being, finding out that he was gene positive was absolute relief. He had held 10 jobs in three years and finally was no longer able to hold any job at all. He was college educated, graduated from the University of Richmond, a very smart man. His wife walked out on him and took all the children. He was left bankrupt, with nothing. She begged his family for money, and they all turned against my brother.

So, on and on, everybody's stories are the same and they're all different. But it's all about the impact on the family.

MS. GIAMBONE: Thank you.

MS. SMALL: And so much of it has to do with the behavioral and the psychiatric symptoms. And I've just listed some of the ones as I's.

MS. GIAMBONE: Thank you.

MS. SMALL: Irrationality, irritability, impulsivity, lack of initiative, inattentiveness, and ultimately, impact on the family.

MS. GIAMBONE: Thank you. Yes. Thank you very much for sharing those with us. Okay. And you touched upon the chorea, and we're going to go onto that in just a second.

Let's take one more comment back there. Okay. We'll start. Okay.

MS. THOMASON: This is a picture of my son, Randy, as I found him in May of 2013, unconscious and barely breathing. Anger, physical violence, depression, anxiety, apathy, perseveration, compulsive spending, paranoia, delusions, hallucinations, and suicide are the so-called "soft symptoms" of HD, but there's nothing soft about them.

These symptoms have ripped my family apart, causing endless heartache and suffering. I lost my husband and two brothers-in-law to HD. But these symptoms took them away from us long before they died. I'm now losing my only child, my greatest love, to the ravages of HD.

These symptoms ended my marriage and caused my son to be able to see his daddy only

under supervised visitation. They caused my brother-in-law to put a gun in his mouth and shoot and kill himself. These symptoms came close to ending my son's life three years ago and again two-and-a-half years ago.

They caused my son to be committed to a state mental hospital for a year, a hospital where he was beaten by a guard and put in isolation, a hospital that said if my son's symptoms were caused by HD, he didn't belong there because HD is a medical illness, not a mental illness, a hospital where the psychiatrist looked at me and said, "Do you know what chorea is?"

My son does not have chorea, though. Neither did his father, nor his uncle. People can live with chorea. It doesn't rip families apart or cause people to be placed in psych wards, mental hospitals, and jails. It doesn't cause people to attempt or complete suicide.

We need recognition of these soft symptoms and treatments that are approved for them as part of HD. We need a cure. We need clinical

trials to be fast- tracked.

I know my son is running out of time, and I don't think I can bear to lose him.

MS. GIAMBONE: Thank you so much.

MS. THOMASON: As it is, I pray every day I never find him again as I did that day two years ago.

MS. GIAMBONE: Thank you for sharing those thoughts with us.

Let me check in with the Web quickly and see.

(Pause.)

MR. THOMPSON: I'm mostly hearing a lot of similar points that we're hearing in the room, a lot of focus on cognition issues and behavioral and psych issues, especially ones that cause outbursts and anger problems.

MS. GIAMBONE: Okay. Thank you.

So, let me ask you this question. Is there a day when there's a -- we've heard from the panel on, there's not really a good day, some of you mentioned. But can you talk a little bit about

what it takes to recover from a severe day of one of these symptoms that you've selected? If you can, is there a day where maybe -- is there an average day? Is there a bad day? Is there a trigger that's -- always bad, I'm hearing.

Do we have any -- we have some hands raised back there.

MS. RUGGIANO: Good morning. My name is Jennifer Ruggiano, and I'm a caregiver. My husband died January 1st this year from Huntington's disease, and I lost my daughter in 2012 at the age of 12.

There is -- every day is different with Huntington's disease. And any event, no matter whether it's good or bad, will always, always affect an HD patient in a negative way. Sometimes, they get those symptoms -- those tasks or whatever back, but most times they don't. So each day was something different.

One day we would have a day where he can walk fine, but his speech was way off. And then there would be another day when it would be the

opposite. And then there would be another day when you couldn't even tell anything.

But of course, as each event took place in our lives, those tasks and his being just started to deteriorate to a point where he couldn't get anything back.

MS. GIAMBONE: Okay. So that's very -- okay. So, do others share that experience also, that the symptom changes kind of on a day-to-day basis? One day, you know, it might be one symptom that's severe, and other days a different symptom that's severe? Does that resonate with others? Or are there different experiences?

(Pause.)

MR. NIERENBERG: This is Roy Nierenberg again. My experience is a little bit different. When I left, when I left work, it was a company that I founded, I found a number of things to do that really buoyed my life and made my life wonderful. I joined a community chorus. We sing like Verde's Requiem and things like that. And it's the point of the week that I look forward to

the most.

I do di-ung (phonetic) Qigong, like a Tai Chi sort of thing. I ride my bicycle. I meditate. I sleep nine-ten hours a day. I eat. I take care of myself. And I think I'm doing really well, which I know is not the general story. But it's my story.

MS. GIAMBONE: Thank you for sharing that.

And we have a hand raised back there.

MS. HOLDER: So, my name is Lauren Holder. I'm here representing my father, who has Huntington's disease. He's 56 years old. And each day, I totally agree each day is different. You know, some days if he's more agitated because of something, we're going to see more symptoms that day. If he's more relaxed, then you're not going to see nearly as many symptoms, and he actually may look completely normal. My dad does not have chorea.

He has a lot of cognitive impairment, memory loss, can't remember a password that he has

had that's the same for 20 years. He can't remember how to use the remote control some days. Making bad decisions, word-finding difficulties, and an overall slowing down in what he does.

But there are days where he will have a completely clear day. And it's actually okay. Other days, he's -- you know, if he's out of routine, it really just depends on what he's focusing on, and that will determine what symptom is worse or what's better.

MS. GIAMBONE: Thank you.

Okay. So it sounds like what I'm hearing is reducing stress can help sometimes manage or, you know, take care of one of the symptoms on that day, and it can change on a daily basis. Does that resonate with others? Yeah? Okay. Okay.

So, let's take -- let me go on to another -- I'd like to ask if you'd like to share some comments or experiences on some of these other symptoms that you've also selected -- unsteady gait or difficulty walking. You've also

selected -- let's see here -- chorea.

So, would you like to share how you're experiencing that or how your loved one is experiencing that?

(Pause.)

MS. GIAMBONE: How you or your loved one is experiencing some of these other symptoms that you've selected here. Yes.

MS. LoCASTRO: Hello?

MS. GIAMBONE: Okay.

MS. LoCASTRO: Yes. Thank you again for the opportunity to be here and share this information. My name is Tara LoCastro, and I'm from Rochester, New York. I'm here representing my past mother and my sister, who's 44 years old with Huntington's disease.

The other symptom beyond cognitive, which I think is a priority, is the depression or anxiety and the mood aspect of the disease. That has really changed my sister's personality in more recent years. She's had the disease actively for about seven or eight. And there is a

transformation of the individual in the personality. And it's important to recognize that is a big change among the daily changes that occur.

So, I think mood, depression, and just these transformations of the person are something to pay attention to. And I'd like to also just emphasize the importance of cognitive and psychiatric as the less- apparent to the physical, but extremely important to pay attention to for treatment.

MS. GIAMBONE: Thank you.

Yes, we have a comment here.

MS. CLARK: Hi. I would like to also echo that. My mom passed away last year from HD. And the hardest part for our family was probably the depression and anxiety, because when I was growing up, she was always extremely depressed and trying to kill herself. And we didn't know that she had HD. And she was untested and didn't know what her father had passed away from.

But she -- growing up, you know, we were

trying to constantly be kind of parents, basically, and take care of her. And it showed in a variety of ways. She tried to take a lot of pills to kill herself. Or she would just lay in bed all day and say, "There's no point in getting up and doing anything." Just stopped doing everything that you need to do on a daily basis, just stop eating, cleaning. Lost her job, all that kind of stuff.

And then she became really aggressive towards me and my sister, and she would not -- she would fight us, you know, physically, verbally. It was -- and then no one would believe us, either, because she didn't have, you know, a diagnosis. So no one would believe what was going on, the situation.

So, the misunderstanding from medical authorities, and it was -- it was extremely -- so it was extremely hard on the family to try to cope with, basically.

And then when we finally did put her in the nursing home, she was screaming at us that she

wanted to die in the house and that she hated us for putting her into the nursing home. And that, you know, she would hate us forever.

I mean, this kind of stuff is like the hardest to cope with, I think, because you are trying really hard to help, and you want to help this person that's your mother. And she's telling you that she hates you and she wants to die, to your face.

MS. GIAMBONE: Thank you so much for sharing that with us.

We have some other -- yes.

MS. HAMEL: My name is Katrina Hamel. I'm from the Central Coast of California. My grandmother committed suicide because there was no test available for her at the time, and everyone kept saying, you know, it's this or that or, you know, "You have depression," and, you know, trying to medicate her.

Then my mother and my two uncles all have HD. My brother has it, and my other brother is symptomatic. And then I have a brother who is

12 and in social services somewhere on the East Coast.

And cognitive impairment is definitely one of our family's biggest struggle. But to touch on some of the others, my mother would basically try to eat her food, and while choking, she would be having a food fight with herself because of her chorea.

And though chorea is not something that is going to, say, ravage families, it is something that is significant in a way that it creates huge embarrassment for the person with the HD when they're trying to eat and they're literally throwing food across the room.

And my mother and my brother both suffer from the fatigue, but also the sleeplessness. So my mother would go three days without sleeping. And I'm being literal, three days. She would lay there and toss and turn, but not close her eyes. And then she would sleep for two, three, four days and not eat anything.

And then there would be days where I

would come home from work, and she would have fecal matter all over herself and her bedroom. And she would not allow me to assist her. She would not get into the shower, because quote-unquote, she was fine. "Everything is fine. It's going to be okay." And she obviously did not -- not realize, but just didn't think it was a problem.

I had to call department of, you know, elder affairs or whatever it is, like adult protective services on myself, because I had no support. And they weren't even able to do anything. They did nothing for me. I had no help, no support, no family support because the family I did have has either died from Huntington's or is affected by Huntington's.

