Transcript: Town Hall Meeting Session 1, April 30, 2025, Medicare Drug Price Negotiation Program Public Engagement Events

This transcript was lightly edited for readability.

Introductory Remarks

Moderator, PhD, RTI International

Good morning. Thank you for joining us today for the CMS Medicare Drug Price Negotiation Town Hall. I am **[MODERATOR]** from RTI International. I'll be the facilitator for the Town Hall today.

If you would like to listen to this Town Hall in Spanish, please follow the directions for accessing the Spanish line shown on the screen. We also will have sign language interpretation throughout the Town Hall.

To start us off, I will share a brief introductory video from Steph Carlton, CMS Chief of Staff and Deputy Administrator.

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.

Moderator, PhD, RTI International

There are several CMS representatives joining the Town Hall today to hear from today's speakers. I would like to hand it over to **[CMS STAFF]** from CMS.

00:03:52

Welcome and Overview

CMS Staff

Good morning. I'm **[CMS STAFF]**, part of the leadership team at CMS. I'm greeting you today on behalf of the dozens of CMS staff and leaders who are listening to the livestream, as well as the panelists and guests who are taking their time to participate in this Town Hall. We appreciate those individuals joining us today to share their input, as well as all of you who are watching on the livestream. Back to you, **[MODERATOR]**.

00:04:19

Moderator, PhD, RTI International

The Town Hall today has a morning and an afternoon session. For this morning session, we will hear from speakers on eight drugs in the order you see listed here. The goal of the Town Hall is to provide an opportunity for clinicians, researchers, and other interested parties to share input focused on the clinical considerations related to the drug selected for the second cycle of negotiating.

CMS will use the information shared during the Town Hall meeting to better understand clinicians' experiences prescribing and/or managing treatment with the selected drugs or therapeutic alternatives, and clinicians' consideration that drive treatment choice between the selected drugs and therapeutic alternatives.

In addition to this Town Hall meeting, CMS hosted 15 private patient-focused roundtable events, one for each selected drug, each of which was open to patients, patient advocate organizations, and caregivers. CMS will use the information shared during the roundtable events to better understand patients' experiences with the conditions and disease treated by the selected drugs and patients' experiences with the selected drugs themselves.

The information shared during both the Town Hall meeting and the roundtable events will also inform CMS' identification of therapeutic alternatives, key outcomes, and adjustment of the starting point to develop the initial offer in negotiating with manufacturers of selected drugs. The speakers at today's Town Hall meeting include clinicians, researchers, patients, caregivers, and patient advocates. The number of speakers for each selected drug varies based on how many speakers registered to speak for each of those drugs.

This meeting is being livestreamed. Participation is voluntary, and speakers acknowledged and agreed by participating in the meeting, that any information provided, including individually identifiable health information and personally identifiable information, will be made public during the meeting through a livestream broadcast. Clinicians should be mindful of their obligations under HIPAA [Health Insurance Portability and Accountability Act] and other privacy laws.

CMS intends to make a redacted version of the transcript for the meeting available in May. Speakers were asked to disclose any potential conflicts of interest, or COI, with the drug they are speaking about. As I introduce each speaker, I will note if there is any disclosed potential COI, which you will see on the slide. To accommodate as many speakers as possible, each speaker will be limited to four minutes for their remarks. There will be time for a brief follow-up after each set of speakers. I appreciate everyone sticking to these time limits so that we are able to hear from everyone.

The first [three] speakers will share remarks about Breo Ellipta, which is commonly used to treat asthma and chronic obstructive pulmonary disease, or COPD. The first speaker is **[Speaker 1]**. This speaker has indicated that there is no conflict of interest.

00:07:33

Speaker Remarks for Breo Ellipta

Speaker 1 (registered as a representative of a patient advocacy organization)

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Good morning. My name is **[Speaker 1]**. I'm the **[REDACTED]** of Allergy and Asthma Network, a 40-year-old patient advocacy organization that advances its mission through education, outreach, advocacy, and research. We're guided by our medical advisors to keep our work evidence-based, and we rely on patient insights to inform our work and ensure it's patient-centered.

