Transcript: Austedo; Austedo XR Roundtable Event, April 16, 2025, Medicare Drug Price Negotiation Program Public Engagement Events

This transcript was lightly edited for readability.

Introductory Remarks

Moderator, RTI International

Hi, everyone. My name is **[MODERATOR]**. I'm so excited to talk with you all today. **[REDACTED]** I work for a company called RTI. And I also want to introduce my colleague, **[SECONDARY MODERATOR]**, who's here with me today as well. **[SECONDARY MODERATOR]**'s going to be speaking to you all at a couple points in our discussion today.

The Centers for Medicare & Medicaid Services, also called CMS, is convening this patient-focused roundtable event and others as part of the Medicare Drug Price Negotiation Program. So, the purpose today of this event is to hear from you all. And you're a group that includes patients, caregivers, and patient advocates, about your experiences with the conditions and diseases treated by Austedo, with Austedo and other medications for the same conditions.

One thing I'll mention today is that there's Austedo and there's also Austedo XR, as probably many of you know. I'm just going to say Austedo, just for simplicity. But we're referring to both Austedo and Austedo XR in today's discussion.

The information shared during the events will help CMS understand patients' experiences with the conditions and diseases treated by the selected drugs, patients' experiences with the selected drugs themselves, and patients' experiences with other drugs that are used to treat the same conditions as Austedo.

CMS may use this information in negotiating Medicare pricing with the manufacturers of the selected drug. Your experiences and perspectives are very important to us, and we genuinely appreciate your time today. Next, let's watch a brief video from CMS leadership so that you can hear from them about how much they value your time and input today.

CMS Remarks

00:01:58

Steph Carlton, Deputy Administrator and Chief of Staff, Centers for Medicare & Medicaid Services

Greetings, everyone. I'm Steph Carlton, the Deputy Administrator and Chief of Staff at the Centers for Medicare & Medicaid Services, or CMS. CMS administers Medicare, our country's federal insurance program, for more than 65 million older Americans and people with disabilities.

I deeply appreciate each one of you for taking the time to join us today. Lowering the cost of prescription drugs for Americans is a top priority of President Trump and his administration. As the second cycle of negotiations begins under the Trump administration, CMS is committed to engaging with stakeholders for ideas to improve the Negotiation Program.

In January 2025, CMS announced the 15 Medicare Part D drugs selected for the second cycle of price negotiations. Medicare's ability to negotiate directly with drug companies will improve access to some of the costliest drugs while fostering market competition and continuing innovation.

Our priority in negotiating with participating drug companies is to come to an agreement on a fair price for Medicare. Promoting transparency and engagement continues to be at the core of how we are implementing the Medicare Drug Price Negotiation Program. And that is why the process for negotiation engages you, the public.

This event is part of our effort to hear directly from a range of stakeholders and receive input that's relevant to the drugs selected for the second cycle of negotiations. Thank you again for joining us. Your input matters. And next, stay tuned to hear from the event moderator to give you more details on what to expect during this event.

00:03:54

Moderator

Also, I want to make you all aware that staff from the CMS will be sitting in on this event so that they can hear your experiences and opinions directly from you today, and I want to hand it over to them for a moment so they can say hello. And I think it's **[CMS STAFF]**, is that right?

00:04:08

CMS Staff

Thank you, **[MODERATOR]**. Hi, I want to welcome everyone on behalf of CMS. And as **[MODERATOR]** said, we have staff from CMS from the Division of Policy and from the Division of Drug Price Negotiations on the call today.

We want to thank you for participating. And we're looking forward to hearing about your experiences during this roundtable discussion. We're going to go off camera now, so you can focus on the discussion, but we will be here listening. Thank you.

Housekeeping

00:04:37

Moderator

Awesome. Thank you, **[CMS STAFF]**.

Before we begin, I do also want to go over some, what we call "housekeeping" items, and just some ground rules that, so everyone knows what to expect. So first, we hope that you all can contribute your perspectives today throughout the session. However, if there's a question you don't want to answer, that's totally okay. You don't have to answer any questions today that you don't want to.

Please minimize background noise by finding a quiet location away from other people and distractions. Be sure to also silence your cell phone, so it's not ringing during our discussion today. And then you can also, on Zoom, you know, mute yourself when you're not speaking. That way, we don't hear background noises during our discussion.

This discussion is not open to the press or to the public. We're going to use all your first names today during our discussion to protect your privacy, and please do not share any unnecessary personally identifying information or personal health information about yourself today during this discussion.

We are going to audio and video record today, which we already are doing now. But these recordings will not be shared publicly. After this event, CMS will prepare transcripts that have participant names and identifying information removed, but then these transcripts will be made public.

I also want to go over a couple other things. Video, please, thanks in advance for keeping your video on throughout discussion. That way, I can see you all, and I can see if you want, you want to say something. This session will last about 90 minutes total. I do have a discussion guide. There's some questions I want to ask today, and I do have to keep to the time. So it's possible that I may have to move our discussion away from an interesting point just so I can cover interesting, other interesting points. So please don't get offended if I have to move the discussion along.

If you get disconnected or something happens, there's an email address on the screen now – IRADAPStechsupport@telligen.com. You can email them, and a friendly person on the other side will help you with any technical problems that you have.

If you need to step away from our discussion today, that is totally fine. Just turn off your camera and microphone and rejoin as soon as you can. You don't need to tell me that you're going to leave. You don't have to ask for permission. You're not second graders. This, if you need to go, that's totally fine and come back as soon as you can. Okay?

All of you are going to have a lot of great opinions to share. I'm going to ask you to try to speak one at a time. I may occasionally have to interrupt if there's two or more people speaking just so that I can hear from everyone. If you want to say something, you feel like you are having trouble getting your voice in, you can also press the raise hand button in Zoom. That will let me know that you want to say something.

Finally, I just want to hear your opinions. All of you are going to have different opinions, different experiences, and that is totally fine. That's what we want to hear today. Your opinions and experiences will differ, and we want to know what each of you thinks honestly about our topics in our discussion today.

Okay, that was a lot of me talking. I want to pause for a second and see if you all have any questions for me before we get started into the session. Just more like housekeeping questions. All right. All right, well, let's get rock and rolling then.

So, I want to begin our discussion by asking each of you to introduce yourself and take about 30 seconds, and I'm going to probably call on you all just to make it a little bit easier. Tell me your first name, condition that you have experience with if you do, and then tell me whether you'll be sharing your personal experiences or those of a loved one, or whether you're sharing the patient experience, or from the perspective of a patient advocate.

I'm going to start with **[Participant 1]**. Hey, **[Participant 1]**.

Discussion

00:08:28

Participant 1 (registered as a caregiver)

Hi, I'm **[Participant 1]**, and I'm here because of my mom. She is currently taking Austedo and I'm not her fulltime caregiver. She is in a nursing home, but I'm really the only other person in her life and dealing with her TD [tardive dyskinesia], and the problems that have arose from it has been kind of exhausting on both of our parts. I feel like it's necessary to have this medication. But I also, for me, because of how Mom got the TD, I worry about the medication that actually caused the TD, too.

And that's something that I feel that more people need to realize is that there are other things that can cause things. And, so maybe letting doctors and patients know that certain medications can cause tardive dyskinesia might make it so prices are cheaper in the long run, so, because you won't be causing the problems.

00:09:52
Moderator:

Thanks, **[Participant 1]**. Again, just for intros, we'll do about 30 seconds just so that we can hear from everyone, and we'll actually delve into that a little bit more, **[Participant 1]**, today. So don't worry. Next on my screen is **[Participant 2]**. Hey, **[Participant 2]**.

00:10:07
Participant 2 (registered as a caregiver)

Hi. I am a caregiver for my husband, who has Huntington's disease. I am also a retired **[REDACTED]**. I had to retire to become his caregiver. My husband is on Austedo XR.

00:10:27
Moderator

Thank you, **[Participant 2]**, for joining. **[Participant 3]**?

00:10:31
Participant 3 (registered as a representative of a patient advocacy organization)

Hi. My name is **[Participant 3]**. I work with people living with Huntington's disease as part of the Huntington's Disease Society of America, so patient advocacy. And prior to this role, I actually worked at, in a medical setting, working directly with patients and families living with Huntington's for almost 20 years. Many, many, many, many patients and family members who've used Austedo to treat the choreiform movements of Huntington's.

00:10:57
Moderator

Great. Thank you so much, **[Participant 3]**. **[Participant 4]**?

00:11:01
Participant 4 (registered as a caregiver)

Good morning, and thank you for the opportunity to speak about Austedo. This is something near and dear to my heart. I am at risk for Huntington's disease, which means I may have the gene, but today I'll be speaking on behalf of my sister, who passed away in 2023, and my brother, who passed away in 2024. Each of them who took Austedo and I was, quote unquote, their bookkeeper, so can speak about the issues that were caused by trying to get medication coverage financially for them.

00:11:32
Moderator

Awesome. Thank you, **[Participant 4]**. Thank you for coming. And **[Participant 5]**?