I am gene-negative. But that brings on a whole other -- a whole other lifestyle.

MS. GIAMBONE: Thank you for sharing that.

And I saw a lot of heads nodding as you were speaking.

Yes. Let's hear from --

MS. THOMASON: Again, I'm Sharon Thomason, from Tallahassee, Florida. Anxiety is a huge issue for my son. He's 30 years old. He will not leave our house. He's been living with me since he got out of the state mental hospital.

His anger and physical violence are controlled by antipsychotics. His depression is controlled by an antidepressant. He also takes a mood stabilizer. His cognitive problems are somewhat controlled by a drug that's approved for Alzheimer's. We haven't found anything that helps with his anxiety.

He cannot leave the house. And on days that he has to, for example, to go to a doctor's appointment, he becomes so anxiety-ridden that he'll sleep all day the next day. And he describes it as just feeling "fried."

MS. GIAMBONE: Thank you.

MS. THOMASON: Fried from his anxiety.

MS. GIAMBONE: Thank you very much.

And let me check in again with the Web.

MR. THOMPSON: So, we did have some people talking about triggers. One person said a bad-day trigger is when you change routine. Routine needs to be maintained. Saying that stress can cause an increase in symptoms, especially behavioral. And one person saying that symptoms can fluctuate, but they are always present and never fade.

MS. GIAMBONE: Okay. And I know that we do have some people that are dialing in, and we'll be hearing from them in just a minute. But before we go there, can we just take another one or two comments on other symptoms that you'd like to share that you experience?

MS. MANNICK: Hi. I'm Janine Mannick. And I'm here representing Andrew Moss. We had met as teenagers. And at the time, there was no indication that HD was in the family. We had drifted apart and found each other again on Facebook in 2009. And at the time, he told me he had Huntington's.

They found out that his dad had

Huntington's. He was diagnosed in 2005 at the age of 68. So it was a late onset. They think his grandmother had Huntington's, but in the gray area. She had no real symptoms.

MS. GIAMBONE: Can you talk about the most significant symptom?

MS. MANNICK: Yeah. As soon as Andrew was diagnosed in 2005, he began with emotional and behavioral symptoms, probably even before 2005. Anxiety plagues him and has since his diagnosis in 2005 at the age of 37. He's now 47. Fear of his ultimate fate plagues him. Fear of losing complete control is the worst thing for him.

We were talking about changes from day to day; for him, it's hour to hour. Yesterday, he was staring and not responding at all. And he was very, very agitated when I gave him his shower. He lives in a nursing home. I'm allowed to shower him. In any case, he was very agitated. And as soon as I got him back to his bed, he smiled and was fine. And he was fine for about an hour. So it changes hour to hour.

And in the two-and-a-half years we've been together, anxiety has been the worst. But he also has paranoia, delusions, hallucinations. He has voices in his head that he talks to. And it's hard to bring him around.

MS. GIAMBONE: Thank you. Thank you for sharing that.

So it's clear that the psychiatric issues -- depression, anxiety, among all of these symptoms, are significantly impacting.

We'll take one more comment, and then we do need to go to the phone. So let's hear from somebody that we haven't heard from.

(Pause.)

MS. WEXLER: Thank you. I'm Nancy Wexler. And it's my pleasure and delight to be here.

(Applause.)

MS. WEXLER: My, my. I think what you're hearing from everybody, Huntington's knocks your socks off. It's mood, movement, memory, mentation, all three areas of functioning. It

gets you down. And we don't have really good treatments in any of these areas. Occasionally, some psychiatric meds, but then it's hard to get ahold of them and people don't prescribe them. Things for movement.

We are all really desperate because, I think, everybody feels that they're sort of out on a limb. And they don't understand it's genetic. And the pressure on your families, I think -- well, you know, this gene. I'm looking at me, the poor woman who had to test positive. You know, what does that do to the family? You know, having discovered the gene, we don't have a cure. We did that in 1993, okay?

So -- and we'll talk about treatment approaches, and we'll definitely touch upon that in Topic 2. We need a lot more to go, because when we had the gene in 1993, and my mom was sick in 1968, we have a few better medicines than 1968, but not many.

MS. GIAMBONE: Thank you so much.

So, I know that the time went by

quickly. Do we have any clarifying questions from FDA on this topic?

DR. DUNN: Yeah, hey, thanks, Soujanya. I'm going to jump in. Yeah, I don't have any clarifying questions. What I want to do is I want to thank each and every one of you for sharing your stories.

And I also want to speak to you directly. I want to make sure I reassure you that if I were in your shoes, I'd be very concerned, do the folks at the FDA understand what we experience? Do they understand what's important to us? Do they understand what we need to help the drug developers incorporate into the clinical trials to sort out what benefit will be provided to patients with this disease?

I can assure you that I and others on the panel who have cared for patients with Huntington's disease, including pediatric patients, in my experience, as well, understand full well what you're saying. This is reiterating our understanding of what is important.

We know that the chorea is a very visible symptom. We understand that the other issues, for a long time, have been more important. This is very consistent with our understanding of the disease, and it's incredibly valuable to hear you reinforce that understanding. I don't want anybody to think that we're not hearing that message, that it's a foreign message to us. It's very valuable to hear it.

But it's consistent. And I think -- I hope that is reassuring to you to hear that we are on the same page with you about what you are telling us about the disease.

I'm particularly interested, we've been discussing things -- I'm particularly interested in the 26 percent of you, according to this small survey, who said that something up there was not captured by the categories. So as the day goes on, if you have a chance to work it in -- we've been doing a little bit of that already -- that's the area I want to make sure that we understand what things we might not be aware of.

So again, I just wanted to say that. I hope that's a useful comment for you to recognize that we share your understanding of the disease.

MS. GIAMBONE: Thank you, Billy.

Okay. So, let's hear from some of the folks on the phone. I know we have a few people lined up. So, operator, could we take caller number one?

THE OPERATOR: Thank you. Our first question comes from Allen.

MR. PFEIFFER: Hi. This is Allen Pfeiffer. My daughter, my 34-year-old daughter, has Huntington's disease. One of the symptoms that she has that has not been discussed at all is sleep disorder. She has no circadian rhythms. She can sleep for two days. She can be up for a day.

The doctor has told her that there is a circadian rhythm drug for people who are blind. But he didn't know for sure whether or not the insurance company would approve it, because she's not blind.

People who approve drugs need to know that gel capsules are easier to swallow than tablets. So format delivery systems are really important.

My daughter has stomach problems, has to take a probiotic. People with Huntington's disease have different intestinal flora than other people. If you do research, you will find out that nicotine has a different effect on people with neurodegenerative disease of the brains, their brains, than normal people. So a form of self-medication is actually smoking cigarettes.

MS. GIAMBONE: Thank you so much.

(Cross-talk.)

MR. PFEIFFER: (Inaudible) create problems.

MS. GIAMBONE: Thank you very much for sharing that. And there were quite a few head nods. You talked about the sleep issues and stomach problems. Do others also experience the stomach -- okay. So we see a lot of head nods here. Okay.

MR. PFEIFFER: Keep in mind that we need to remove medical -- we need to have medical marijuana. Marijuana needs to be removed from controlled drugs and substance lists in order for research to proceed.

MS. GIAMBONE: Thank you very much for that.

Can we have our next caller talk about -- do you have any other symptoms, for the second caller, that you'd like to talk about that haven't been mentioned here?

THE OPERATOR: Our next caller is Ms. Kinser.

MS. CANCELMO: Yes, good morning. My name is Kinser Cancelmo. I'm from Springfield, Massachusetts. My daughter is 16. She has juvenile Huntington's disease. She does have a whole slew of issues. One that hasn't been mentioned is extreme bowel and stomach issues that no one can seem to get a handle on.

We've tried simethicone. They've given her pain meds. She's currently in the hospital,

where she's been for eight weeks, because they can't get a medication regime under control for her. And I can't bring her home because I can't care for her by myself. And there is no place, there is no facility near me that can accommodate her.

So she's been stuck in-patient in the adolescent unit for going on eight weeks now -- eight weeks today, as a matter of fact.

Along with her sleep issues, where once again she won't sleep for nights on end. Her itching is uncontrolled. But her stomach, right up until last night when I left her at about nine o'clock, she was writhing around in pain because of her stomach issues.

MS. GIAMBONE: Thank you for sharing that.

MS. CANCELMO: Yes. You're welcome. Thank you.

MS. GIAMBONE: So, stomach issues, sleep issues, and itching, which I know, Denise, you had also mentioned regarding Aden. Yes.

Okay. Let's take one more caller.

THE OPERATOR: Our next caller is Judy.

MS. ROBERTSON: Oh, hi. I'm Judy Robertson, from Sacramento, California. And my husband, Tim, was diagnosed with Huntington's at age 39. I've got -- we were in marriage counseling at the time. He had symptoms of -- he was just chronically unhappy. He was unsettled. He was pacing a lot. I thought he was having like a midlife crisis. And I was thinking, because of his impatience and cruelty towards our four children, who were young, that I needed to get a divorce.

And it was during the counseling that he told me that he thought he had Huntington's. His mother died of Huntington's, and his brother had it. But I thought you had to have chorea or movement to have Huntington's. And he didn't. He had more of the rigidity.

Turns out I'm very active in the community and have been for over 20 years. And I think about 40 to 50 percent of all patients with

Huntington's have more of the dystonia, rigidity, which is a little bit more like Parkinson's. And they don't have the chorea. They seem to have more -- for my husband, had more depression, psychosis. Medicines help, Seroquel, Prozac. But he always had erratic temperament and sleep disorder.

He did have stomach issues also. And I'm a nurse. So I asked him to be tested for H. pylori, and he tested positive.

MS. GIAMBONE: Thank you, Judy. Thank you very much.

MS. ROBERTSON: We need faster approvals from the FDA for clinical trials. Please help us.

MS. GIAMBONE: Thank you. Thank you.

Okay. So, technically, it is break time. But let's just hear one more comment, and then we'll just take a short break before we get on with Topic 2.