From a treatment perspective, the goals are clear for patients with asthma to allow the patient to breathe more easily, to reduce exacerbations, and to allow people with asthma to maintain an active and fulfilling life. The right treatment supports the patient to be fully functioning and a contributing citizen to society and reduces health care utilization. The right medication for a particular patient can achieve these goals.

Breo Ellipta, as we know, is a combination inhaler. It contains an inhaler corticosteroid and a long-acting beta agonist. Here are some factors that are important in the shared decision-making process for Breo Ellipta as a treatment option for a patient with asthma.

Number one, affordability. Clinicians work with patients to prescribe what they believe will be the medication to manage asthma when used as prescribed. Ultimately treatment choices hinge on access and affordability, efficacy, and ease of use. Presently, some people can't afford Breo Ellipta. Clinicians are then challenged to work with patients in these situations to find an alternative that may or may not work as well as Breo.

Number two, therapeutic alternatives are not equal or equivalent. The asthma guidelines offer different treatment options for patients based on their asthma severity. This does not mean that these drugs are the same or equivalent compared to other drugs. Switching from one to another could impact efficacy and adherence, especially if different devices are involved.

Number three, therapeutic alternatives may not work as well for patients. Using less optimal treatment options may lead to lower efficacy. This is an important factor when considering a switch from Breo to a medication that may be therapeutically equivalent but does not control their asthma as well. We currently know of patients who would be better on Breo and have told us that, but other options are what they have to work with because they can't afford Breo Ellipta.

Number four, once-daily dosing versus twice-daily dosing. Breo is once-daily dosing. For people with multiple comorbid conditions and complicated medication schedules, the simplicity of a once-a-day daily dosing really helps with ease of use and adherence.

Number five, combination device versus two separate devices. Combination inhalers increase adherence compared to using two separate inhalers. We've heard from caregivers of some patients with dementia that might find two inhalers challenging, as would patients with multiple comorbid conditions, who already have a complicated medication schedule, and having to purchase two inhalers may increase the cost.

Six, DPI [dry powder inhaler] versus MDI [metered dose inhaler]. Breo Ellipta is a dry powder inhaler, or DPI. Some alternative treatment options use a DPI, whereas others use an MDI, or a metered dose inhaler. DPI inhalers are a completely different technique than an MDI. This is an important consideration for many patients since the change requires the patients to be trained on the new device, and research shows that 50 to 80 percent of people who use inhalers use them incorrectly. This is an important consideration when choosing to switch people to different types of inhalers. In practice, we know some people do better on DPIs than MDIs and vice versa, so it's important to acknowledge that.

In conclusion, from a clinician perspective, we know that asthma is a heterogenous disease with diverse phenotypes, severities, and treatment responsiveness. What medication works well for one patient may not be the best choice for another. That's why options are important. And then switching to alternatives might seem reasonable in concept, but it may not work for a particular patient in practice for a variety of factors I've outlined above.

Thank you for your time and for allowing us to have a voice in this negotiation process.

00:11:48

Moderator, PhD, RTI International

Thank you. The second speaker is **[Speaker 2]**. This speaker disclosed a potential conflict of interest as shown on this slide.

00:12:08

Speaker 2 (registered as a representative of a patient advocacy organization)

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Thank you so much for having me. My name is **[Speaker 2]**, and I serve **[REDACTED]** of the Alliance for Aging Research. The Alliance is the leading nonprofit organization dedicated to changing the narrative to achieve healthy aging and equitable access to care.

I looked at the CMS fact sheet, and it looks like in the last year there were 634,000 beneficiaries that were on Breo at an average of about $186 a month. So, this drug, we believe, was chosen for volume rather than cost. I think when these talks originally came up, the idea was to get to the highest priced, and this is a moderately, certainly, if not low-priced, medication for older adults.