00:11:37

Participant 5 (registered as a caregiver)

Hi. My name is **[Participant 5]**. I'm a caregiver for my husband, who has Huntington's disease. We also just recently found out that my stepdaughter has inherited the gene as well. I will say this real quick. I'm here to tell both my husband's story and mine. I never know what my reaction is going to be, so if I cry, I apologize.

00:12:00

Moderator

Totally fine. Thank you for joining, **[Participant 5]**. We appreciate it. **[Participant 6]**?

00:12:06

Participant 6 (registered as a caregiver)

Hi, I'm **[Participant 6]**. I wear a lot of hats. So, I'm **[REDACTED]** of Help 4 HD, which is a nonprofit serving the Huntington's disease community. I also, my husband passed away from Huntington's disease six years ago, and I have three children at risk. My husband was on everything, from tetrabenazine to Austedo.

I have thousands of friends across the United States that I support on a daily basis, and work with, talk about everything from Austedo to living with HD [Huntington's disease], so I wear many hats. But thank you for having us today and listening to our stories.

00:12:43

Moderator

Thank you, **[Participant 6]**. **[Participant 7]**?

00:12:49

Participant 7 (registered as a representative of a patient advocacy organization)

Yes, thank you very much. My name is **[Participant 7]**. I'm the **[REDACTED]** of the National Organization for Tardive Dyskinesia, and representing patients approximately, possibly as many as 750,000 in the U.S., who have tardive dyskinesia, movement disorder caused by primarily antipsychotic use, and very proud to be, or very happy to be with you, to share both their information. Also, I do have some experience as a caregiver for someone with tardive dyskinesia, but it, luckily her case was not that severe. And so it wasn't a full-time position but have some experience. I will be possibly sharing some experience as a caregiver as well. Thank you.

00:13:40

Moderator

Great. Thank you, **[Participant 7]**. **[Participant 8]**?

00:13:44

Participant 8 (registered as a patient)

Hi, thank you for allowing me to share my story. I suffer from TD. I had a really bad bout of it with the left side of my mouth. People were asking me if I had a stroke, and it was really debilitating. It made my mental health situation much worse, and I am happy that you are allowing me to share my experience with you. Thank you.

00:14:13
Moderator

We're happy you're here to share your experience, too. **[Participant 9]**? **[Participant 9]**, sorry.

00:14:20
Participant 9 (registered as a representative of a patient advocacy organization)

It's **[Participant 9]**. But...

00:14:23
Moderator

[Participant 9], sorry. I had three options, and I picked the wrong one. Sorry, **[Participant 9]**.

00:14:27
Participant 9 (registered as a representative of a patient advocacy organization)

So, **[REDACTED]** and I lead PMD Alliance. We're a patient advocacy organization supporting families with movement disorders which includes both Huntington's and tardive dyskinesia. For the purposes of this panel, and actually, I do have more experience with families with TD, so that works out well, since there's a lot of HD experience already in this roundtable.

00:14:54
Moderator

Great. Thank you, **[Participant 9]**.

For our next part, we're actually going to use the chat window just for a quick yes or no. Let me know in the chat window, have you or a loved one taken Austedo, either currently or in the past, and just put either yes, no in the chat. Again, have you or a loved one taken Austedo, either currently or in the past, and you can just put yes or no in the chat window. And if you can't find a chat window, you just want to say it, that's totally fine, too.

00:15:23
Participant 8 (registered as a patient)

I don't have the chat window.

00:15:25
Moderator

That's totally fine. But I think, is it yes for you, **[Participant 8]**?

00:15:28
Participant 8 (registered as a patient)

It's yes.

00:15:29
Moderator

Okay. Awesome. Thank you. All right. So, I'm seeing a lot of yeses. I think all yeses for the most part. So, thank you all for introducing yourselves and telling us your experiences that you'll be drawing from today. Let's start talking about you and your loved ones or patient experiences with the conditions that are treated by Austedo. And we know there's going to be a lot to talk about, with the question, so please feel free to raise your hand again, share your experiences, or you can just say it or put it in the comments.

Austedo can treat two separate conditions that we're going to talk about today that have been both discussed today. One is for tardive dyskinesia. One is for chorea associated with Huntington's disease, and I want to start first with chorea associated with Huntington's disease, and then we'll come back to tardive dyskinesia. But first, in general, for those who know people who have chorea or treat people with chorea, or you have it yourself, in general, how does chorea affect people's daily lives? Yeah, I'll say it again, how does it affect their daily lives?

[Participant 4], go ahead.

00:16:43

Participant 4 (registered as a caregiver)

So, with Huntington's disease, the chorea part of it may be a major side effect or a major issue with the disease, or it cannot be. Sometimes it's more of a cognitive decline issue. In the case of the siblings that I'm going to talk with you about, they both had very drastic chorea, and so Austedo was absolutely essential for them in reducing their movement, which made their lives so much better if you don't have to worry about stumbling and falling all the time because you have less movement. It makes it easier to eat. It makes it easier to swallow. It makes it easier to communicate. The chorea is a major, major factor for a good number of people with Huntington's disease.

00:17:29

Moderator

Thank you, **[Participant 4]**. **[Participant 2].**

00:17:33

Participant 2 (registered as a caregiver)

Yes, so my husband began experiencing more chorea over the last couple of years. He began falling more with injuries to himself. His knees are all scarred up. Also, taking him to get a haircut could be a major, major problem. His barber noticed significant benefit from the Austedo. She was so pleased that she didn't have to chase him around with the clippers.

The other thing is weight loss. During the pandemic years, and for the two years after, my husband had a significant weight loss, probably due to the excessive movements, the increased movements. He lost eight pounds in, in like two months. He was very thin, very... looked emaciated. We got him on Austedo, and he has gained 15 pounds.

00:18:57

Moderator

Thank you. **[Participant 5**], I see also you have your hand up. Go ahead, **[Participant 5**].

00:19:01

Participant 5 (registered as a caregiver)

My husband was a diesel mechanic when he could still work, and he would drop tools and mess up decals at work, and that kind of thing. And once we got diagnosed, and I say 'we' a lot, one of our doctors suggested going on a medication. Well, the first medication he tried was not a good fit for him, and we've used Austedo since it became available in 2017.

My husband is able to sit still. He can enjoy playtime with our son, who is at risk. He can enjoy downtime with our daughter, who just got tested, and it used to be a case of he would be highly embarrassed to go to public places because of his movements. Ever since starting Austedo, his movements have been so controlled, and he doesn't mind going out in public. He enjoys spending time with the family.

His weight, as **[Participant 2]** was talking about her husband, we've not had the issue of the significant weight loss that a lot of Huntington's patients have due to the choreatic movements, and we just feel that the Austedo has been, for lack of a better term, a miracle drug for a disease that there is no miracle. There is no cure, there is no treatment. You can only treat the symptoms, and we just feel that this has significantly impacted our life in a positive way, and I will shut up now.

00:20:37

Moderator

You're totally fine. Thank you, **[Participant 5]**. I appreciate that perspective. **[Participant 6]**, go ahead.

00:20:42

Participant 6 (registered as a caregiver)

Yeah, I think that what everyone said is so important. But I think one thing we really need to focus on, too, is generational trauma. So, a lot of times with our public impact that we face when we're in public, I mean me and my husband were asked to on a cruise to go back to our room because he was drunk in public, and he was embarrassing. We would look across my little children playing soccer and people teasing his movements and mocking him, falling, acting like they were falling and tripping.

So, the public impact that my children had to face, knowing they are at risk, too. And that's something that really needs to be thought about. We are a terminal disease. We have very limited time with our loved one. The quality that we have with our loved ones is so important. On top of my husband was 70 pounds when he passed away. 6'1", cop, fit, died with skin and bones like it was the choking, the falls, the injuries, the hospitalizations. You know, I can go on and on about impact of chorea and my husband, and how terrible it was. But now, when he's gone, the impact on my children, I'm glad that we got some symptom management towards the end of his life to help their quality of life with their dad at the end of his life, through Austedo and different drugs that were available to us later that weren't in the beginning of our journey.

00:22:02

Moderator

Thank you, **[Participant 6]**. **[Participant 3]**?

00:22:10

Participant 3 (registered as a representative of a patient advocacy organization)

I just want to say thank you to everyone who shared your personal stories, and I think that's something I just wanted to emphasize is what you're hearing with Huntington's disease, the symptoms include motor, which is the chorea movements, cognitive and psychiatric symptoms.

But what all the personal stories just emphasized to me was, the motor symptoms generally start in what we call the prime of life years, in the thirties to late forties. So, people are still working. They're still engaging in society. They're raising families. They're living life as the symptoms start. And it's drugs like Austedo that allow them to help manage those choreiform movements so that they can still participate.