So, would anybody like to share any other symptoms that have not been mentioned or elaborate on one of the symptoms that you've chosen here? Let's try to hear from somebody --

we have some hands back there.

(Pause.)

MS. PAPPADEAS: My name is Mary K. Pappadeas. I live in Columbia, Maryland. And I have a family history of Huntington's. But it hasn't been a horror story like I've heard here. The symptoms in my family, nobody had to be hospitalized for psychosis. My Uncle Al -- I mean, my Uncle George lived next door. They thought he had Parkinson's at first. And he lived at home. He had chorea. I don't have any chorea. My cognitive ability is doing very well.

I have dystonia more than anything, probably. I call it "posturing," Napoleon complex, where your arm goes up, and that's my most bothersome. I'm involved with clinical trials at Hopkins right now. The Prana drug was very, ironically, helped with balance and gait, which is my biggest problem. I'm in a clinical trial right now with Teva. It's going very well. So I'm very hopeful.

They referred me to a physical therapist

for movement disorders up at Hopkins, and I've been working on retraining my brain with standing postures, not just giving in to the balance and the gait problems. And it has helped. And I went to Barcelona and walked the city. And I found walking further and longer helped my core. I used to have a very strong core. I was a cross-country runner in college.

MS. GIAMBONE: Thank you so much for that. I hate to cut you off, but that's definitely something that we want to hear from in Topic 2, which is on treatments and what you're doing to manage some of the symptoms.

So we're going to go to break now. But I just want to thank you again so much. I know that it was a short amount of time, but you really gave us some great information. So, thank you very much. So we'll take a five-minute break. We'll be back here --

DR. EGGERS: Fifteen? Fifteen minutes?

MS. GIAMBONE: -- right around 10:55. Oh, 15 minutes. Okay. We're going to take a 10-

minute break.

(Whereupon, at 10:43 a.m., a recess was taken, to reconvene at 10:54 a.m.)

DR. EGGERS: Okay. So I think we'll get started if we can.

(Pause.)

DR. EGGERS: And as you're making your way to the tables, I'll just reiterate what a rich discussion this morning on the experiences and the burden of your disease and those of your family members.

We're going to be moving into a discussion, Topic 2, on the perspectives on current treatment approaches to treating Huntington's disease. I'm Sara Eggers. I'm in the Office of Strategic Programs. I'm one of Soujanya's colleagues. And I will be facilitating the conversation in the second half.

We have a lot to cover in a short amount of time. So, we have a very full public comment interval. So we will be having to make sure that we save the appropriate amount of time for that.

We're going to try to cover as much as we can in the next 55 minutes.

As we talk about treatments and current treatment approaches, we did hear a little bit this morning, earlier in the morning, that reiterated and provided some thinking of patients and caretakers on treatment approaches. We're going to delve a little bit further into that.

Because we have so much interest in this meeting today, what I'll ask in Topic 2 is that we really try to work on the range of experiences. And I'll go to you and say, "Does the experience that you heard from this person resonate with you?" And that's how we'll really build upon the discussion.

So let us know with any way you can to indicate that what someone else is saying is the thinking that you have as well, so that we can focus the comments on really trying to get as much material covered, as many treatments, as many things as you care about treatments covered in the next 55 minutes.

We have five panelists for Topic 2. They're going to go through their experiences and set the dialog, just like we did for Topic 1. And then we'll move into the facilitated discussion.

With that, I think we're going to go with James first. Yep. James, thank you.

MR. D'AMBOLA: My name is James D'Ambola. And, you know, I have Huntington's disease for 10 years. I'm having a bad HD day, you know, so I can't talk a lot. And I'm going to have my girlfriend, Jessica, talk for me.

JESSICA: Hi, guys. May name is Jessica, and like Jim just said, he's had Huntington's disease for 10 years. And today, he woke up not feeling very great and doesn't feel like he's going to be able to communicate. So I'm going to try and read his speech to you.

He is currently treating his HD with Namenda, venlafaxine, risperidone, tetrabenazine, mirtazapine, and trazodone. He also uses over-the-counter meds of CoQ, fish oil, and multi-vitamins. Other things he does is pray, exercise

when he's able to, sleep. He does not use alcohol, caffeine, drugs, or tobacco at this time.

The treatments address the chorea, impaired mental processing, and personality changes. After he tries a med, if it doesn't have any side effects or make his symptoms worse, it works by improving his symptoms, but never completely cures him.

His meds have changed frequently over the years, because at some point, every increase or new medication becomes overpowered by the disease. When he first displayed symptoms, he was only put on one medication. Now he's on many.

He starts the med at the lowest-possible dose and then increases the dose slowly over time to maximize the beneficial way the medicine works for as long as possible.

At this point, he can no longer even exercise anymore.

His current treatment regimen makes the most significant symptoms of his disease better, but nowhere near a tolerable condition. Even at

the current state, his HD is horrible, awful, the worst disease he could have ever been given by God.

He says he just started tetrabenazine, and it's improved his cognition and chorea. He's now actually able to play with his son, Vincent, three, who is playing on the floor here. He cannot drive, cook, or interact with friends, or do many other things that he used to be able to do. But tetrabenazine did help him to be able to play with his son, which is amazing.

Although the tetrabenazine is helping, it's not taking enough of his symptoms away to lead a quote- unquote "normal life." Before he started the tetrabenazine, he felt like he might die soon because he was at the point where the meds were overridden by the disease. Now, after taking it, his symptoms are much better. But it's also not a perfect or even tolerable condition.

The most significant downside to his current treatment and how it affects his daily life is that the treatments are never enough to

give him that quote- unquote "normal life," and the treatments don't cure him completely or permanently. No matter how well the treatments improve the symptoms, it never stays that way, and his disease continues to progress.

And ideal treatment for Jim would be a medication that could control the chorea that it is gone completely, without having the effect of the drug being overpowered by the disease.

Do you want to say anything else?

MR. D'AMBOLA: You know, (inaudible), so hope to improve, you know, motor vision, to cure it. You know, I'm really hopeful.

DR. EGGERS: Thank you. James, thank you, and thank you, too. I'm sorry. I didn't catch your name.

JESSICA: Jessica.

DR. EGGERS: Thank you, Jessica.

And the little one?

JESSICA: Vincent.

DR. EGGERS: Thank you, Vincent. A round of applause for Vincent, please.

(Applause.)

DR. EGGERS: I think our youngest ambassador in the room.

And now we will hear from -- who is? Karen will go next. And we'll try to get the -- okay, you're going to use the microphone.

MS. MILEK: Hi. I'm Karen Milek, from Florida. I am 54 years old. In order to try and delay the symptoms and the disease from taking over my body, I do a lot of things. I got gene-tested 21 years ago at the age of 33. That was the year my mom was pretty sick in the later stages of Huntington's. I needed to know if I had HD, and if I did, I wanted to do everything I could to stop HD from taking any more people in my life or any more people in the world.

I tested positive and signed up for a drug study called CARE HD for CoQ10 one month after I got my results. Just being part of a drug study makes you feel a lot better already. I have been taking CoQ10 still for the last 21 years. I know they just did a lab study, and they said the

CoQ10 wasn't working as they wanted it to, and they stopped it. But I am still on it. I am not going to stop something I knew helped energize my brain when I first was on it, and there have not been any bad side effects on it.

I also have been in PREDICT-HD for 11 years, and I sign up for all the research they let me do. I am lucky that being in studies makes me feel useful and gives me hope. Some people get nervous doing the studies; not me.

I also went to my first HDSA convention, where I learned so much information about the disease, met so many folks, and I helped start a support group back in Florida and started fundraising for Huntington's disease. I've been to 19 HDSA national conventions, and they help me stay very informed. They always make me feel good and include me in a lot of things they're doing.

I also have been on the FDA Advisory Committee for the new drug, for tetrabenazine, when it was approved. I was the person for Huntington's that got to come, so thank you, guys,

for letting me be a part of that.

I also -- I exercise all the time on my own. And ever since I was a kid, I like to run. And I continue to exercise, because now they have so many studies that they're saying that exercise is going to help our bodies and our brain fight off everything. I run, bike, and swim. Even I work, so I run on my lunch break every day.

As far as the diet, I eat a lot of blueberries for the antioxidants. I also take 2,400 milligrams of fish oil, 6-10 milligrams of creatine, 1,200 milligrams of acai, and the 600 milligrams of the CoQ10, and a multivitamin daily. Sometimes, I forget to take them, but I try to do it.

Since I'm a single person with no children, I can take more risks with my body, like signing up for spinal taps and taking the new drugs and the studies. I have been taking a medicine for irritability and my OCD for a very long time, like 21 years, way before any of my -- you know, Huntington's disease symptoms would show

up. But that happened way, way before.

Right now, I take 100 milligrams of Zoloft daily. And if I forget to take that, I am irritable and the people around me notice. A lot. (Laughing.) So I try to keep my brain cells alive and working.

I still have a job. I used to -- I work at FedEx. I used to drive, but I went off -- I got off the road, and I don't drive anymore, because I know driving seems to be an issue with us Huntington's people. And I just chose to get in the office ahead of time, before someone was going to tell me it was a problem.

A lot of us people with Huntington's keep driving even when we shouldn't, and I was afraid that I might be one of them. So I just decided ahead of time to get a non-driving job.

Anything I work on, any chores take a lot longer. I kind of go from one task to the next and leave a lot of things unfinished. I don't like to clean my house like I used to. I just watched my sister pass away at the age of 50

this year from Huntington's, and my other sister is 56 in a nursing home with Huntington's. And both of them have tried to commit suicide a number of -- well, one actually put herself in the facility before she did it, and the other one has tried it three times. It's not good. We don't like that.

I drink Diet Coke still. And I know it's supposed to be bad for us, but since my body seems to be liking what I'm doing, I'm going to stay on everything I've been doing. And I'm not going to change. So, all right.

DR. EGGERS: Any final thoughts, Karen, that you'd like to share? Any important messages?