And combination therapies are really important for older adults who often have multiple chronic conditions and take multiple medications. So, anything that can actually combine medications can be incredibly valuable for them, and, as has already been stated, Breo is very, very helpful for chronic bronchitis or emphysema. It's a once-daily dose. It can help open airways and reduce flareups. And, because it's combination, it combines precise dosages appropriate for the condition, and it provides ease of use and compliance with the daily regimen.

I think sometimes combination therapies are considered to just be about convenience. But, in our view and our experience with our patient advocate network and the many, many hundreds of groups that we work with, it's really more about adherence. You want people to have ease of use.

And there was a real-world evidence study that was published in the January 2025 issue of the journal *Pulmonary Therapy*, that looked at records of more than 15,500 older adults, and found that their asthma, with or without COPD, is not often well-controlled on rescue inhalers alone. So, access to medications like Breo are really important to asthma control in older adults.

The Alliance is really concerned about increases in utilization management practices by PBMs [pharmacy benefit managers] and Part D plans since passage of the IRA [Inflation Reduction Act]. While it's very well-meaning, the legislation has great things in it like the cap on Part D plans and smoothing, there were simultaneously changes to the Part D structure and then stricter limits on premium increases. So, the unintended consequences of this is that Part D plans and PBMs that negotiate for them have little recourse to make up their revenue other than increasing utilization management, and we've seen rapid increases in UM [utilization management] since the IRA was passed. We commissioned a report that came out June 24 by Manatt Health, and we found that basically the incentives to cut losses by excluding selected drugs were done through formulary changes, tightening utilization management with prior authorization and step therapy, and all those types of things delay or limit access to drugs selected for negotiation.

Now we've met with CMS in the Part D group a number of times. It's within CMS' authority to crack down on this process, and we really encourage the agency to take the necessary steps to do so. We know that you care about your beneficiaries, and hope that you'll do more on this. It also limits where people might be able to get their medications. There was a January 2025 survey by the National Pharmacists Association, and that showed that about 61% of independent pharmacies

are strongly considering not stocking one or more of the drugs with prices negotiated under Medicare Part D, while an additional 33% have already decided not to stock one or more of the drugs because of cash flow problems and payment delays. So, we hope CMS actually deals with this issue.

And then the last thing I wanted to say, in addition, certainly, to thank you, is we hope, we really appreciated the change in the dialog this year. So, thank you for taking many of our recommendations, the Q&A dialog in the stakeholder meetings was terrific.

00:17:02

Moderator, PhD, RTI International

Please take a moment to wrap up your remarks.

00:17:06

Speaker 2 (registered as a representative of a patient advocacy organization)

Absolutely. But we really hope that CMS staff will come out of listening mode next year and interact directly with patients and family caregivers to allow for question-and-answer dialog back and forth between the agency and the people that are affected by these conditions and by the drugs that you're selecting.

Basically, I mean, all of us are patients or family caregivers, or will be someday. These were originally going to be modeled after the FDA [Food and Drug Administration] patient-focused drug development meetings. That's what they do. You all can do it. We would love to work with you on it, but better job this year. Thank you so much for the work you're doing and thank you for the opportunity.

00:17:53

Moderator, PhD, RTI International

Thank you. The third speaker is **[Speaker 3]**. This speaker has indicated that there is no conflict of interest.

00:18:12

Speaker 3 (registered as a healthcare provider)

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Hello. Hi, my name is **[Speaker 3]**. I am a family physician in **[REDACTED]**, Illinois, and I wanted to thank you all for the opportunity to speak on this important matter. As a family physician, I treat patients from cradle to grave across all age demographics and racial and ethnic backgrounds.