And without drugs like Austedo, people are afraid to go in public. They have experiences like **[Participant 6]** shared where they are accused of being drunk or on drugs, and their tendency to become isolated increases which leads to further psychiatric implications of feeling like you're ostracized from society. So, I, I just wanted to point out that fact that it is a prime of life disease when these symptoms start, and it is essential for drugs like Austedo that have the ability to control the movements that allow people to stay engaged.

00:23:34

Moderator

[S]o **[Participant 3]** was talking about managing symptoms, and I wanted to talk to you all about chorea. But for you all, what aspects of chorea are most important to your loved ones, or to other people, to have managed or treated? **[Participant 4]**, go ahead.

00:24:02

Participant 4 (registered as a caregiver)

I think the most important thing, especially for my brother, was because we were controlling his movement and didn't have to worry as much about his falling and choking, he was able to stay independent for a lot longer than he would have without the Austedo. Because of the Austedo and controlling that movement, he lived on his own for probably a good 12 to 18 months longer. Then we moved him to an assisted living facility, where he was able to stay for a much longer time. We did not have to step up care. Once we moved him to the Austedo, Austedo XR because we only had to remind him once a day to take the medication. We didn't have to have somebody there on a regular basis to remind him about meds.

So, I think the most important thing for him, as relates to Austedo, was a longer time of independence, which we all know we want to happen to us. We want to be independent for a much longer time then we would have the opportunity to do for... I will say that with my sister, more importantly, because she had no side effects, more importantly, she was able to eat because she didn't have the movement and trying to get the food to her mouth. And so I think that's something that everyone would agree with, too, is that decreasing that movement and being able to eat also increases your length of independence, as well.

00:25:29

Moderator

And then I'm going to, I see both **[Participant 2]** and **[Participant 5]** have their hands up. I think I'm only going to have time for **[Participant 2]**, because I want to turn to tardive dyskinesia. So **[Participant 5]**, I'm sorry, but we'll come back to you later. But, **[Participant 2]**, you wanted to say something?

00:25:42

Participant 2 (registered as a caregiver)

Yeah, **[Participant 4]** hit most of it. I would say, the falls. My mother-in-law spent a lot of time in the emergency room. She did not... in the emergency... for the chorea. So the falls, and keeping my husband...

00:26:01

Moderator

Awesome thanks, **[Participant 2]**.

I want to give an opportunity for people talking about tardive dyskinesia to speak as well. And for, for those folks, in general, how does tardive dyskinesia affect your or your loved one's day-to-day life?

[Participant 8], I see you have your hand up.

00:26:25

Participant 8 (registered as a patient)

For me, it was very, very difficult, because doctors were not familiar with TD at that particular time when I got it a couple of years ago, and so I was sent to the emergency room. And I was told that maybe I had a stroke. They checked me for that because my mouth was just hanging really low and my speech was slurred, and as a result I became a very introverted person. I'm usually extroverted.

There was a great deal of shame associated with what I was going through. I became a shut-in, and once I discovered, my doctor discovered TD and then Austedo, I was able to get back into the world and become a part of it, and so I cannot tell you enough how this medication has really changed my life. It really turned me into someone who was just ashamed to talk to people, afraid, ashamed to look people in the eye. Despite my mental health problems, this piled up on it and made it even more disturbing.

So, I think Austedo really made a difference in my life. If not for Austedo. I would not be on this call. I would be someplace hiding, crumbling into myself, and just kind of saying, why, God, why me.

00:28:12

Moderator

I'm glad you're here with us today, **[Participant 8]**. **[Participant 9]**?

00:28:15

Participant 9 (registered as a representative of a patient advocacy organization)

Yes, so well said **[Participant 8]**, and the comments that **[Participant 6]** and some others mentioned about that, the stigma and the public shame, the symptoms of tardive dyskinesia are exacerbated by stress and anxiety. And so, it becomes this vicious cycle where being worried that you're going to, you know, someone's going to call you out or shame you publicly, or stare, exacerbates the symptoms, and then, like **[Participant 8]** said, people retreat into their homes oftentimes, like some of the HD stories, are written up at work, for you know, being accused of being high or drunk and just withdraw from life, you know. Can't work, can't be independent, can't socialize, can't be with their families, aren't going to kids' weddings. So just wanted to underscore what **[Participant 8]** said so well, and talk about how, just that, that vicious cycle just preys upon people, and removes them from their normal lives.

00:29:22
Moderator

Thanks, **[Participant 9]**. **[Participant 1]**, I see your hand's up.

00:29:30
Participant 1 (registered as a caregiver)

Yes, with my mom, I mean over the past year she has ended up getting pneumonia, which she currently has at this time. I think we're on the fourth bout. Because of her constant mouth movement, she has a very hard time swallowing, and she gets pneumonia, which is associated with getting food bacteria in the lungs, and so forth. She spent more days in the hospital than she really should have had to, and so forth.

But the other part is, I don't get to see her in person very often. We have to communicate over the phone. And it is definitely a test of my patience to have to talk with her on the phone, because I can't understand her. And I know it's a test to her patience because she just wants to be heard and understood. And I'm constantly having to say, "Okay, slow down, Mom, enunciate." And I wouldn't want people telling me that, let alone, your own daughter, but it's the only way I can understand her.

So it's really hard not being able to communicate with your loved ones, because you have a condition that prevents you from doing that, but also on the stigma part of it, she makes, she lives in a nursing home, and she makes a lot of the other residents very uncomfortable, because not only of the mouth movements, but the hand movements also. She touches things a lot. People think that she is trying to steal things or she's being inappropriate. And that makes it very hard on her. Mom's TD has definitely come from a mental health medication that was prescribed later in her life that should not have been. And I addressed that with a doctor at the time. I wasn't listened to. And now my mom is suffering.

00:31:50
Moderator

Thanks, **[Participant 1]**. I'm sorry to hear that.

For those who are here and want to talk about tardive dyskinesia, what aspects of tardive dyskinesia are, do you think, the most important to people to be managed or treated? So again, what aspects of tardive dyskinesia are most important to be managed or treated?

00:32:17
Participant 7 (registered as a representative of a patient advocacy organization)

[MODERATOR], I might chime in a little bit on this. You know, it's interesting. We run support groups at **[REDACTED]** for people with tardive dyskinesia. And we asked this question, what is the most problematic or troubling part of tardive dyskinesia? And being a movement disorder, we anticipated that people would say a specific type of movement. But we actually got back that, interestingly, the cognition aspects, the difficulty with cognition was actually one of the most prevalent symptoms that was problematic or troublesome, and that was surprising to us.

And there are some studies that compare people who have schizophrenia with tardive dyskinesia versus schizophrenia without tardive dyskinesia, and they show that there is a difficulty in tardive dyskinesia, we don't exactly know why, with cognition.

So, it could go back to some of the underlying anticholinergic aspects. Sorry to use that word, but basically some of the medicines that are treated that are used, such as antipsychotics, can go ahead and cause difficulty in cognition, so that could be part of the aspect.

But that was a surprising, revealing part of it for us. Of course, embarrassment and self-consciousness. **[Participant 8]** talked about that **[Participant 1]** talked about that. That's probably at the top of the list. A recent survey showed that greater than 75% of people with tardive dyskinesia, if it's not properly managed or treated, feel embarrassed or self-conscious because a lot of the primary focus on tardive dyskinesia, as far as movement goes, with the orofacial.

So the movement of the tongue, the grimacing. A lot of those very simple things or sound like they could be minor, could actually be causing a major impact on the way, whether they're being misconstrued or just the embarrassment factor. So, it really causes a problem with people socializing. Or if they have a career where they're even making a Zoom call, for instance, in this modern day, they could be making movements that cause them to feel embarrassed and affect their socialization. So, on top of the impact on speech and eating and swallowing and even sometimes breathing, those are probably the primary things that we have seen as, by working with patients, and just wanted to express that.

00:35:15

Moderator

Thank you, **[Participant 7]**. And **[Participant 8]**, did you have something you wanted to say also?

00:35:19

Participant 8 (registered as a patient)

Yes, yes, I did. Because of COVID, I find that I am meeting with my mental health professional via Zoom. And there were things that should be asked that should be part of the overall interview or visit. For instance, all my teeth fell in the bottom. They fell all the way. They slid all the way to the side. And I think that there are a lot of questions that should be asked, that normally should not be, that normally aren't asked. Like for me. I had to spend more for dental service, more than I paid for my car.

And I think that there should be a list of questions that should be compiled to ask people. Because if you're seeing people on the screen, there are questions like, okay, have you had dental problems? Or do you find yourself moving more often? Because you're just looking at the face, and I think that there should be some kind of process, or some kind of wording, to just see what the whole complete body is, how it's operating, instead of just how are you doing? We hope everything is going well for you, and just things that, questions that you don't normally get asked.

00:36:56

Moderator

Great. All right. Thank you, **[Participant 8]**.

I want to change the topics just a little bit, and I want to talk about your experiences with Austedo, and in addition to Austedo, I want to note that we also want to hear your experiences that you've had with other medications like Austedo, to treat either of the two conditions. We call these drugs "therapeutic alternatives." And so, I may use that term today. So, these are other drugs that also treat these conditions.