MS. MILEK: Okay. So, well, my independence is very important. The more I can do to stay independent was what I want to do. Because after that, I don't think that's living anymore. So I want things to make us independent.

And I want to say, for the drugs, in treatment, I would like something that can slow down the progression so that we can start showing

symptoms a lot later in our lives, not at 30, 40, or 50, but like at 70 years old would be okay with me. Death is a part of life. I just like it to be a lot later on.

DR. EGGERS: Thank you so much, Karen. Thank you.

Now, Stacey? Okay. We'll have Stacey go.

MS. SARGENT: Hello. My name is Stacey Sargent. I'm from Douglasville, Georgia. And this is a picture of my son, Corey, who's now 21. I was very young when I decided to get married and have children, and family history was not important to me. I thought I was young and in love.

When Corey was born, due to an abusive relationship, he was born three months premature, weighing only two pounds. No one thought he was going to make it in his little incubator, attached to a ventilator, feeding tube, and numerous other machines. But he was trying to push himself over. So I knew early on I had a fighter on my hands.

I knew due to his premature birth that he would always be developmentally delayed. When he started school, Corey had some learning difficulties and a slight speech impairment. First grade, a student intern in the special-ed program tried to diagnose Corey as autistic, due to echolalia.

We went to a neurologist, who decided Corey had ADHD, and at that time the drug of choice was Ritalin. Luckily, knowing what I know now, I refused and did diet modification.

A few years later, we started noticing that Corey had a facial chorea, took him in to his neurologist for an MRI, and was given the diagnosis encephalopathy. But in order to get insurance to cover therapies and to put a label on him that everyone would understand, he was given the diagnosis of spastic cerebral palsy.

By age 10, he started walking on his toes. By age 12, he had another decline affecting his posture, ability to walk, speech, eat, and swallow. It was time to find a new doctor,

because this one wasn't listening. I knew something was wrong with my child.

Numerous neurologists looked at me like I was crazy. Finally, one showed concern and ran tests, ruling out metabolic disorders. Finally, Corey started having itching, kicking, kicking like a horse, unable to sleep at night, often lasting for days at a time. The doctor decided to try Sinemet at that time. It was our miracle drug. He still had the itching, but it was controlling his sleep and he was able to -- it was controlling the kicking and he was able to sleep.

That medication was started in 2009, one tab at bedtime. Here now, at 2015, we are on two tabs three times a day, and one as needed.

Often, when he gets agitated, it increases his chorea and dystonia. Sometimes, even the Sinemet doesn't help, and we have to give him pain meds in order to help him sleep and give him rest.

It was discovered when Corey was in the hospital getting diagnosed in 2009 that he had

many ulcers of his esophagus and stomach. I was devastated in 2009 when I got his diagnosis, a CAG of 85, because there's nothing I knew I could do to help my baby boy.

I cried, I screamed, and then I cried some more. As a mother, I'm supposed to kiss my child's hurts away, but because of Huntington's, those hurts are so much more profound than I ever, ever imagined. At the age of 15, instead of getting a learner's permit, he was learning how to use a wheelchair.

The disease progressed so quickly that, by the age of 17, he was unable to speak, unable to attend school, bedbound and completely dependent on us for his every need. He is on meds for chorea, dystonia, reflux, muscle relaxers, seizures, sleep, itching, pain, and agitation -- all only providing minimal relief.

At the age of 18 when he is supposed to be choosing a college to go to, we are choosing which hospice agency to use. He did manage to graduate at 19, something that four years before

we had been told we probably wouldn't see.

He is now 21, less alert of his surroundings, never had a girlfriend, never had his first kiss, never got to go to prom.

Corey never met a stranger, having a hug and kiss for everyone who ever crossed his path. I know that not being able to control his facial expressions, to smile at someone he has just met, to hug and kiss us every night when we tell him good-night, I know it has to hurt him emotionally, even though in his eyes I can see him smiling.

I do know that this bothers him, because recently my sister gave birth to a little girl, a little girl that I took Corey to see when she was 11 days old. He was so excited, the dystonia was just so bad I couldn't let him hold her, because I was scared he would hurt her. Telling him no triggered a seizure.

He has to have Botox injections every three months into his cheeks into chin, into his saliva glands, to keep his saliva down, because he produces so much he chokes. He can't even control

his head to keep from -- to try to spit it out. I do this to keep him safe, to keep him safe from aspirating.

He receives therapies on homebound education, originally to preserve independence. Now they are for comfort and to give me some relief. But those are services that, once he turns 22 in February, I'm going to lose because, see, with him on hospice, we won't qualify for community-based programs.

I believe JHD research and trials are important because these kids don't have time to wait. These kids deserve a better quality of life. We need to preserve their ability to think and to communicate so that they can tell us what's wrong and how to help them.

We realize that there are risks, but our children are dying. We will do anything to save them. Some of us are even doing things that are illegal, like using medical marijuana in states where it's not permitted. We believe that if they are allowed to participate in research and trials,

that at least their death won't be in vain.

Our family made a promise to Corey six years ago that we're going to fight this as long as there are breaths in not just our bodies. And we're not going to fight it just for him, but for all the children affected by Huntington's disease.

DR. EGGERS: Thank you so much, Stacey.

(Applause.)

DR. EGGERS: Everyone deserves a round of applause, I think.

(Applause.)

DR. EGGERS: So, is it Cheryl next? Cheryl, thank you.

MS. SULLIVAN STAVELEY: Hi, everyone. My name is Cheryl Sullivan Staveley. I'm from Massachusetts, and I'm a patient representative for my husband, John, who was diagnosed at the age of 37, and for my daughter, Meghan, who was diagnosed with JHD at the age of 19. John passed away seven years ago at the age of 56, and Meghan, last May at the age of 26.

John was a 12-time Boston Marathoner,

who ran six miles each day for about eight years in the beginning to early mid-stage of his HD, to keep physically active. He kept himself intellectually stimulated by reading textbooks related to his law enforcement career, daily newspapers, and watching the news. He took vitamin supplements, niacin, riboflavin, and coenzyme Q10. After about 10 years, he took Zyprexa to help with his anxiety and depression.

In 1996, he underwent a fetal pig tissue transplant to see if the fetal pig tissue could be a substitute for his own brain tissue that was being destroyed. After four years of close observation and taking cyclosporine for possible tissue rejection, the medical researchers felt that the surgery had neither helped nor harmed him. However, emotionally, I believe that it helped him maintain more of a positive attitude.

Meghan went to the gym three times a week, doing cardio and stretching during the early to mid- stages of her JHD. After receiving her associate's degree, she continued to audit a

couple of classes each semester for about two years to try to keep her mind cognitively active.

She took 30 grams of creatine and 2,400 milligrams of coenzyme Q10 for about five years. She was prescribed a wide variety of antidepressants, anti- anxiety, and antipsychotics during her 10-year battle with JHD. At one time or another, she was on Zyprexa, Zoloft, Celexa, Abilify, Ativan, Klonopin, and Remeron.

Both John and Meghan strived to eat 6,000 calories a day by eating a combination of fruits, vegetables, healthy protein, and carbs, as well as the not-so-healthy high-caloric junk food like ice cream, donuts, cookies, and candy.

All of the abovementioned treatments were designed to maintain physical strength, balance, agility, emotional stability, and to remain as cognitively intact and to keep their own personality for as long as possible.

As their HD progressed, their level of physical activity declined due to increased chorea for Meghan, and rigidity for John. They both,

obviously, experienced decreased strength and balance.

Meghan tried Xenazine in 2009, but it did not decrease her chorea. It made her very depressed, agitated, and caused difficulty sleeping for the three months she was on it. John died before the Xenazine became available. However, although this was not its intended use, for Meghan, Abilify decreased Meghan's chorea for almost two years in the early stages of her JHD.

Cognitively, their ability to focus and read declined, although they both enjoyed being read to. Carrying on conversations ultimately became very difficult, due to the delayed thought processes and slurred speech. However, they always seemed to understand what was being said to them, and they could answer yes-or-no questions. They basically continued to enjoy the entertainment that they liked.

John and Meghan's treatment regimen ultimately did not help with their chorea or

prevent their cognitive or personality decline. Emotionally, their meds did a good job in controlling the anxiety, depression, and perseveration, which did improve their emotional overall wellbeing.

Significant downsides for both John, and especially Meghan, from the emotional medications were that sometimes it made them apathetic, irritable, restless, and lethargic. When this happened, neither one wanted to participate in varying activities, or interacting with their family or friends.

It also often caused them to sleep all day, be up all night, which was very disruptive for them both when they were at home, and each in their respective nursing homes.

For Meg, her personality could change, making her more aggressive or negative at times. For my family and I, an ideal treatment would be one that could slow down the progression of all three aspects of HD in general, but for my family in particular, especially the cognitive aspects.

I see this -- I say this because I know there is some treatment for the emotional as well as the motor aspects.

To me and for my family, if each stage of HD could be longer and the symptoms slower to evolve, ultimately that would mean a better, more productive quality of life for a longer period of time. We would consider this a victory should a cure not be able to be fully realized. Thank you.

DR. EGGERS: Thank you so much, Cheryl.

(Applause.)

DR. EGGERS: And finally, we have Karen Douglas.

MS. DOUGLAS: Good morning. My name is Karen Douglas. I'm here with my husband, Matt, who has Huntington's disease in his family. I'm fairly new to the Huntington's world. We've been married for 20 years. But I didn't know what Huntington's disease was before we were married.

Currently, we are doing some treatments that, we've done treatments in the past that we're not doing now, and I'm sure we'll have other

treatments that we're doing in the future.

But currently, right now, we have been doing exercise with an exercise DVD that he has. He loves the Wii, with the hand-eye coordination, and the Wii Fit for balance. He has ankle weights that he'll wear to wear at his muscles and to keep his feet from flying up when he loses balance throughout the day.

We were able to obtain a service dog. Some of you have seen Jerry around today. He's down here. And that's been a huge blessing in so many ways. It helps Matt with balance, so as he's maintaining his walk, he can hold onto the handle. He also helps with picking him up when he falls. I'm still working part of the day, most of the day. And when Matt falls, I know that he's able to get up and continue on.