Asthma and COPD are two of the most common respiratory illnesses that I have to manage in all of those populations, and combination inhalers like Breo Ellipta are critical to successful treatment of them. As many asthma and COPD exacerbations can be not just frustrating and annoying, but potentially life-threatening. And so, the ability to give people controller inhalers like Breo Ellipta can help them lead not just better lives, but safer lives to prevent them from having to see me for exacerbations, or even worse, having to go to the emergency room or into the hospital.

While Breo Ellipta is similar to other combination inhalers in many respects, some of its unique qualities are particularly helpful and make it a good choice for many of my patients over some of the alternatives. Specifically, being able to dose it once daily is very helpful, particularly for younger and older patients who need this medication because some inhalers that need to be dosed multiple times a day can be physically or mentally taxing, both on the patient or potentially their caregivers who have to administer their medications to these patients. Similarly, the fact that this is a powder inhaler can be helpful for patients who haven't been able to tolerate the non-powder form of controller inhalers.

And, from a purely practical aspect as a physician, particularly amongst the Medicare population and those who have Medicare Advantage or Medicare Part D plans that control which medications are on their formularies, inhalers are some of the most challenging medications to prescribe. As physicians, we are often in the position of not knowing which controller inhaler is covered. And, even when we do find one that is covered year-to-year, they're often changed for patients, often solely on the basis of the cost to the insurance plans. And this makes it extremely challenging to get patients into a good and consistent routine with their inhalers, because once we found one that works well for them, like Breo Ellipta, we may be switching to another one the next year, and then switching again the year after that.

Having a more favorable price for something like Breo Ellipta, which is already one of our more preferred inhalers, would be so critical to getting patients on this for the long term and having a more stable and consistent treatment plan for them for the years that they are managing this condition.

I've been incredibly frustrated to have to negotiate back and forth with pharmacies for many patients who take these medications trying to figure out which inhalers are going to be covered by their formulary. So, anything that CMS can do to make that a little less burdensome on patients and providers like me will be really critical in helping patients manage these complex conditions.

So, just wanted to thank you for your time on this important matter. And again, emphasize the importance of bringing these medications like Breo Ellipta to a more affordable place for all of us.

Thank you.

00:21:58

Moderator, PhD, RTI International

Thank you. I have one follow-up question for you all. Could any of you please address or expand on what other information or evidence do you think CMS should consider in evaluating Breo Ellipta? Please use the raise hand function, and we will select the first respondent. We have just a minute for your brief response.

00:22:34

Speaker 3 (registered as a healthcare provider)

Would you like me to start speaking?

00:22:39

Moderator, PhD, RTI International

Yes, please. Go ahead.

00:22:41

Speaker 3 (registered as a healthcare provider)

Okay. So, I think the evidence that we should really be considering is how Breo Ellipta compares to other inhalers in terms of its ability to control this medication in real-world settings. I'm not aware if that evidence is already existing, but, as was previously noted by myself and other speakers, the once-daily dosing for this inhaler is unique among some of the other controller inhalers. It makes it a lot more patient-friendly and is one of the reasons why it's accessible to so many patients who need to use this.

So, if such a real-world study doesn't already exist, which hopefully it does, I would strongly encourage looking into something of that nature, because that would emphasize why medications like this that are more patient-friendly in their dosing are so critical to be included in negotiation, and are superior in that aspect to their competitors that are not as effective in patients' real-world settings.

00:23:47

Speaker Remarks for Trelegy Ellipta

Moderator, PhD, RTI International

Okay, thank you. We will move on now to Trelegy Ellipta with three speakers. Trelegy Ellipta is commonly used to treat asthma and chronic obstructive pulmonary disease, or COPD. The first speaker is **[Speaker 1]**. This speaker has indicated that there is no conflict of interest.

00:24:16

Speaker 1 (registered as an academic researcher)

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Hello. Can you hear me?

00:24:20

Moderator, PhD, RTI International

Yes, we can.