When you consider potential medications for chorea, to you, what matters most to people? For instance, is it how quickly it works, how frequently it needs to be taken, or so forth. What, what matters the most for taking it?

00:37:53

Participant 6 (registered as a caregiver)

Is this for all of us?

00:37:54

Moderator

Yes, go ahead, **[Participant 6]**.

00:37:56

Participant 6 (registered as a caregiver)

I was just making sure. Sorry. You know, it's funny, I was back in the day, when tetrabenazine finally came to the United States, and we were having to get it back from, in Canada and the FDA approved it. We were so excited. But tetrabenazine was having, you know, we had to take it so often. It was causing severe sedation. My husband was nauseous. He was getting sick. The side effect profile was so large. So, we started getting these better options out, like Austedo and things like that, where we didn't have to take it as often. The side effect profile was so much less.

I just do not want to see a day that we have to go back to taking things like tetrabenazine before we get like a better drug like Austedo, that we have seen over the years has improved life for our loved ones. I just don't want to go backwards and go there. So, the one thing is obviously side effect profile, but also how often we have to take it as caregivers. We're giving our loved ones drugs like crazy all day long. So, to be, especially with HD, all the symptom profile. So anyways, I hope that was answered.

00:39:04

Moderator

That's helpful, **[Participant 6]**. **[Participant 9]**, are you going to speak about TD?

Go ahead.

00:39:10

Participant 9 (registered as a representative of a patient advocacy organization)

I was just going to say that the very basic level, just being able to get it, not having to deal with a specialty pharmacy, and headaches at the doctor's office, being on hold with insurance, and then the affordability, so just really basic access and affordability is really important.

00:39:32

Moderator

[Participant 2]?

00:39:36

Participant 2 (registered as a caregiver)

Yes, I was going to mention that I have my pharmacist hat on a little bit. The access. Yes, that's important, but the VMAT2 [vesicular monoamine transporter 2] inhibitor is the class that your Austedo is in, is the only class approved by the FDA [Food and Drug Administration] for use in Huntington's chorea. We did try off-label medications like antipsychotic, which helped some, but it was especially useful for controlling my husband's behavior. So, there is only one class of drug approved by the FDA for the Huntington's chorea, and again the side effect profile with having something with the extended controlled release is so much better, and the once a day, as opposed to multiple dosing, is huge.

00:40:50

Moderator

And then for those of you, who are speaking for tardive dyskinesia, when considering all the different medications that are available for tardive dyskinesia, to you, what matters most for people – yourself, loved ones and so forth? What's most important?

[Participant 8]? Yeah, go ahead.

00:41:14

Participant 8 (registered as a patient)

Well for me, I have only done Austedo, and it worked really well.

I have not been offered any of the, probably, possibly lower price drugs or anything like that. So, I'm not sure if doctors are offering alternatives that work better for me, or that could possibly work better for me. So, my answer to that question is that I've only been offered Austedo and not alternatives.

00:41:50

Moderator

Okay. And then, **[Participant 7]**, I saw you unmuted yourself.

00:41:53

Participant 7 (registered as a representative of a patient advocacy organization)

Yeah, I was just going to chime in. It's very often the case that if Austedo is not effective, the other VMAT2 inhibitor Ingrezza may be effective. Sometimes, in many cases neither one can be effective, and patients have to kind of search out another option, and according to the American Academy of Neurologists, there are a few other things that they generally recommend. There's a benzodiazepine that people are probably familiar with, called Klonopin. Of course, that... benzos have some things that are not the greatest, as far as like people get dependent on them, but these don't always work. I mean the VMAT2 inhibitors as far as this treatment of tardive dyskinesia are the most studied treatments, the data I can just share you with a quick data point.

The most important part, I think that was your original question. Most important part is, of course calming the symptoms of tardive dyskinesia. We don't have anything that actually solves it or cures it. But you know, calming this, the involuntary movements. And according to clinical studies, 67% of the people who are using Austedo have about 50% of reduction in their involuntary movements. So, it's really hitting the target really well. Ingrezza has similar, has some similar outcomes. If neither one works, there are, like I said, there are some other options that have been studied, but the VMAT2 inhibitors are generally the first line of, in the best studied with the best outcomes. However, there are some things like antioxidants and things like this that we do find that anecdotally, and even in studies, depending on what started the tardive dyskinesia, if you had a first-generation antipsychotic, it causes a lot of oxidation in the brain, so using an antioxidant makes a lot of sense. But that is something that very few doctors actually know about or go down the road of trying to figure out. What was the actual initial cause? Was it a first-generation or a second-generation antipsychotic? But I might be getting into the weeds there, so sorry about that.

00:44:39
Moderator

Totally fine. **[Participant 1]**, I think I saw your hand up as well.

00:44:44
Participant 1 (registered as a caregiver)

[Participant 8] said that Austedo was the only thing that she had taken. I'm pretty sure that is all my mom has ever been offered, also. So, and I honestly haven't, I could not tell you another medication or anything that is used to treat TD.

00:45:04
Moderator

Okay. And actually, I was going to ask that question in just a moment too, **[Participant 1]**. Yes, **[Participant 9]**?

00:45:08
Participant 9 (registered as a representative of a patient advocacy organization)

Yes, it's so handy to have a pharmacist with multiple hats. But yes, it's only Austedo and Ingrezza that are FDA-approved for tardive dyskinesia, and Ingrezza does tend to be more expensive and harder to get. You can only get it through a specialty pharmacy. So that is a factor. And then you mentioned Austedo and Austedo XR. The XR is only once a day, so that is also easier. With tardive dyskinesia, people are often on other medications, and you know, very complex, so the pill burden is high, and it is a factor to consider to the Austedo XR only once a day it really helps with a lot of people in their lives.

00:45:59
Moderator

So, taking Austedo could have benefits and drawbacks. And we're going to talk about them both. But, thinking about people's experiences taking Austedo for, we'll start with tardive dyskinesia first, what are the main benefits of taking Austedo for TD that you've seen for people who take it? What do you like about the drug?

[Participant 8]?

00:46:34

Participant 8 (registered as a patient)

Well for me, Austedo really stopped my mouth from drooping. I mean there's still a slight tilt to the left side of my mouth, but it was all the way down, showing my teeth whenever I tried to speak.

It actually does work, and I'm hoping that by me being here today, I can be an advocate for it, because it has really made a difference in my life. If I was taught at three colleges, and I was unable to enunciate when I got TD, and now, hopefully, I'm enunciating properly. But it really does make a difference, and I'm hoping that I didn't have any side effects other than the fact that it improved my TD. And I'm hoping that Austedo will be approved for Medicare.

00:47:42

Moderator

Others for TD, some of the main benefits that you've seen? **[Participant 1]**, that might be your hand up. Yeah, go ahead.

00:47:49

Participant 1 (registered as a caregiver)

Yeah, with Mom, it, it has helped with actually speaking with her because… **[Participant 8]**, first of all, you enunciate very well. I am very, very impressed with you so much. But it's also when she does get excited, or even upset, or anything like that, it all goes out the window again. But on the whole it has definitely slowed down the movements enough that she is able to communicate better, and also, I mean, because she's not constantly licking around her mouth and such, I mean, she doesn't have the rawness and everything, the chapness, so… But it's definitely helped improve her communication.

00:48:40

Moderator

Okay. Now on the other side, again talking about TD for now, what have been some of the drawbacks or challenges that you or your loved ones have experienced with Austedo?

00:48:59

Participant 1 (registered as a caregiver)

To be honest, I don't think I'm aware of any drawbacks that have happened with Mom, but I'm also not there even on a monthly basis sometimes. So I haven't been, but I don't know of anything that has been so overt that it, it's come to the surface. So, I would say there hasn't really been any drawbacks.

00:49:27

Moderator

And **[Participant 9]**, were you going to say something?

00:49:29

Participant 9 (registered as a representative of a patient advocacy organization)

I just unmuted to say that the people I serve, the biggest drawbacks people stop taking it because they don't find it works, which often I've heard from doctors is a dosing problem.

So, they don't see any effect. And so why spend the extra money, go through the hassle, add that extra potential side effect? Also, some of the side effects people, the nausea, fatigue, brain fog, they experience, and from talking to doctors, it's often hard for doctors to say is that the Austedo? Is it something else? But when patients and their families are making that decision, oftentimes that's what they don't like, and discontinue taking it.

00:50:23

Moderator

Okay, **[Participant 8]**, I want to hear from you. Yeah, go ahead.

00:50:26

Participant 8 (registered as a patient)

Yes, I have to agree with **[Participant 1]**. For me, I can only see an upside with taking Austedo. I don't have the brain fog. I don't have the embarrassment. I'm out there in the world participating again. And for me, that is definitely a plus. I have not noticed any side effects. And I have gone through different medications. And, as a matter of fact, whenever I feel something going on, I know that it's the medication change that's occurring, and not the Austedo, because I've been taking Austedo for years now, and so I find no drawbacks to it.