He opens the doors for him, and he picks up things for him. And besides that, he's a great companion and a great joy and responsibility for Matt to take care of.

Every other week, we go to OMT, which is

Osteopathic Manipulative Services. It helps to relax his muscles and the spasms for about a day. So going every other week really is not enough. But it does help in some aspects. He has nutritional drink supplements, like Boost, to try to take care of the 5,000 calories each day that we've been seeing.

And he also uses technology tools. Praise the Lord for the technology that we have today, with different apps and so many ways that you can use them. He does Luminosity, with the brain games, to try to keep that memory going and sharp. The hand-eye coordination, he loves these racing games.

And weight management, we've been using a Fitbit, which has been really a lot of help to me. As he wears the Fitbit, I can tell how many calories he is burning that day, and then how many calories he needs to eat in order to even maintain his weight. So he's been averaging 28,000 to 30,000 steps a day and burns about 5,000 calories a day.

There's another app for oral management, which is Oral-B. It sets reminders to remind him to do his teeth and to be able to remember to floss and rinse and all that type of thing. And then I don't feel like a nag. And then he also uses the social interaction with Facebook and that type of thing. Since he's not able to drive and go out, he's able to still socialize with people that he's had previously in his life, and he enjoys that.

The prescription medicines -- trazodone for sleep, mood, and anxiety. It has worked, but not to the degree that it helps anymore. So we try to increase and add Haldol onto that. I'm going to talk about that a little bit more later.

For movements, he was on Xenazine, tetrabenazine. He's been on that for 12 years. Thank you to the FDA for approving that. Chorea has been our most prominent symptom to have to deal with, and it has been not cosmetic. I've heard that before, but it's very dangerous. And we've had some situations that he could very well

have harmed himself with. So we're very thankful that he's been able to prolong independence for a little bit longer with the Xenazine, tetrabenazine.

He's got Galantamine Hydrobromide for memory, Zoloft for mood, Flomax for bladder control, and Ativan for anxiety. As you can well imagine, with all of these different drugs, prescription medicines that he is taking, there's always side effects to each one of them. And so, it is challenging. Sometimes, you have to take another medicine in order to make up for the medicine that you're on. So it's a constant journey of trying to balance things out.

How does your treatment regimen change over time? It has changed by us having to increase the strength. As new treatments are added and as the decline continues, we just continue increasing strength. Obviously, it doesn't get to the point that everything is taken away. You just manage.

DR. EGGERS: Any final thought? You

were going to talk about one more product.

MS. DOUGLAS: Sure.

DR. EGGERS: And then, what really Matt would like to see out of treatments.

MS. DOUGLAS: Absolutely. Thanks.

DR. EGGERS: Um-hm.

MS. DOUGLAS: One of the ways that we would really like to see an ideal treatment, obviously, we would love for a cure, and you've heard that a lot today. But I did call some friends and family. It is a family disease. It doesn't just affect one person.

And I found that, as you've heard from many stories today, so some of the friends and family have said communication options would be really helpful. Speech becomes very difficult to understand, as you've seen, and then it's nonexistent. And so I did see in his mother that passed away that she communicated with her eyes. And that was just really interesting to be able to still see her trying to communicate that way.

Speaking and swallowing process, the

fear of frequent choking -- got to get that weight management taken care of, and therefore you need to eat.

And then the movement, as I mentioned, and mood -- if there's some type of effective low-side- effect antidepressant to cope with depression and mental anguish, that was another thing that somebody mentioned.

But in summary, my request would be to continue to approve new treatments coming down the pipeline as quickly as possible. Matt had that request, that sometimes it does take a long time. And so, we need it sooner than later, as you've seen and heard from many people here.

With it being hereditary and a family disease, our families need the help of the FDA. People ask me where Matt and I find our hope. And we really find it in our faith, our family, and our friends. We appreciate all that you guys have done to take a moment to hear from the families here today. It really does make a huge difference, and we've seen your sincerity. And it

just really has moved us.

So I appreciate your time, and hope that echo from everyone else. Thank you.

(Applause.)

DR. EGGERS: Thank you. Thank you, Matt. Thank you, Matt, and thank you, Karen.

And thank you again to all of the panelists for so eloquently showing the complexity of the management of your condition, ranging from the pharmaceutical treatments, but you can't -- there's so much else to cover and so much else that you require. And that came out very clearly in your comments today.

So I think that this demonstrates the range of perspectives, and I'll ask for an indication from the audience. Did that reflect the range? You might not have -- not everything would have resonated with you, but something did? Something did? Okay. Great. Thank you.

Please, one more round of applause for the panelists.

(Applause.)

DR. EGGERS: We are going to now move in and try to delve in a little bit more into aspects of treatment that you've experienced or your loved ones have experienced and that matter to you. Again, we're not going to be able to cover every possible treatment or every experience. But I think we'll be able to cover both pharmaceutical treatments first, and then the range of other therapies, but more, other life management that require.

So I'm going to put up a polling question first. And this is first focused on the pharmaceutical treatments. And I have to stand over here. I'm not going to -- or I can go through this list. It's not an extremely long list, so let me walk through it.

So again, if you've got your clickers out, have you ever used any of the following drug therapies to help reduce your symptoms of Huntington's disease? And you can check all that apply. Tetrabenazine, A; antipsychotic drugs, B; antidepressants, C; other drug therapies. And

those focus on the drug therapies not mentioned. And E, not sure.

(Pause.)

DR. EGGERS: Okay. I think we can -- okay. A wide range, with the most focus on the antidepressants, but there is experience reflected all throughout in person, including the tetrabenazine.

On the Web, can I ask what we have?

(Pause.)

DR. EGGERS: We will come back. We will come back to that. Graham --

MR. THOMPSON: Never mind. All right. We're back now. Bad local mic's.

Everybody on the Web who answered this said that they're taking antidepressants. Sixty-five percent said they take antipsychotic drugs. Thirty- five percent say tetrabenazine. And then 50 percent say other drug therapies not mentioned.

DR. EGGERS: Okay. All right. Thank you.

One thing I want to reiterate, if we

don't get to cover all the topics here, we had a hard time identifying the panelists who would represent and kick off the discussion, because you sent in, collectively, a tremendous response. You sent in your comments to us.

We will use those comments, and they will be reflected in our report as well. So we have all the comments that you might have sent us. We also have the public docket. So if you're hearing something and you want to build upon it and we don't get to discuss it today, that docket, where you can electronically submit your comments, is extremely important. It doesn't matter if you already said it here today. You can expand upon it in the docket.

So, please remember to go home, to think through, and to submit a comment on what you're hearing today.

So with that, let's go into a few comments on tetrabenazine. And when we talk about any of these therapies, what we're looking for is, how does it work for you, or how did it not work

for you? And how did you know? And how long did you give it? How long did it take before you were able to come to some sort of determination? Immediately, or some time, or you tried it for long enough, and then you decided -- it was determined that that wasn't the right treatment for you? Any comments on tetrabenazine?

So we'll go here first, and then we'll go into the back, over there.

MS. ROSLING: Tetrabenazine is a great drug.

DR. EGGERS: So, this is Julie.

MS. ROSLING: It is the only drug we have -- I'm sorry. But tetrabenazine, as we know, is only good for chorea. They put me on chorea-type drugs, tetrabenazine, just because there's nothing else around. I felt so sick on that drug that it was not worthwhile for me to take.

DR. EGGERS: Okay.

MS. ROSLING: And again, we have to work on things that are available for other types of symptoms than for that.

DR. EGGERS: Thank you for that point, which is a point that is, I think, going to be reiterated and is resonating.

Back there?

MS. RANDALL: Hi. My name is Laura Randall.

DR. EGGERS: Hi, Laura.

MS. RANDALL: My son is 26 and has Huntington's. I'm just -- we don't -- he doesn't take any of those drugs up there. So, he has really bad tremors that affect everything that we've heard today. He can't eat, he can't really do anything himself. So he's taking Depakote, which is a seizure medicine. He takes Sinemet, that we've heard of, and he takes amantadine for the rigidness.

But what I've noticed is it seems to -- it's all trial and error, right? And you don't know if something's working until you don't take it. So, you know --

DR. EGGERS: So what do you look for? How do you know it's working for your son?

MS. RANDALL: He -- it's horrible. He misses a pill, I know right away. He can't walk, he can't do anything himself. You know, he has a really bad day. And most of the time, I find a pill in the pillbox or I find something on the floor. So I've tried to take him off some medicines because I don't think they're working and, you know, as soon as we go off them it's like the symptoms are ridiculous.

So I feel like there should be a better way -- you know, and we've heard today that everybody has different symptoms and it's affecting everybody differently. But it's just such a hunt-and-peck at this point. You know, what works and what doesn't? It's very maddening.

And I want to ask the dumb question of the day. Since we've known of the gene since 1993, why aren't we working on that? That's what we should be working on.

(Applause.)

DR. EGGERS: Your question has -- is an important one and is resonating with others.

So, let's go on to another comment, another experience shared. Right here. Oh, and then -- I'm sorry. Then we'll go back. Is there someone back there? Um-hm.

MS. LoCASTRO: Again, my name is Tara LoCastro. I think that I want to emphasize the comments made by the panel about prevention and somehow making sure that we're not discussing treatment during the symptoms as much as we might be able to before they occur. Whether it's over-the-counter, mixtures of fish oils and things of that nature that have some efficacy, but also things that can be given to whether you're at-risk or you are gene-positive very, very early, well before onset.

DR. EGGERS: Thank you very much.

Ashley, was there someone back there? Okay. Then we'll come -- then we'll stay.

MS. HUDGELL: I just want to make a comment. Aden hasn't been on tetrabenazine, since it's for chorea, but he's been on antipsychotic medication and Depakote and several anti-

epileptics, not only for his seizures, but for mood stabilization.