00:24:21

Speaker 1 (registered as an academic researcher)

Perfect. Thank you so much for the opportunity. I'm **[Speaker 1]**, a **[REDACTED]** at Public Citizen's Research Group, a nonprofit consumer advocacy organization. We have no financial conflicts of interest. According to the prescribing information, Trelegy Ellipta is indicated for a relatively broad patient population as maintenance treatment for patients with either COPD or asthma in patients aged 18 years and older. This is of concern because the efficacy of this combination drug has not been adequately established for such a broad patient population.

Moreover, components of this triple inhaler are associated with serious adverse events. These include increased risks of Candida albicans infections, glaucoma and cataracts, and worsening of existing infections. Treatment with Trelegy Ellipta can also lead to clinically significant cardiovascular effects in some patients, including increased blood pressure and cardiac arrhythmia.

An important caveat is also that many of the studies supporting the benefits of Trelegy Ellipta were funded by its manufacturer. It's also important to know that although one of the touted advantages of a single combined inhaler is its convenience, one study, funded by the manufacturer, found that only 26% of asthma patients persisted with treatment with the single inhaler after one year compared with 15% of those that used multiple inhaler triple therapy.

With that in mind, I briefly want to summarize Public Citizen's position on Trelegy Ellipta for COPD and asthma, which we have also submitted as a written comment. For COPD, there are no adequate data from randomized, controlled trials comparing the effectiveness and safety of different ICS/LABA/LAMA [inhaled corticosteroids/long-acting beta-agonists/long-acting muscarinic antagonists] combinations with each other. However, approval was based on several studies that demonstrated Trelegy Ellipta overall led to significant improvements in lung function, significantly greater health-related quality of life, as well as significantly lower annual rates of moderate or severe exacerbations than patients who received only two of the components.

Two retrospective observational studies based on U.S. insurance claims data showed that patients who started treatment with Trelegy Ellipta had lower rates of moderate or severe exacerbations than did patients who started treatment with another triple inhaler, a twice-daily combination.

Public Citizen recommends that triple therapy, such as with Trelegy Ellipta, should only be considered as a maintenance treatment for patients for whom COPD exacerbations are no longer adequately controlled with dual therapy, such as with an ICS and a LABA. In such patients, treatment with a triple ingredient inhaler can be beneficial for lung function and quality of life.

For asthma, however, the therapeutic benefit of Trelegy Ellipta compared to both dual inhaler ingredient inhalers, as well as other triple ingredient treatments has not been established. Moreover, the long-term safety of this triple inhaler for this indication has not been adequately demonstrated. For example, several studies show that although treatment with Trelegy Ellipta led to some improvement in asthma control, not all of these improvements were significant or clinically meaningful. Importantly, treatment with Trelegy Ellipta did not consistently lead to significant differences in asthma-related quality of life or asthma control. Public Citizen, therefore, recommends that patients should not consider taking Trelegy Ellipta for the maintenance treatment of asthma until its benefit and long-term safety have been established.

Thank you for your time.

00:28:08

Moderator, PhD, RTI International

Thank you. The second speaker is **[Speaker 2]**. This speaker has indicated that there is no conflict of interest.

00:28:35

Speaker 2 (registered as a representative of a patient advocacy organization)

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Thank you. My name is **[Speaker 2]**. I'm a respiratory therapist by training and currently serve as **[REDACTED]** for the COPD Foundation, a patient advocacy organization. I appreciate the opportunity to advocate on behalf of our community and contribute to the conversation today.

As former Surgeon General C. Everett Koop once said, "Drugs don't work in patients that don't take them." This is certainly no less true in lung problems like chronic obstructive pulmonary disease than it is for other conditions, but COPD drugs often have characteristics that serve as unique barriers to prescription adherence. It's therefore critical that as many of these barriers as possible be addressed for as many people as possible.

COPD places a substantial burden on health care systems around the world, including, of course, ours here in America. Approximately 16 million people have been diagnosed with COPD, and statistical surveys suggest that another 16 million deal with COPD symptoms impacting their lives but are undiagnosed.