00:51:14

Moderator

Okay. And I was going to ask again for TD, other drugs? I think I heard it, Ingrezza, or any other drugs? I think that might be the only other one. Is that... okay?

00:51:26

Participant 7 (registered as a representative of a patient advocacy organization)

Well, there have been many different drugs, because tardive dyskinesia started back in the sixties, or even in the fifties, when first antipsychotics first came on the market, and at that time about 30% of the people who were taking these antipsychotics, the first generation, were experiencing the, on top of that the very difficult tardive dyskinesia symptoms were setting in. So, there's been over the decades before 2017, which is, I believe when the VMAT2 inhibitors were FDA-approved for this usage. They've tried everything from Keppra to piracetam, even Ambien with varying degrees of success.

Nothing really has been as well studied as the VMAT2 inhibitors, which are now the only FDA-approved treatments. There are some natural things that do help, that reduce dopamine in the brain. The leading theory behind tardive dyskinesia is that people become hypersensitive to dopamine because of the use of these dopamine blocking medications, such as antipsychotics or anti-nausea medicines.

So basically, anything that can reduce dopamine, and there are some natural things, even branched-chain amino acids, which is a totally natural kind of approach, can be used before going to the VMAT2 inhibitors. But a lot of doctors don't have that information, and they don't know about it. So, they go straight to the VMAT2 inhibitors, which is understandable. But it is a rather costly drug, so getting the knowledge of the nutrition behind what causes dopamine and what could reduce these things that cause that, the things that could limit dopamine in the system are... it's just not that widely known. So, we've pretty much come down to, okay, if you have tardive dyskinesia try Ingrezza, try Austedo and those, and then maybe try Klonopin. You could try amantadine.

I'm sorry the list is long over the decades of the things that have been used, some with and without good effects. Amantadine has been used for people who have multiple different ones. And then there are actually things that are non-drug approaches, such as deep brain stimulation, which is usually safe for people who have the most severe cases. But it is highly, usually highly successful. So that's, when they actually put, if you're not familiar with deep brain stimulation, they put near the movement centers of the brain, they have electrodes that help manage the firing of the movement. So deep brain stimulation has been quite successful, but it's usually reserved for the people with the most severe cases who don't react well or don't... Ingrezza or Austedo doesn't work. Hope, I hope that wasn't...

00:55:16

Moderator

Just for time. I need to move on. **[Participant 1]** wanted to speak, and I'll let her speak, and then I need to...

00:55:22

Participant 7 (registered as a representative of a patient advocacy organization)

Okay, sorry about that.

00:55:24

Participant 1 (registered as a caregiver)

No, I was just, I was shocked because of when he said, anti-nausea medicines. I did not know that was a factor at all until right now, so I have lots of questions going through my head, but we will continue.

00:55:38

Moderator

All right. I want to come back to the folks talking about Huntington's disease, and I think you've talked me into saying chorea instead of chorea [different pronunciation] for chorea, talking about the benefits and challenges of Austedo for chorea. First, what have been some of the main benefits of taking chorea that you've seen and Austedo?

[Participant 5]?

00:56:04

Participant 5 (registered as a caregiver)

For us, just quality of life. My husband has such a quality of life that he wouldn't if he had the chorea movement. Like I said earlier, he's able to spend time with both of our children. We attribute the Austedo controlling his movements for him, being at what's considered late-middle stage Huntington's to still be able to walk a few steps with help. I mean occupational therapy had him walking all over our living room two weeks ago.

Without that, he would never have been able to do that because of that control, because a lot of, I don't know that a lot of people are familiar with just how bad the chorea gets with Huntington's. The only downside of Huntington's is, I mean, the only downside of Austedo, because we've had a great experience like **[Participant 8]** said, no side effects whatsoever, and especially compared to the tetrabenazine, were like **[Participant 6]** said, were absolutely horrendous. That was not my husband, who was taking tetrabenazine. He was a totally different person, and when our doctor suggested Austedo, because he knew it was about to come out and it was about to be available, I got my husband back.

And the only downside about Austedo is the worry about being able to get it. Whether or not, before, when my husband was on my employee or employer-paid insurance, my insurance would not cover it at all. And that is the biggest concern for families like mine is being able to have access to the medicine, which is a part of the reason why we're all here today.

00:57:56

Moderator

Great. Thank you, **[Participant 5]**. **[Participant 2]**?

00:57:59

Participant 2 (registered as a caregiver)

Yes, I'll say again, the weight loss that my husband experienced in 2023, early 2024 was tremendous. I mean, people were really worried about him. I tried everything in the diet. Trying to get him to eat 3,000 calories, 4,000, 5,000 calories a day is difficult with someone who has a movement disorder. One, there's the increased risk of choking. So, the huge benefit with us has been able to get that, the weight gain, and I've had to, I can cut back a little bit of trying to increase that, the food, getting him, you know, because he's able to maintain that weight, I feel less worried. And again the falls. I'm not worried as much now.

00:59:06

Moderator

[Participant 4], I see you nodding your head, so tell me your experiences.

00:59:09

Participant 4 (registered as a caregiver)

I'm going to echo what **[Participant 5]** and **[Participant 2]** have already said that decreasing falls and being able to eat better are amazing, and I've already shared with you that I think that longevity of independence for my brother was the best positive.

Now those of you who know me already, know I'm the eternal optimist. So, I'm going to turn the only negative side effects into the positives that we did, because my brother did have... my sister had absolutely no side effects whatsoever... negative side effects. My brother had two. One, Austedo can cause some increased sleepiness. My brother lived with me for a while. I can tell you that he was not a good sleeper, and once he went on Austedo, he became a good sleeper. He slept longer at night. Whether it was a decrease in medication or decrease in movement, or because of the medication, I don't know, and I don't care. It was a positive side effect for him.

The second thing was, he did experience dry mouth, and because of my dental background, I always worried about his ability to keep his mouth as clean as he should have because of his movement interrupting his ability to clean his mouth. So, I always wanted him to take a medication called xylitol. It's a little tablet that increases your salivary production, and it reduces the activity of the bacteria that causes decay. So, the dry mouth caused him to then take, use the xylitol on a regular basis, and we solved the problem of the decreased decay as well. Even though there were a couple of negative side effects, we turned those into positives. Bottom line was, every single thing for him was simply a positive that less movement, that being able to eat, decreased weight loss, all the things that the girls have already said.

01:00:48
Moderator

You're an optimist like me, **[Participant 4]**. I appreciate that.

[Participant 6]. Go ahead.

01:00:53
Participant 6 (registered as a caregiver)

Real quick, I'm just bringing it back to the caregiver. My life got better when we had symptom management. I got to let my guard down. I got to let go out in public a little bit more with my family and my children. When my husband, when I gave my newborn baby that I delivered, my husband rocked her. I lost a rocking chair, maybe, because he rocked her to sleep every night with his chorea. But in all seriousness, when we finally got symptom management, our lives just got better. My husband's depression went down, his anxiety went down. My depression and anxiety went down. We felt free a little bit. We felt free to be normal. My husband we knew he was going to pass. We knew he was going to die very young. We knew my kids had minimal time with him. Why not have the best quality of life when you have such a short life? My husband died at **[REDACTED]** so he needed the best quality of life we could get while we got it. So, I'm so thankful for the drugs we did have.

Let me also just real quick, backing up. We do sometimes use a multiple drug kind of situation. Like, for instance, my husband was on a VMAT2 inhibitor, but we also had to use risperidone for that breakthrough chorea. So, we need these tools, and if a tool gets taken away from us or we don't have access to a tool, it can rock our whole lives. So, I would say speaking up for the caregivers, it makes our life better, too, and easier.

01:02:20
Moderator

Thanks, **[Participant 6]**.

I wanted to talk also about some of the drawbacks for chorea for Austedo, and I heard [Participant 5], you mentioned, it may or may not be on the formulary, that was one challenge that you experienced. I think, [Participant 4], you mentioned dry mouth. What are some of the other drawbacks or some of the challenges that you've had with Austedo for chorea.

01:02:52
Participant 6 (registered as a caregiver)

If I could just break in, the one thing is, if we lose access, it's mostly the fear of what's going to happen when they [quit] cold turkey. So, sometimes I see families, they lose access immediately, and we're trying to scramble to get samples and trying to keep them on the drug until they're renegotiated to get their drug for another year. So, I think that's a big fear for people, is going off cold turkey, a big scare.

And then my husband did have sedation from VMAT2, but it also helped him sleep a little bit, and I have to tell you sleep was an issue for us. So, once again, sometimes these things have multiple benefit. For us, the sedation was actually not a bad thing. It helped him sleep a little bit better.

01:03:37
Moderator

[Participant 3]?