But I think one thing that I want to point out is that when we talk about the way that HD patients metabolize medication, we can be on a medication for a very brief amount of time and we have to increase it.

So, of course, we all want a cure. But when we're talking about meds and when we're talking about how our kids and our husbands and wives and whatever loved one we're talking about, sometimes the meds just aren't it. We need to find a cure.

DR. EGGERS: Okay. We'll go here, and then we'll go with Colleen. Okay.

MS. THOMASON: Another thing I'd like to mention is that, except for tetrabenazine, none of these others are approved for Huntington's disease. And that can be a problem.

For example, my son had been taking memantine, or Namenda. And it was helping him a lot. But then, the insurance company said they

wouldn't pay for it anymore because it wasn't approved for Huntington's disease. And so, we had to jump through some hoops and do appeals that failed. And then I had to take him to a neurologist and have a neuropsychiatric battery of tests done and prove that he had dementia. And then they would approve it.

So, we need these other drugs, the antipsychotics, the antidepressants, the mood stabilizers, the drugs for cognition. We need those to be approved for Huntington's.

DR. EGGERS: Thank you for that point.

Okay. We'll go with Colleen.

Feel free to clap. It's a good way to show that a comment really resonates for you. So feel free to clap when something resonates.

MS. WALSH-BARNES: As I mentioned before, my sons were complete opposites, my sun and my moon. The only drug that helped Miles with his severe cognitive processing and integrating the outside world into his reality was Depakote. The only drug that helped Jason with his violence

was Depakote.

And I actually had to fight for Jason to go on Depakote because no one thought it was help his violence. But it did. He was being sent to the psych ward regularly from the nursing home. Once he went on the Depakote, that completely ended. He became very calm.

The other two non-drug things -- clinical trials. My sons were in the best place during clinical trials, especially clinical trials that they saw -- you know, they went into the hospital frequently. You know, not the ones where you go like maybe once every two months.

That, of course, alleviated some of their depression. It made them feel in control of things. And it also gave them hope. The only hope they ever had was clinical trials. So, you know, the expansion of that would be great. I started with clinical trials with my children and myself and my husband in 1979. And it's one of the best things for the treatment of Huntington's disease.

The other thing is not really related,

but my grandson was born using genetic testing, in vitro fertilization. And my son, knowing that he wasn't going to pass the disease on was huge, huge. Because I know what it did to me to know that my children were at risk, and he didn't have to suffer that.

So those were the three things that I think had the best impact on my son.

(Applause.)

DR. EGGERS: Thank you, Colleen. Thank you.

Are there any questions on any particular drug therapy that you'd like to ask?

(No audible response.)

DR. EGGERS: Time is tight, so we're going to move on.

Okay. Let's go in and see -- let's move on into the non -- the other types of therapies. I'm sorry. Time is -- we've only got a few minutes left. So, okay, we'll take one comment here from -- what was your name again?

MR. NIERENBERG: Roy Nierenberg. I'm

taking a couple of things off-label.

DR. EGGERS: Um-hm. Okay.

MR. NIERENBERG: I'm taking Verapamil, which is a calcium channel blocker. And it's -- when I took it, it restored my creative thinking abilities and it countered my apathy. I'm also taking Namenda, but I have to take -- you know, I have to pay extra for it. And I'm also taking melatonin.

The Namenda and melatonin were recommended by a neurologist who's not an HD neurologist. And the Verapamil was -- I heard about through the Stanford HOPES site, but I'm taking it -- my internist, he prescribed it.

DR. EGGERS: Um-hm. Thank you, Roy. I saw a lot of people nodding with what you were saying.

Okay. We will really go with one more, and then we are going to move on to the nondrug therapies. Please, go ahead.

MS. CLARK: Okay. I just wanted to add really quickly that tetrabenazine worked really

well for my mom. And that was a really important drug that really helped control her chorea. But near the end, it got really bad and even that couldn't control it.

And the worst part about the drugs and trying to treat the symptoms with my mom was, she would keep on changing. Like one symptom would get really bad, and then you would fix that. And then something else would get really bad. So, like the motor symptoms or the cognitive.

So it was just constantly juggling. And every single day was a new puzzle to be solved. And as soon as you fixed that, then it would just all start all over again. And she was obviously deteriorating throughout. And so, I mean, it's really, really tricky to try -- there's like definitely not like one answer here.

And I wanted to also say that I tested positive. And I have 45 CAG count. And I am 31, and I don't really know what I should be doing to try to be as healthy as possible, other than eat a good diet and work out and, you know -- I see my

therapist, and I have Prozac, 40 milligrams a day for my depression, which seems to help some. But I don't really know what to do.

I was on CoQ -- CoQ10 and creatine until I saw that those really weren't working. So, yeah. So, I don't really -- for a person that is gene-positive that is trying to stave off HD, it's really -- it's really hard to have that cloud, that shadow hanging over my head, especially after seeing what it did to my mom and taking care of her. So.

DR. EGGERS: Thank you. Thank you.

For the others whose symptoms have --

(Applause.)

DR. EGGERS: Okay. You've answered my question then, that this is an experience and perspective that's shared by others who maybe have not reached the point of progression of symptoms yet.

We were going to ask a question about other therapies. But I don't think -- I think in the interest of time, the few wrap-up questions we

want to get at, and we want to get at a Web summary. But I think we heard -- so let me just show. If you can indicate somehow the importance -- the importance as was shared of others of the nondrug therapies currently to your overall management or the management of your family member.

Okay. We have one comment back there. So we will take that.

MR. SERBIN: Yeah. My name is Kenneth Serbin. I'm known in the HD community as Gene Veritas. My mother died of Huntington's disease in 2006. She had 40 CAG repeats. We tested our daughter during the pregnancy in '99, 2000, before pre-implantation was available. She tested negative. It was the happiest day of our lives. Today she's a healthy 15-year-old in high school.

When I saw my mother get symptoms and die, and when I see someone with HD, I say it's like looking into the genetic mirror because of looking at my own future. Like Karen, an asymptomatic gene carrier, and I really would like

to see a medication that prevents me from ever getting any kind of symptoms.

I'm very lucky at this point, at age 55, well past the point when my mother had onset of all types of the symptoms. She began with the mood symptoms. Then it developed into chorea, and cognitive loss, memory loss, so on and so forth, swallowing difficulties.

A really important point I want to make to the FDA is that I think that there's got to be a really open dialog with the scientists working on the new areas such as gene silencing. I don't really know why, but the first gene silencing clinical trial we have is happening in Europe and Canada. And I couldn't really find out from the company, Isis Pharmaceuticals, why they didn't want to do it in the U.S., but they're not.

I was disappointed at that; so were a lot of us in the HD community. Hopefully, in phase two and three, if it gets to that, we can include people in the United States.

I've also heard from drug company

executives who want to use MRI, cerebral, spinal fluid, and other kinds of new technologies or ways of looking at these parts of the body, the brain, the CSF, the blood, where the new types of biomarkers are being found, that there's been, as it were, a kind of lack of flexibility on the part of the FDA. And I'd really like to urge that these new kinds of technologies that can get us to looking at drugs that will help gene-positive pre-symptomatic people like me avoid the disease.

DR. EGGERS: I think you're raising a really good point that I'm going to ask for in the docket. Because the asymptomatic gene carriers, you have a different -- everyone has a different perspective, but your perspective is very different from people who are more, in the more advanced stages.

So make sure that the asymptomatic gene carriers, you're telling us, sharing with us what you want to see in the future before you ever have symptoms, what's going to matter most to you, to either identify or delay or address it right away.

The same goes -- we're going to have to move on. The same -- so I'm going to put your homework assignment for the docket. At all stages --

(Applause.)

DR. EGGERS: Well, you didn't hear your homework assignment yet.

At all stages, you know, what is it that you wish you could stop, or you wish you could slow down, or you wish you could regain if that was at all possible? Tell us what those specific aspects -- think of them as things that could be addressed, perhaps, by a pharmaceutical therapy -- the cognitive, the behavioral -- and write that in to us. We aren't able to address all your comments, but we will read all of them as they come in. So there's your homework assignment.

We have one person on the phone. And before that -- we'll go to that in a second -- Graham, could we get just a brief summary of what we're hearing on the Web? We have a very large Web presence, and we so much appreciate it. I'm looking where a camera would be. So that we

appreciate your contributions as well. So keep those webcast comments coming in, because that's what the camera is looking at.

We had the camera today pointing so that people on the Web could see you all. You are the most important people to be viewing today. Yes, so wave hi to everyone on the Web.

Graham, please.

MR. THOMPSON: So we have several people were talking about tetrabenazine, various responses. People said that it helped gain weight and reduce movement. But several people mentioning issues with perception and depression.

And then we had several people who asked some questions about the current state of gene therapy and the progress of gene therapy in the future.

DR. EGGERS: And you can ask -- we might not be able to answer questions. But you can ask your questions. What are those most important questions that you have? What are the things you want FDA to know that you're concerned about and

that you have questions about? And that is just important feedback on what your perspectives are in terms of treating this condition.

We have one person on the phone. And so, I'm going to ask, who's on the phone? Oh, yes, that's right. Operator, can we have a caller, please?

THE OPERATOR: Yvonne (phonetic), your line is open.

DR. EGGERS: Is it Yvonne?

MS. SWEETON: Good morning, everybody. My name is Ivonne Sweeton (phonetic). I live in Henderson, Nevada. I am an HD ambassador, which means I'm very involved in the HD cure. I do have a diagnosis, just got it. I have a 41 CAG. It ran in my father's family. He had it. His father had it. We've lost most of his side of the family to HD. It's like a Holocaust, but a different kind.

I agree with everybody. I echo all of you. And I applaud you for going there. What I agree with mostly is, first thing, you need a

diagnosis. Thank goodness we had one early in our family, so we all had choices on whether or not to have children, whether or not to pursue a certain career, whether or not to get married. So we didn't allow it to define our life, but it definitely helped us make decisions.

The second thing is the connection, the support. I feel connected to all of you because I have a defective HD gene from my father and the normal HD gene from my mother. So therefore, we're all connected either by whether or not you have the disease or were part of a family.