Financial impact of COPD is significant with total COPD-attributable costs topping 80 billion dollars, 33 billion of which are directly related to COPD care like hospitalizations and prescriptions. A key driver of these costs is unexpected health care utilization. People with COPD are prone to symptom flare-ups, known as exacerbations. Acute exacerbations of COPD require care above and beyond one's baseline therapy regimen, including things ranging from additional prescription meds like antibiotics and corticosteroids, to visiting an urgent care clinic, all the way up to a full hospital admission.

These exacerbations are also a leading driver of lung function decline, which in turn leads to an increasing need for urgent and emergent care, as well as the increased likelihood of disability and similar loss of function. However, one of the best ways to avoid these exacerbations is adherence to an optimized COPD therapy regimen. These regimens include things like exercise and proper nutrition, but at their foundation sits inhaled medications like bronchodilators. These medications are so critical that one of my good friends and a patient herself in the COPD advocacy world once told me, "Medications allow me to do the exercise that really lets me live my life." Truly the foundation of care.

However, taking these meds is not like taking pills, capsules, patches, or other common medications. These are complex systems that include both therapeutic molecules and delivery devices. They often require specific inhalation techniques to optimize dosing, and this technique can be wildly different from device to device. So, if someone is prescribed multiple medications, they may also be required to master multiple techniques, not to mention dosing schedules.

One specific example from my own practice is someone who was prescribed three medications, similar to what is in Trelegy Ellipta, but where these medications were delivered using two different inhalers. A combination med with a bronchodilator and a corticosteroid was taken twice daily, using a single strong inhalation both times to aerosolize the dry powder that contained the medicine. The other medication was taken only once a day but using two puffs and two softer inhalations as it came out as more of a mist. It took several months of coaching, reteaching, reminder cards, and other direct training in order for this patient to be able to use their medications properly.

Unfortunately, people having access to respiratory therapists like myself is not the norm, and many other clinicians these days, including the pharmacists and primary care providers who provide the majority of COPD care in America, are not able to provide that level and depth of instruction and ongoing reassessment. That means many people have not been properly trained to use their inhaler devices, which in turn means they may believe they are fully adherent to their regimen but are actually not.

Considering that studies show that between half and three-quarters of all people prescribed inhalers do not use them correctly, and even in best scenarios, technique competence tends to decline over time. This is a significant, yet hidden, barrier to optimal care. And as previously discussed, simply prescribing simplified regimens does not always provide access. That is often driven by cost, which can obviously create a significant barrier for many folks. Trelegy Ellipta represents a simplified barrier with three effective medications delivered in one device with one technique with once-a-day dosing. Reducing the cost of this medication can potentially improve access and improve the quality of life for millions of folks living with COPD, as well as reducing overall costs across the American health care system.

So once again, thank you for being able to participate in the conversation and advocate on behalf of our COPD community.

00:32:49

Moderator, PhD, RTI International

Thank you. The third speaker is **[Speaker 3]**. This speaker disclosed a potential conflict of interest, as shown on the slide.

00:33:03

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

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Hello. My name is **[Speaker 3]**, and I have been an advocate and caregiver for multiple people living with asthma and COPD for the past 25 years. I am here today to speak about the potential impact of the CMS decision regarding Trelegy. It is a novel treatment that has no therapeutic alternatives. It is a fixed dose, triple combination that's delivered in a single inhaler, requiring only once-daily dosing. This is vital for my loved ones and so many others who live with the conditions. This treatment helps to control symptoms like breathlessness, cough, and wheeze.

And, CMS' decision could negatively impact millions currently living with controlled COPD, asthma, and more, and potentially result in unnecessary inhaler switching and exacerbations. All inhalers are not interchangeable. Studies show switching an inhaler often takes more than 30 minutes to address the concerns and to effectively retrain on inhaler technique.