01:03:39
Participant 3 (registered as a representative of a patient advocacy organization)

I'll let the folks speak about the experiential piece of concerns about losing Austedo or access to Austedo or other meds like that. But some of the larger, the bigger picture issues that we talk about are concerns about changes that would restrict access to Austedo, like having to deal with non-medical switching, having to deal with prior authorization, having to deal with step therapies. Those are things that people are very concerned about, especially when you hear about the experiences that others have had with the other medications that are available to treat chorea, so it is a concern in systematic changes like that that would restrict access.

01:04:18
Moderator

[Participant 2]?

01:04:21
Participant 2 (registered as a caregiver)

I'm going to wear both hats right now. Okay, so I have not had this problem. But I know others who have had the problem with, really working the system to get the prior authorization. I was able to get everything taken care of. Of course, I also have some knowledge of the process. I feel bad for the people who don't have a caregiver who is in the system or knows how to work the healthcare system. It can be daunting.

Okay.... Pharmacist's hat now. One of the things that the new pricing with CMS with the Medicare Part D, this is a bit of a concern, because there is no longer a donut hole, which is awesome. We don't have people stopping medication because they can't afford it. There's a new payment plan where people can extend their copayment throughout the year. That's great. The problem is, now the insurance company, after one month of treatment, my husband is now in catastrophic coverage, meaning, after the first month, the insurance company is picking up the entire bill $81,000 over the course of a year.

They don't want to lose money. What's going to happen? What are they going to do? Negative reimbursement for pharmacies is something that happens, I would hope not with this drug, but because it's limited to specialty pharmacies, there's some concern there that insurance is not going to lose money. So other people are going to pick up the burden.

01:06:29
Moderator

Yeah, and **[Participant 5]**?

01:06:33

Participant 5 (registered as a caregiver)

I had spoken earlier about my private insurance, but to kind of piggyback on what **[Participant 6]** and **[Participant 2]** said, we had a fight earlier this year with my husband's Medicare Part D on whether or not they would cover it. And it turned out, eventually we found out that he had something that's called the extra care coverage due to the fact that most people that... Huntington's is not one of your cheap, glamorous diseases. It's one of the expensive, hardest diseases, I mean, they don't call it the devil's disease for nothing.

And as **[Participant 6]** said, I was scrambling, and it just so happened... because my husband last year went from Austedo to Austedo XR, and in that switch we still had some regular Austedo left, and there was a concern there in that, what's going to happen if insurance doesn't cover this and we run out of what we just so happened that I held on to just in case something like this happens. And this is the biggest fear that I have as a caregiver, is being able to have the access and the tools. And, like **[Participant 6]** said, everything that we need to take care of our loved one, because again, I am taking care of my husband. He can't handle any of this stuff. It's me, I am the brain for our family, and knowing that we will be able to have access to this medication, takes a huge load of stress off of me and other caregivers like me.

01:08:17

Moderator

Awesome thanks, **[Participant 5]**.

I want to talk a little bit about how Austedo compares to alternative medications that are available for both TD and for chorea. But I want to start with chorea first.

How do the benefits of other medications differ from Austedo, if at all, for chorea?

Yeah, **[Participant 5]**?

01:08:51

Participant 5 (registered as a caregiver)

I know you're going to get sick of hearing me talk, but...

01:08:54

Moderator

No, not at all, of course.

01:08:56

Participant 5 (registered as a caregiver)

Well, it's like **[Participant 6]** and I were just talking about earlier. Tetrabenazine was a nightmare, and Ingrezza is so expensive that if I'm having trouble getting Austedo, there's no way I'm going to be able to get Ingrezza. And nothing against Ingrezza or anyone that takes them or tetrabenazine. It was just a nightmare for us.

And we know Austedo works. **[REDACTED]** has been on it since day one of it becoming available. And if it's not broken, let's not try to fix it with something we know won't fix it. Because, like I said, my husband was not my husband the entire time he was on tetrabenazine. I got my husband back when he got on Austedo, just from the lack of side effects.

01:09:49
Moderator

So, **[Participant 5]** when you said that you lost your husband, or he wasn't the same person. Tell me a little bit more about that. What do you mean?

01:09:56
Participant 5 (registered as a caregiver)

Well, I mean my husband's personality changed. He was a zombie. All he wanted to do was sleep. He didn't care to spend time with us meaning myself, my at the time 9-year-old stepdaughter, and one-and-a-half-year-old son and my husband, to know my husband, his kids, Sorry, his kids are his world.

They're, I mean, the sun rises and sets with my husband's family, and with tetrabenazine he just didn't care. He didn't care about work. He didn't care about anything except sleeping, and he just, that was not my husband. My husband is the nicest guy you will ever meet. I've seen my husband chase down a homeless veteran to give him the last $60 he had before Huntington's really took over, and with tetrabenazine that was not my husband. My husband didn't care about anything, and I want to be able to keep my husband and Austedo helped me get my husband back.

01:11:03
Moderator

Thanks, **[Participant 5**]. I appreciate that. **[Participant 6]**, go ahead.

01:11:08
Participant 6 (registered as a caregiver)

I think another thing is the dizziness and the nausea that, that tetrabenazine caused. My husband, I mean living, being dizzy all the time is just not fair. He didn't deal and then also, with the weight loss he would get nauseous. He would throw up. We were trying to keep weight on with him getting sick. So, I think it was just, it was real, there was a lot of side effects that really impacted our family, especially the nausea and throwing up... the kids, we never knew why. Did he have the flu? Was he sick? Should we take him in? And then we finally realized it was tetrabenazine.

01:11:42
Moderator

Now I want to give... **[Participant 3]**, I'm going to have to cut you off, which I apologize for. But I want to give the people talking about TD a moment to talk. For you all, what have been some of the benefits, or maybe drawbacks, of Austedo compared to other drugs that can treat TD?

[Participant 1]? Go ahead.

01:12:07
Participant 1 (registered as a caregiver)

Like I said, I am unaware of anything my mother has ever taken, be it besides Austedo, so I don't have an answer to that question.

01:12:15

Moderator

Totally fine.

01:12:18

Participant 7 (registered as a representative of a patient advocacy organization)

I think I could just chime in with a little bit of information about the differences between Ingrezza and Austedo. I mean the, the primary difference, and really you can only tell if your doctor uses say Austedo and it doesn't have the desired result, they'll generally go try Ingrezza or the other way around. And Ingrezza is a more targeted, is more targeted specifically to the dopamine situation that occurs in TD, so Austedo has a broader effect, which could be good or could be bad, because we still don't know enough about tardive dyskinesia. It doesn't just work on the dopamine path, pathway. And that's what these medicines, VMAT2 inhibitors, do is they basically keep the dopamine from hitting those hypersensitive dopamine receptors.

If I could get scientific for a second, so the main difference being that Austedo doesn't hit is a broader effect. It has an effect on other systems. Ingrezza is completely targeted, all of its metabolites, to reducing the effect on dopamine, so that could be the underlying reason why one works better on a patient than another. The other thing that I'll chime in on is that some people, there have been a lot of antioxidants over the years that have been tested on people with tardive dyskinesia. Vitamin E is something that anecdotally, and also through reports, has been taken with some success and grapeseed extract. So, you have these antioxidants like ginkgo biloba.

But, however, generally we believe that that's only a situation that can help a patient that has gotten tardive dyskinesia from the older first-generation antipsychotics, which created a lot of antioxidant or oxidation in the brain. So, if you got TD from a newer antioxidant, or I'm sorry, a newer atypical antipsychotic, which is generally more the case these days, then you're not going to be able to use some of these, or have as good effect with an antioxidant, because it causes almost no antioxidation in the brain whatsoever that we can tell from studies. So, a little in the weeds there.

But unfortunately, doctors don't delve into enough like, okay, how did this tardive dyskinesia start? Did you take a first-generation, or did you take a second-generation antipsychotic? Because there could be some real differences in what is effective for you, and that would just cause a lot, that would just require a lot of research and education in the clinician community to be able to delve into. And it's probably beyond what we can really hope for at this point. But there's a lot of confusion about, okay, because we've had, for instance, ginkgo biloba is sort of like a second line thing that's recommended by the neurologists. However, on working with patients over the 5 or 6 years that we've been in existence, we've seen almost nobody have good results with ginkgo biloba. So there's probably an underlying reason for that. And it comes back to the fact that, hey, did you take first-generation antipsychotic? Or did you take a second-generation antipsychotic? So again, very much in the weeds. But that's what we know about alternatives.

01:16:36

Moderator

That's great. Thanks, **[Participant 7]**.

[Participant 9], I see you had your hand up, but **[Participant 8]** had her hand up first and after her I need to move on to another topic. So, I apologize, **[Participant 9]**. **[Participant 8]**, let me get a chance to hear from you.

01:16:47

Participant 8 (registered as a patient)

Yes, I was going to agree with **[Participant 1]**. In my particular case, Austedo worked, so there was no need for my doctor to try to give me another drug. It did take time. It took about three months before I saw real results, and I think in many cases patients expect instant results. In my case, it didn't happen. I just kept going, plugging along, saying, okay, I'm going to take this as long as I need to. And it worked, and like **[Participant 1]** or someone said, if it ain't broke, don't fix it.