The third thing is the cure. What I learned at convention this summer -- and thank you to HDSA for a great convention. I learned that the scientists are so excited about a cure. They are going to find a cure, and it will be in our lifetime. Thank you.

DR. EGGERS: Thank you. Thank you, Yvonne. There were a lot of heads nodding. You're getting a round of applause from the folks in the audience.

(Applause.)

DR. EGGERS: Any final things from the panelists? No.

So, I want to thank you all for such a rich discussion. It was so rich that we have gone a little bit over our time. So we will ask the -- there's going to be a public comment next. And we're going to ask you really to keep your comments brief. If the comment that you have to make has really resonated with something someone else said, you could just agree with that person, and that way we won't be rushed as we go through our public comments. Okay? Thank you very much.

MS. VAIDYA: Hello, everyone. I'd like to thank you all for coming today. So we're now moving into the Open Public Comment session. And for those of you who are not aware, the purpose of this session is to allow an opportunity for those who have not had a chance to speak on issues that are not related to our two main discussion topics today to present your thoughts.

Please keep in mind that we will not be

responding to your comments, but they will be transcribed and be part of the public record.

Since we would like to make this a transparent process, we do encourage you to note any financial interests that you may have related to your comment. If you do not have such interests, you may state for the record. And if you prefer not to provide this information, you can still go ahead and provide your comments.

So we have collected signup before the meeting and during the break. We have 12 people who have signed up, and roughly 30 -- 25 minutes, let's say, for this session. So please be respectful for other colleagues here and other patients, and try to stick to the two-minute limit that we have for each comment.

I'll be keeping track of time here. And as you approach the two-minute mark, I will have to slowly nudge you to wrap up.

So I will run through the order of speakers, and I apologize in advance if I mispronounce your name. So, we have first Cheryl

Sullivan, then we will have Jonathan Monkemeyer, Loretta, Melanie Rehm, Jennifer Mann, Karen Clark, Louise Vetter, Brian Win -- Brian, Sharon Thomason, Katrina Hamel, Lauren Holder, and Kenneth Serbin.

So, could I please have the mic to Cheryl, please? Cheryl Sullivan.

MS. SULLIVAN STAVELEY: I just find I don't think there's anything additional that I wanted to say that has not been said.

MS. VAIDYA: Okay. Great. Okay.

So, can we move on to Jonathan Monkemeyer?

MR. MONKEMEYER: Hi. I'm Jonathan Monkemeyer. Just wanted to emphasize that our relationship with the FDA is, we really want to bring therapeutics through absolutely as fast as possible. You could see it's a horrendous disease, from JHD on to HD. It's just a whole spectrum of suffering.

And we're willing to take a lot of risk and are not so concerned about the safety of what

we want to try. And we're really looking forward to some of these high-tech gene therapies that are curative solutions.

A lot of the treatments are really just palliative that are even in the pipeline right now. And it's when everyone starts to make a significant difference in their suffering, and we really appreciate being able to speak with you and have this dialog to emphasize how fast we need to go. Thank you.

MS. VAIDYA: Thank you so much, Jonathan.

(Applause.)

MS. VAIDYA: Next, we have Loretta Morris. Loretta, are you in the audience? Oh, right there.

MS. MORRIS: Hello. I'm Loretta Morris. I have no financial connection here, just supportive of my friend whose husband is suffering from it.

My question is, having no affiliation with the FDA, what kind of reports -- I understand

you send reports to the pharmaceutical manufacturers. Besides, is there a communication back and forth as they're making progress with their research and development? As an outsider, I would like to know an answer eventually, I guess, about that. Thank you.

DR. DUNN: We don't have time for specific responses, but I'm just going to quickly reassure you that we're intensely engaged in ongoing dialog throughout the spectrum of drug development for any given drug. So there's active and ongoing dynamic dialog. I know you'd probably like a longer answer, but we have to move on. But I wanted at least to address your specific question.

MS. VAIDYA: Next, we have Melanie Rehm. Melanie, are you in the audience? Oh, yes.

MS. REHM: I don't really need a mic. I'm very loud. The heartwarming stories that were told today, and I cannot, you know, follow up with anything like that. So thank you for everybody sharing, and thank you for everybody attending

today.

(Applause.)

MS. VAIDYA: Thank you, Melanie.

Next, we have Jennifer Mann.

(Pause.)

MS. VAIDYA: Okay, great. Thank you, Jennifer.

Karen Clark. Do we have Karen in the audience? Ashley, could you get the mic over to Karen?

(Pause.)

MS. CLARK: Thanks. Since we only have two minutes and I have a lot of really like hefty subjects, I don't really want to delve into them too much. I just wanted to mention that we should also be thinking about issues like genetic discrimination and getting access to -- changing the benefits.

And I'm sure someone will follow me and talk more about HD Parity Act and talk about how we need to get that passed so that people don't fall into the gap or they don't get any benefits.

Because I know when I first put my mom into the nursing home, she wasn't able to get access to an HD specialist for two years. And she wasn't able to get specific HD treatment for two years.

And that was just really horrible at the beginning stages of her disease -- well, not the beginning, but the early to mid-stages in which some of the cognitive and psychiatric symptoms could have been managed a lot better.

So, in terms of quality of life and all of that, and -- I'll just write the rest of my comments in the docket.

MS. VAIDYA: Okay. Thank you, Karen.

Next, we have Louise Vetter.

MS. VETTER: Good afternoon, everyone, and thank you to the FDA for hosting this hearing, and to everyone in the room for contributing your powerful stories. My name is Louise Vetter, and I'm the CEO of the Huntington's Disease Society of America.

I wanted to just raise awareness to everyone in the room, and especially all of the

members of FDA who may not have seen the results of surveys that HDSA ran last year to compile the feedback from the HD community, so many of whom could not be here today. We have 250 or so participants in the room and online, but there are hundreds of thousands of families affected by Huntington's who could not be here.

HDSA ran two surveys focused on the symptoms and the treatments for Huntington's disease. And we had more than 3,600 respondents that provided powerful information related to the symptoms that are most impactful, and also the treatments they are most hopeful for. We've provided top-line summaries to the FDA in advance of this meeting. All of that data is available to you as you mine this area and bring treatments forward.

I also want to pick up on just one other thing that Ken Serbin and several others in the room have mentioned. And that is the need to really consider the needs of pre-symptomatic individuals affected by Huntington's disease.

As we talk about treatments for those who are symptomatic, it is also very powerful to include the feedback of those who are watching the future play out in front of them, the families that they're caring for, and the needs and visions they have. So I would encourage you to listen carefully to their feedback, and I would encourage everyone in the room who may be thinking about this to make sure that your feedback is included in the docket, loudly. Thank you.

MS. VAIDYA: Thank you, Louise.

(Applause.)

MS. VAIDYA: Next, we have Brian Wisnet (phonetic). Did I say that correctly? Brian, are you in the room?

(No audible response.)

MS. VAIDYA: Okay. We will move on to Sharon Thomason. Sharon Thomason?

MS. THOMASON: First of all, thank you so much to the FDA for giving us the opportunity to make our voices heard.

(Applause.)

MS. THOMASON: Those of us who are here are speaking for thousands of others who couldn't be here.

I have two things that I would like to add. First of all, with the treatment of the psychiatric symptoms, we're all familiar with genetic testing, but there is now also genetic testing for psychiatric meds. And I don't know that many people are aware of that yet.

The psychiatrist who treats my son has used it with a number of patients who are very difficult to medicate, and it helps to pinpoint which particular psych meds will help, rather than, you know, trying something for a month and then something else for another month until you hit on the right thing.

The other thing that I wanted to talk about is, we need to change the diagnostic criteria for Huntington's. We need to be able to get a diagnosis on the basis of the so-called "soft symptoms," the psychiatric and cognitive symptoms, and not depend on chorea for a

diagnosis.

(Applause.)

MS. VAIDYA: Thank you, Sharon.

Next, we have Katrina Hamel.

MS. HAMEL: Thank you. I spoke a little bit earlier about my family's situation. And there was just something that I had left out that I thought was, you know, not mentioned. And that had to do with my mother becoming homeless due to her situation.

So, I'll just read a little bit of what I wrote, quickly. That my mother died three years ago at the age of 50 from complications of HD. She suffered for half of her life. Half of those years she was suffering. She left her family of three kids, her husband, and she left Connecticut with a man that she didn't really know. Of course, that was HD-related. And she just went on a whim. She just thought it would be a good idea.

So, when she came back, I was 20 years old, and she was 40, and both of us were pregnant. And my, my -- I'm sorry. But my son is two weeks

older than his uncle. She left again, and this time, you know -- and these relationships created her to be homeless. And at that point, you know, she lost her son to social services due to safety issues. And we didn't know what was going on because (inaudible).

When I found her in Connecticut, I picked her up. You know, I got somebody to bring her to LA from Connecticut. And when I found her, when I finally got her, she was full of urine and her hair was one big red blob. She was homeless, had no one and nothing to her name.

She was beaten. She was swollen from taken advantage of, and that's something that a lot of us aren't here to talk about, because if that were the case, if we loved them so much, they wouldn't be out there and homeless. But sometimes situations create that. And for me, our family loved her, supported her. But because she left us, we were unable to know where she was. And she ended up that way because she was no longer able to care for herself and her basic needs.

(Applause.)

MS. VAIDYA: Thank you so much, Katrina.

(Applause.)

MS. VAIDYA: Next, we have Lauren Holder. Lauren, are you in the room? Lauren, could you just raise your hand if you're here?

(No audible response.)

MS. VAIDYA: Okay. And then finally, we have Kenneth Serbin.

MR. SERBIN: Thank you to the FDA for this opportunity. And I just wanted to -- my day job is as a historian. So I do a lot of historical work and reading in social sciences. And I've observed some interesting things.

Panel 1, no men. Panel 2, practically all women representing men. Interesting bias in the data, don't know what caused it, but as a person who reads in social science, I thought it was interesting, especially in this day of gender equality.