I certainly understand the dire need to reduce the $464 billion prescription drug spend in our country. I also understand the need for PBM reform and reducing out-of-pocket expense, especially for those living on fixed income like our seniors and CMS, including my own parents and loved ones living with multiple chronic conditions. The monthly expense of three or more medications at more than $100 each out-of-pocket is simply not sustainable.

I would like to close by asking each of us to imagine trying to drink a thick milkshake through a coffee stirrer. This is exactly what breathing feels like when a person is living with COPD or uncontrolled asthma and experiencing symptoms. Each of us will take more than 20,000 breaths today. Most of us take this for granted. But the 50 million Americans living with asthma and COPD do not. We need effective treatment options like Trelegy to meet the unmet needs in our community. I respectfully ask CMS to maintain coverage of Trelegy for those who are currently controlled on this life-saving medication. My parents' lives depend on it. My loved ones' lives depend on it. Thank you for the opportunity to be here and share today.

00:35:38

Moderator, PhD, RTI International

Thank you. And thank you all for sharing your experiences and perspectives about Trelegy Ellipta.

I have one follow-up question for you all. Could any of you please talk more about the main benefits of taking Trelegy Ellipta based on your experience or understanding?

Again, we will select the first respondent to raise their hand, and we have just a minute for your brief response. **[Speaker 3]**, go ahead.

00:36:04

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

Thank you. For both my parents and a dear friend who I'm the caregiver for with COPD, Trelegy is the only option that has minimized the out-of-pocket cost for them, but also has provided that triple dose, once-daily inhaler. And again, the convenience of that, the effectiveness of the product has been able to allow them to have more active lives and spend time with their children, grandchildren, and great-grandchildren.

00:36:40

Moderator, PhD, RTI International

Thank you. We do have time for one more response if another speaker would like to respond. **[Speaker 2]**, go ahead. You have about 20 seconds.

00:37:00

Speaker 2 (registered as a representative of a patient advocacy organization)

I would just say that, again, it's one of the best solutions for somebody who has COPD exacerbations that cannot be well-controlled, and again, keeping them engaged with family and their community and the workforce as appropriate.

00:37:20

Speaker Remarks for Austedo; Austedo XR

Moderator, PhD, RTI International

All right. Thank you very much for your time. We will move on now to Austedo and Austedo XR with four speakers. Austedo is commonly used to treat chorea in Huntington's disease and tardive dyskinesia. The first speaker is **[Speaker 1]**. This speaker has indicated that there is no conflict of interest.

00:37:53

Speaker 1 (registered as a representative of a patient advocacy organization)

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Hello, I'm **[Speaker 1]**. I am a neurologist in **[REDACTED]**, Tennessee, part of a large group of neurologists that are subspecialists that take care of people with conditions like Parkinson's disease, Huntington's disease, and tardive dyskinesia. Tardive dyskinesia is a condition that can result when people need to take medications for serious mental health disorders. Some of those medications can induce tardive dyskinesia. What the patient with tardive dyskinesia experiences is involuntary hyperkinetic movements. They are these uncontrollable writhing type movements that really interfere with all sorts of activities and come with significant stigma. You can imagine just trying to be around friends or family or at a restaurant or in a theater, having just uncontrolled, involuntary movements, and unfortunately, that can occur following treatment with some medications that are needed for serious mental health conditions.

So, when thinking about people with tardive dyskinesia, what we hear is that they experience pain, discomfort, stigma, and the more movements they have, the more and more a recluse they become in their home. A recent survey found that two-thirds of people with tardive dyskinesia have impairment of activities of daily living. If they're employed, up to 30% report that they miss work because of their dyskinesia. And two-thirds of them report that they have trouble doing work even when at work.

But here's something that's really important. People with tardive dyskinesia often stop the medications that they need for their mental health disorder, feeling like the medications are causing their involuntary movements. So, that's why having treatments like Austedo and Austedo XR are so critically important, because with these medications we can now allow people to work, participate in their community, interact with their friends and their families, and most importantly, they can continue to take the medications if they're needed for their serious mental health conditions.