01:17:33

Moderator

Now.... Thanks, **[Participant 8]**. And for those of you who are talking about TD, I want to talk a little bit about what life would be like if you didn't have Austedo or similar medications. So, if someone had tardive dyskinesia and Austedo or other medications were not available to you, what would it be like? What would life be like for you or for them?

[Participant 1]? I think I saw your hand up.

01:18:02

Participant 1 (registered as a caregiver)

Yeah, I mean me and my mom wouldn't talk. We wouldn't be able to. Right now, I, she has a cousin that calls me because her cousin can't actually communicate with Mom. She's older herself, and it's just really hard. But, I mean sometimes right now, it's a real test of patience like I said, but there would be no communication between me and my mom at all. She would not be able to form words. She would not be able to enunciate, and she also probably would be being fed by a tube by this time, because her swallowing is so bad and she wouldn't be able to.

01:18:46

Moderator

How about others? What would TD be like if not for Austedo?

[Participant 8]? Yeah.

01:18:55

Participant 8 (registered as a patient)

Well for me, I would probably be more depressed than what I was before I started taking my medication. My life would just become unbearable. And my symptom would have probably gotten so much more, much worse. Now I am going out. I share my story with my family members. I'm not embarrassed to say that I've had mental health problems.

I just find myself talking to people openly. And not being afraid to say, hey I have this problem. This is how I took care of it. And if you have this problem as well, like I have a family member who has TD, and she is having such a difficult time trying to get Austedo. But I am willing to share my experience with people, and I'm unafraid of what the stigma is. I just feel like my confidence level, while I was confident before, my confidence, I really appreciate the fact that I can speak properly, that I can interact with people, that I can socialize.

I just have a new lease on life. And what was sweet then is even sweeter now. So, all that is to say is that my life has greatly improved because of Austedo.

01:20:32
Moderator

And question for **[Participant 8]** or others… talking about TD, what aspects of TD, if any, do Austedo and other medications are they not able to address? What is it not able to help you with?

01:20:49
Participant 8 (registered as a patient)

Well, my problem was that my symptoms were in my face, my speech was slurred, my lips were hanging to the, my left side of my mouth was hanging to the side. I still every now and then find myself moving my hands a little bit, but not as much as I used to. I used to shuffle my feet quite a bit, but the real Achilles heel for me was my facial expression. So, it's addressed just about everything perfectly as far as I'm concerned. I'm a totally different person than before I started taking Austedo, and I cannot say enough good things about how my quality of life has changed.

01:21:42
Moderator

Now, I want to give a chance for those who want to talk about chorea. If not for Austedo or other drugs that treat chorea, what would life be like and how does it meet people's needs?

01:22:01
Participant 5 (registered as a caregiver)

Is this opened up to Huntington's now, too?

01:22:04
Moderator

Yes.

01:22:04
Participant 5 (registered as a caregiver)

Okay. If you don't mind me going ahead since I've kind of got the floor.

01:22:10
Moderator

Go right ahead.

01:22:12
Participant 5 (registered as a caregiver)

For us, without Austedo, there are things that we, as a family, wouldn't be able to do. This past weekend, we went to an HD event that we would not have been able to do if **[REDACTED]**'s chorea had not been controlled. There's no way we would have been able to travel and do the things as a family, and I mean entire family. Extended family came to this event.

And you know those are things that are very important to myself and my husband is being able to still do things while he still can do things, because with Huntington's end-stage Huntington's typically is trapped inside their own body, not able to talk, not able to feed themselves, take care of themselves in any way, shape, form, or fashion.

And so, we try to have experiences now before my husband gets to that point, and having the chorea control and the Austedo has opened that world for us to be able to do that, even though he's considered late-mid stage Huntington's, he's still able to do things. And we really attribute Austedo to us being able to travel for him to have quality time playing video games with our kids, for us to be able to sit down and hold hands and watch a movie. And we really feel that the Austedo has changed our lives, and we want to be able to continue to take it for as long as we can.

01:23:53

Moderator

Thanks, **[Participant 5]**. **[Participant 4]**?

01:23:56

Participant 4 (registered as a caregiver)

Thank you, **[Participant 5]**, for the lead in. We use the terminology "life-changing" way too much. But that's exactly what Austedo is for people with Huntington's. It's life-changing, because everything that **[Participant 5]** just talked about, one thing feeds to the other. If her husband can have quality time with his children, look at what that does for those children in years and months and days, or whatever to come. If he can eat better, he can then have better functionality of everything in his body.

Everything feeds to one another. Because my brother had less falls, he had less doctor's visits. Do you know how huge that is for somebody with Huntington's to sit in a doctor's office for a length of time every day? So less doctor's visits, less weight loss, everything feeds to one another. So, bottom line is, it's life-changing. It's simply life-changing, because there are so many positive results from taking this medication.

01:25:01

Moderator

Thanks, **[Participant 4].** On another angle here I want to ask about for chorea is what aspects of chorea, if any are Austedo or other drugs unable to address for you? What doesn't it help you with, with chorea?

No? Okay, totally fine. All right. **[Participant 3]**. Sorry. Go ahead.

01:25:37

Participant 3 (registered as a representative of a patient advocacy organization)

I would just say that Austedo and other drugs like it, but Austedo in particular, based on its formulary, controls chorea very well in people living with Huntington's disease. It's the psychiatric and cognitive function symptoms of the disease that aren't managed by it. But as far as the motoric symptoms go, the excessive movements, the choreiform movements are oftentimes well controlled by Austedo, or like diseases. It's some of the volitional movement issues that are not as impacted by Austedo. But that extra movement, the chorea movements that get in the way of daily function are often well controlled by it.

01:26:18
Moderator

[Participant 2]?

01:26:20
Participant 2 (registered as a caregiver)

Yeah, so something that it doesn't, and it's not really chorea, but it goes hand-in-hand with Huntington's disease is the stiffness, the dystonia, they may have less movements, but the patient is still very stiff.

01:26:50
Moderator

Thank you, **[Participant 2]**.

I have one final question. But, **[SECONDARY MODERATOR]**, I just want to make sure there are no follow-up questions that you had before I start closing this out.

01:27:01
[SECONDARY MODERATOR]

Thanks for checking, none at this time.

01:27:03
Moderator

Okay, thanks, **[SECONDARY MODERATOR]**.

So those are all the questions today. And I just want to say, I so appreciate you all, your comments. **[Participant 5]** and **[Participant 8]**, especially, some of your vulnerability, and talking about what you've gone through. I so appreciate it. I learned so much today, even though I had read up so much before today. I learned so much more today. And I appreciate that.

Before we start a journey out, I want to pause for a moment and ask you all if there's anything else that wasn't covered in our discussion that you think is important for CMS to know, or things that you want to share with CMS while they're listening.

[Participant 4]? I think **[Participant 4]** got the...

01:27:49
Participant 4 (registered as a caregiver)

I have just one thing to add and that is, that once people have gone down the road of Huntington's disease in their family, in their parents, their grandparents, the financial situation is oftentimes devastated. And so, if, indeed, we get to a point where there has to be out-of-pocket costs of any amount, copayments, or anything for people to continue to take Austedo or any medications like that, that's simply not feasible for these patients to do.

The generational decrease in income is very much an issue in families with Huntington's. And I'm guessing with other neurological diseases as well. But that is something that we need to be cognizant of is that any copayments or any out-of-pocket costs can be very devastating or completely inhibiting for people to be able to take this medication.

01:28:45
Moderator

And I just want to be mindful of time. So, **[Participant 7]**, maybe just like, 30 seconds. And then **[Participant 2]**, 30 seconds.

01:28:52
Participant 7 (registered as a representative of a patient advocacy organization)

Okay, just a quick pair of data to keep in mind about tardive dyskinesia. It's estimated that about 750,000 people in the U.S. have it. But only 15% of people who have it have actually been diagnosed. So, with this new kind of world of Zoom and health through telehealth, it only becomes more difficult to go ahead and diagnose unless you're inpatient in front of a clinician. One other thing is of those 15% that have been diagnosed, it's estimated that only 5% have received treatment. So, the amount of people moving forward as clinicians become better aware that tardive dyskinesia is very much a massive problem that can be well treated. I mean, it's only going to become more in demand if you consider that only 5% of that number has been treated.

01:30:00
Moderator

Thanks, **[Participant 7]**. And then **[Participant 2]**, just real quickly.

01:30:04
Participant 2 (registered as a caregiver)

Yeah, I just wanted to reiterate, and the video said, CMS is transparent and that is appreciated. The PBM, the pharmacy benefit managers, are not transparent. Their negative reimbursement schedule, there's pharmacies that are going out of business, so that affects everybody in the system without having, know Austedo is a specialty drug. It has to be gotten through specialty pharmacy. But I'm worried about the people in **[REDACTED]** those are almost desert conditions and desert for pharmacies sometimes as well.