And the very sample of the meeting itself is probably skewed because a lot of, as

Louise pointed out, Louise Vetter pointed out, it's a whole big community out there. A lot of those people can't come to a meeting. People like my mom could never have participated in something like this because she couldn't walk or talk, although she lived many years with HD.

And even the people at home looking at that list of questions would have a hard time filling out that questionnaire. So I think we're getting -- and I'm not necessarily saying this is bad. But we're getting a bit of a caregiver bias on what's going on in the disease. And there's nothing wrong with that, but we have to keep in mind that the patients' data needs to be there, too. And that's why the docket will be, I think, extremely important, and tapping into the data that Louise referred to is important, too.

Early on, it was said that HD is not a very common disease. It's my understanding that among the rare diseases, it's one of the most common. In fact, hundreds if not thousands of -- I mean, in the past two decades there have been

thousands and thousands of papers published on Huntington's disease. So it's a very well-known disease.

And Michael Hayden's work, and others, have shown that there may be many more HD people out there. In the community, we constantly talk about the fact that there are probably more than 30,000 affected individuals out there.

(Applause.)

MR. SERBIN: Regarding pre-symptomatic people such as myself, most of us don't get tested because of the immense fear of the disease and the fact that there are no treatments. I see this in my own personal experience. And there's also, associated with genetic testing and getting your results, a lot of suicidal tendencies.

And I myself, when I first saw what was happening to my mother, before I got tested, I thought to myself, "There's no way I'm going to go on in life with this, if I have to live like my mother." And I did think a lot. I never actually had, I guess, a real suicidal thought. But I had

a lot of fantasies, you know. "I'll get my family together, my friends together, and I'll just, you know, drink some hemlock like Socrates and say good-bye to the world." I used to think that way.

But once my daughter was born and once I got more involved in this movement, I saw that I couldn't possibly take my life. But I know that's a thought that occurs to a lot of us having to face this situation.

So, I guess I just want to close by saying that that pre-symptomatic community out there needs to really be a part of the conversation. And thank you to the FDA for this opportunity.

MS. VAIDYA: Okay. Thank you, Kenneth.

(Applause.)

MS. VAIDYA: So, Billy, would you like to say something?

DR. DUNN: Yeah, I just wanted to make a brief comment. Thank you for all the comments. I think that concludes the comment session.

MS. VAIDYA: We actually have one person

on the phone.

DR. EGGERS: No, no, no. One more person.

MS. VAIDYA: Oh, one person here. Okay.

But, Billy, did you want to address something?

DR. DUNN: Sure. This is as good a time as any. A number of ya'll said "thank you" to the FDA, and I just want to make a brief comment. We appreciate your thanks. But we signed up for this. Ya'll didn't. You know, this is our job.

(Applause.)

DR. DUNN: I want to say thank you, and I want to encourage all of my colleagues from the Agency here to say thank you for you to your courage to come here to share your stories with us. Thank you very much.

(Applause.)

DR. EGGERS: So, there's -- so I think we have one more person who wanted to -- who had signed up, didn't get signed up on time. But I do also, I had meant to say and put up on a resource,

so before we go to that last speaker, that you have talked, and the last commenter talked more about the personal struggles of the condition.

And we just wanted to remind you that there are resources available. And the Huntington's Disease Society of America reminded us of their hotline. So, please seek resources if you need them.

MS. VAIDYA: Okay. So, finally, we have LaVonne.

MS. GOODMAN: Ken Serbin will be a very hard act to follow. And my comments are really much shorter. I'm LaVonne Goodman. My first husband died of Huntington's disease. I'm a physician who takes care of Huntington's disease families, and I'm a patient advocate. So I wear many hats. I also facilitate a support group and help monitor other support groups in the Northwest, near Seattle.

We conducted a survey there similar to the one that HDSA did, but we added a couple of questions. And that was about slowing down

progression. And the questions had to do with how much disease slowing would be acceptable before you would want to take a drug that was given orally or a drug that was given intravenously or a drug that was given intra-cerebrally?

And those results were interesting from a number of perspectives. One, they weren't so sure they wanted to take something if it were 10 percent. It needed to be closer to 25 percent. That was their perception. I queried only people who had the gene or had symptoms already. This was not a care provider -- excuse me, a care partner or caretaker answer.

And it was interesting to me that, and I think interesting for everyone, that the discomfort with taking something via spinal fluid, by an LP, was as frightening as was an intra-cerebral delivery.

The other thing that I think the FDA needs to think about, and perhaps our research community needs to think about, too, is that there was a question in there about, if there were gene-

lowering therapy, what risks would you take and how much benefit would you want before you would take that risk?

And another question that had to do with slowing down the disease progression by the same amount if it were -- and it wasn't gene-lowering. People were more interested in taking the gene-lowering therapy, although something that were equally neuroprotective was not as acceptable.

And I think the FDA and our community needs to educate people that neuroprotection is neuroprotection, whether it's gene therapy protection or not. And I think our community isn't aware of that, and perhaps that's because the FDA won't let people taking -- or drug companies taking things into clinical trial that may have some neuroprotective benefit from mentioning that.

MS. VAIDYA: Thank you, Lavonne.

MS. GOODMAN: Thank you very much.

MS. VAIDYA: And if you have any additional comments, please submit it to the

docket.

(Applause.)

MS. VAIDYA: So that ends our Open Public Comment period. Before we move on to the last agenda item, I would like to let you know that folks will be going around and picking up clickers. And also, we have evaluation forms at your tables. We really want to hear from you to get your feedback on how this meeting went today. So, please fill it out. Thank you.

And finally, I'd like to call upon Dr. Eric Bastings to the stand for our closing.

DR. EGGERS: As Eric is coming up, we have one earring that we found. So if it's your earring, come find us

DR. BASTINGS: Hi. I am Dr. Eric Bastings. I'm the Deputy Director of the Division of Neurology Products. I want to thank all of you today for participating in this meeting. I think what we heard from you today is incredibly valuable. And I want to assure you that we will use that information as we review new drugs for

the treatment of Huntington's.

There was a lot of information discussed this morning. And I would like to use the next few minutes to summarize some of the key points of what we heard.

So, first, we heard loud and clear that the behavioral and cognitive aspects of the disease have a very deep impact on patients and on their families. In particular, we heard about the memory problems, the difficulties with mental processing, difficulties with initiation, and difficulties with speech.

We also heard of problems with swallowing and the deep impact that that can have on patients, and sometimes the embarrassment that that can give when people have to interact with others and be outside of their homes. And we heard the deep impact on all of these symptoms sometime making friends stay away, other family members, other families stay away, leading to isolation of patients. So, all of these cognitive symptoms are very important.

Another important aspects are the behavior aspects of the disease. We heard of sleep being a big problem, in part because of movements that people, patients can have during the night, and issues with circadian rhythm. So, sleep is a big problem.

Depression is identified very often as a big impact as well. Anxiety, irritability, and apathy are also aspects of the disease that need to have a lot of attention.

The motor symptoms are, of course, still present. They're not presented by most people as the most problematic, but they also deserve treatment, as you know. We have a drug available to treat chorea. And a number of people reported some good success with the drug, but it's not an absolute treatment. And there is still a major and medical need in that area, as well.

Gait is often reported as a problem in the disease. And we need to have some treatments that would address the issue of gait and movement. And again, speech and swallowing are reported by

many as being a problem.

In terms of treatment available, we all know that there is a major, unmet medical need. We at the FDA fully recognize the devastating nature of Huntington's and the need to have more treatments, not only to treat the motor symptoms, but especially to treat the behavior aspects and the psychiatric aspects of the disease and the cognitive symptoms.

We heard of people using a variety of treatment. They can be non-drug therapies, exercise, nutritional supplements, and they can be a variety of drugs such as antidepressant, antipsychotic medications, and tetrabenazine. Anti-epileptic medications are also used.

I think this is a good point to assure you that safety is really not what is keeping your drugs from coming to the market. Again, we really recognize the severity of the condition, and we take that fully into consideration as we balance the risks and the benefits of the treatment. And for a disease like Huntington's, we really would

tolerate some significant safety issues before considering not putting a drug on the market.

And again, we want to exercise flexibility for conditions like this. And we are fully aware of, you know, the major unmet needs.

Finally, I want again to thank you for coming today. I want to assure you that we share the same goal, which is to find a cure for this devastating condition, and on the way to that, to find any way to slow down the disease. If there are treatments that can be given before patients become symptomatic -- I heard some comments about that before -- that certainly it would be a very good area for targeting treatments.

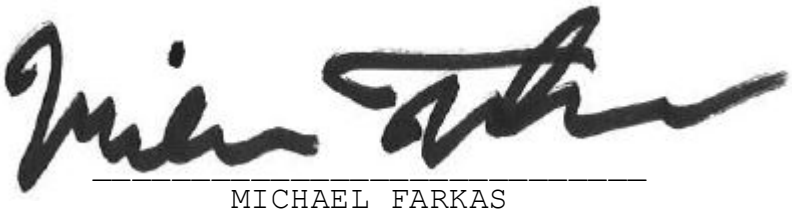
And I want to assure you that the team present here will be extremely responsive and supportive for the development of your drugs for Huntington's. So, thank you very much for coming today.

(Applause.)

(Whereupon, at 12:21 p.m., a luncheon recess was taken, to reconvene at 1:27 p.m.)

CERTIFICATE OF NOTARY PUBLIC

I, MICHAEL FARKAS, the officer before whom the foregoing proceeding was taken, do hereby certify that the proceedings were recorded by me and thereafter reduced to typewriting under my direction; that said proceedings are a true and accurate record to the best of my knowledge, skills, and ability; that I am neither counsel for, related to, nor employed by any of the parties to the action in which this was taken; and, further, that I am not a relative or employee of any counsel or attorney employed by the parties hereto, nor financially or otherwise interested in the outcome of this action.



MICHAEL FARKAS
Notary Public in and for the
State of Maryland

My commission expires: 6/27/2018

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