Closing Remarks

01:30:54
Moderator

So, thank you all again for your time and participation in today's group. We appreciate the time you gave us today, and talking about your experiences. Your experiences were really important and valuable and will help CMS with their negotiations for these drugs. CMS staff have been listening to the roundtable, and will be able to bring your perspective back to their teams. **[CMS STAFF]**, did you want to add anything from CMS' perspective?

01:31:21
CMS Staff

I'll just briefly say thank you on behalf of CMS and on behalf of my colleagues who are on this call and were listening. We greatly appreciate you for sharing your experiences, experiences of your loved ones and those in the communities that you serve. You've given us a lot to think about, and we are deeply grateful for you participating today. Thank you.

01:31:45
Moderator

Awesome. Thank you, **[CMS STAFF]**, and if you have any questions after today's session, you can submit them to this email address that's shown here. It's IRARebateAndNegotiation@cms.hhs.gov. Again, IRARebateAndNegotiation@cms.hhs.gov.

And that's all I have. Thank you so much, everyone, and I hope you all have a wonderful rest of your day. Thank you so much.

01:32:15
Participant 4 (registered as a caregiver)

Thank you.

01:32:16
Participant 8 (registered as a patient)

Thank you.

===== END OF TRANSCRIPT =====

For a list of the drugs selected for the second cycle of the Medicare Drug Price Negotiation Program, click on the following link: https://www.cms.gov/files/document/factsheet-medicare-negotiation-selected-drug-list-ipay-2027.pdf

For more information on the Medicare Drug Price Negotiation Program, please click on the following link: https://www.cms.gov/priorities/medicare-prescription-drug-affordability/overview/medicare-drug-price-negotiation-program

Appendix

Participant 1: Registered as a caregiver for an individual who has experience with the selected drug, the condition(s) treated by the selected drug, or other treatment(s) similar to the selected drug for those condition(s)

Declared Conflicts of Interest	
No	Receipt of financial payments (e.g., gifts, funding, research support, honoraria, travel, or other expenses) from a company with direct/indirect interest in the Negotiation Program, in excess of $10,000 by you, your spouse, or an immediate family member
No	Direct assistance preparing your remarks from someone who is NOT a family member, caregiver, friend, or your healthcare provider
No	You, your spouse, or an immediate family member is employed by or holds equity interest (stock or ownership interest) in excess of $10,000 in a company or related association with direct or indirect interest in the Negotiation Program
No	Any other personal or professional relationship or interaction with a company or related association with direct or indirect interest in the Negotiation Program that may be considered a financial conflict of interest

Participant 2: Registered as a caregiver for an individual who has experience with the selected drug, the condition(s) treated by the selected drug, or other treatment(s) similar to the selected drug for those condition(s)

Declared Conflicts of Interest	
No	Receipt of financial payments (e.g., gifts, funding, research support, honoraria, travel, or other expenses) from a company with direct/indirect interest in the Negotiation Program, in excess of $10,000 by you, your spouse, or an immediate family member
No	Direct assistance preparing your remarks from someone who is NOT a family member, caregiver, friend, or your healthcare provider
No	You, your spouse, or an immediate family member is employed by or holds equity interest (stock or ownership interest) in excess of $10,000 in a company or related association with direct or indirect interest in the Negotiation Program
No	Any other personal or professional relationship or interaction with a company or related association with direct or indirect interest in the Negotiation Program that may be considered a financial conflict of interest

Participant 3: Registered as a representative of a patient advocacy organization

Declared Conflicts of Interest	
Yes	Receipt of financial payments (e.g., gifts, funding, research support, honoraria, travel, or other expenses) from a company with direct/indirect interest in the Negotiation Program, in excess of $10,000 by you, your spouse, or an immediate family member
No	Direct assistance preparing your remarks from someone who is NOT a family member, caregiver, friend, or your healthcare provider
No	You, your spouse, or an immediate family member is employed by or holds equity interest (stock or ownership interest) in excess of $10,000 in a company or related association with direct or indirect interest in the Negotiation Program
No	Any other personal or professional relationship or interaction with a company or related association with direct or indirect interest in the Negotiation Program that may be considered a financial conflict of interest

Participant 4: Registered as a caregiver for an individual who has experience with the selected drug, the condition(s) treated by the selected drug, or other treatment(s) similar to the selected drug for those condition(s)

Declared Conflicts of Interest	
No	Receipt of financial payments (e.g., gifts, funding, research support, honoraria, travel, or other expenses) from a company with direct/indirect interest in the Negotiation Program, in excess of $10,000 by you, your spouse, or an immediate family member
No	Direct assistance preparing your remarks from someone who is NOT a family member, caregiver, friend, or your healthcare provider
No	You, your spouse, or an immediate family member is employed by or holds equity interest (stock or ownership interest) in excess of $10,000 in a company or related association with direct or indirect interest in the Negotiation Program
No	Any other personal or professional relationship or interaction with a company or related association with direct or indirect interest in the Negotiation Program that may be considered a financial conflict of interest

Participant 5: Registered as a caregiver for an individual who has experience with the selected drug, the condition(s) treated by the selected drug, or other treatment(s) similar to the selected drug for those condition(s).

Declared Conflicts of Interest	
No	Receipt of financial payments (e.g., gifts, funding, research support, honoraria, travel, or other expenses) from a company with direct/indirect interest in the Negotiation Program, in excess of $10,000 by you, your spouse, or an immediate family member
No	Direct assistance preparing your remarks from someone who is NOT a family member, caregiver, friend, or your healthcare provider
No	You, your spouse, or an immediate family member is employed by or holds equity interest (stock or ownership interest) in excess of $10,000 in a company or related association with direct or indirect interest in the Negotiation Program
No	Any other personal or professional relationship or interaction with a company or related association with direct or indirect interest in the Negotiation Program that may be considered a financial conflict of interest

Participant 6: Registered as a caregiver for an individual who has experience with the selected drug, the condition(s) treated by the selected drug, or other treatment(s) similar to the selected drug for those condition(s); a representative of a patient advocacy organization

Declared Conflicts of Interest	
No	Receipt of financial payments (e.g., gifts, funding, research support, honoraria, travel, or other expenses) from a company with direct/indirect interest in the Negotiation Program, in excess of $10,000 by you, your spouse, or an immediate family member
No	Direct assistance preparing your remarks from someone who is NOT a family member, caregiver, friend, or your healthcare provider
Yes	You, your spouse, or an immediate family member is employed by or holds equity interest (stock or ownership interest) in excess of $10,000 in a company or related association with direct or indirect interest in the Negotiation Program
No	Any other personal or professional relationship or interaction with a company or related association with direct or indirect interest in the Negotiation Program that may be considered a financial conflict of interest

Participant 7: Registered as a representative of a patient advocacy organization

Declared Conflicts of Interest	
Yes	Receipt of financial payments (e.g., gifts, funding, research support, honoraria, travel, or other expenses) from a company with direct/indirect interest in the Negotiation Program, in excess of $10,000 by you, your spouse, or an immediate family member
No	Direct assistance preparing your remarks from someone who is NOT a family member, caregiver, friend, or your healthcare provider
No	You, your spouse, or an immediate family member is employed by or holds equity interest (stock or ownership interest) in excess of $10,000 in a company or related association with direct or indirect interest in the Negotiation Program
No	Any other personal or professional relationship or interaction with a company or related association with direct or indirect interest in the Negotiation Program that may be considered a financial conflict of interest

Participant 8: Registered as a patient who has experience with the selected drug; a patient who has experience with the condition(s) treated by the selected drug

Declared Conflicts of Interest	
No	Receipt of financial payments (e.g., gifts, funding, research support, honoraria, travel, or other expenses) from a company with direct/indirect interest in the Negotiation Program, in excess of $10,000 by you, your spouse, or an immediate family member
No	Direct assistance preparing your remarks from someone who is NOT a family member, caregiver, friend, or your healthcare provider
No	You, your spouse, or an immediate family member is employed by or holds equity interest (stock or ownership interest) in excess of $10,000 in a company or related association with direct or indirect interest in the Negotiation Program
No	Any other personal or professional relationship or interaction with a company or related association with direct or indirect interest in the Negotiation Program that may be considered a financial conflict of interest

Participant 9: Registered as a representative of a patient advocacy organization

Declared Conflicts of Interest	
No	Receipt of financial payments (e.g., gifts, funding, research support, honoraria, travel, or other expenses) from a company with direct/indirect interest in the Negotiation Program, in excess of $10,000 by you, your spouse, or an immediate family member
No	Direct assistance preparing your remarks from someone who is NOT a family member, caregiver, friend, or your healthcare provider
No	You, your spouse, or an immediate family member is employed by or holds equity interest (stock or ownership interest) in excess of $10,000 in a company or related association with direct or indirect interest in the Negotiation Program
Yes	Any other personal or professional relationship or interaction with a company or related association with direct or indirect interest in the Negotiation Program that may be considered a financial conflict of interest