Prior to this class of medicine, the VMAT2 [vesicular monoamine transporter 2] inhibitors being approved, people with tardive dyskinesia didn't have an FDA-approved option for treatment, but now we do have this class of medicines that are FDA-approved for the treatment of tardive dyskinesia. Austedo can also be used in the treatment of people with Huntington's disease, and Huntington's disease is a condition that also causes those hyperkinetic involuntary movements.

So, when people are treated for their tardive dyskinesia, here are the key take-home messages. One, their involuntary movements are significantly reduced. Two, it reduces the stigma and the impairment that comes along with that. Three, they're able to take a medication that's on-label for the condition that they have, and then most importantly, they're often able to remain on the medications that they need for their mental health conditions.

So, VMAT2 inhibitors, Austedo and this class of medications are a true clinical advancement, and they allow people with tardive dyskinesia to live a more full life with their work, family, and friends. Thank you.

00:41:45

Moderator, PhD, RTI International

And thank you. The second speaker is **[Speaker 2]**. This speaker has indicated that there is no conflict of interest.

00:42:14

Speaker 2 (registered as a healthcare provider)

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Thank you for giving me the opportunity to speak at this CMS Town Hall. I am a physician involved in multiple leadership positions within the Huntington's disease community, but today I'm here representing myself. I have no conflicts to disclose, and I'm not being compensated for my time, nor was any organization involved in developing my remarks. Of note, I served as the **[REDACTED]** for the Austedo studies in Huntington's disease from an academic perspective.

My main goal in speaking related to Austedo is to advocate for all users of the medication, especially those with Huntington's disease. I'm a neurologist specializing in movement disorders. And I see patients with about half my time focusing specifically on Huntington's disease. I see patients across all stages of the disease and have followed well over 1,000 patients with this devastating disease over the 20 plus years of my practice.

For those not familiar, HD [Huntington's disease] is a genetic brain disorder that causes issues with movements, mood, behavior, and cognition. It progresses over time, and ultimately patients require 24-hour care for their safety, prior to their ultimate demise. There are about 40,000 people in the U.S. living with the disease and over 200,000 people at-risk by virtue of having a parent or a sibling with Huntington's disease. There's no cure, but there are some treatments that can make a notable difference in patients' lives.

Chorea is the hallmark of Huntington's disease, characterized by abnormal, involuntary, flowing type movement, and there's an old myth that chorea is simply a cosmetic issue. I can assure you that it can be a safety issue, resulting in bruises, scrapes, falls, and other injuries. Early in the disease, chorea can limit function related to dressing, eating, walking, and other important tasks. And importantly, chorea can also be an isolating issue, since caregivers may be reluctant to take their loved ones into situations that require the effort of explanation or result in damage, such as to someone else's home or in a restaurant.

Later in the disease, chorea amplitude can increase, resulting in skin issues, falls, and other injuries. Also, in advanced disease, caregivers are at risk for injury when trying to care for the person with Huntington's disease, such as trying to get them dressed or bathing, if chorea is not adequately controlled. Theoretically, chorea can be controlled with any of the three VMAT2 inhibitor medications that are currently approved by the FDA. However, tetrabenazine is dosed three times daily, and has notable side effects in a substantial portion of patients.

Austedo has the advantage of smaller-sized tablets, which permits better individualized titration, to maximize benefit and reduce the chance of side effects. And the other aspect of Austedo, which is rather unique in the Huntington's disease treatment field, is that weight loss is common in Huntington's disease and can be difficult to treat, but in the double-blind, placebo-controlled study that ultimately led to Austedo's approval, we found that there was weight gain when Austedo was used.

I recognize we're here to talk about Austedo use in general, and most of the use is related to tardive dyskinesia, but I wanted to make sure that Huntington's disease is considered in the decisions related to these negotiations. It would be devastating for the population to lose access to this medication. My job as a clinician would be more difficult to have limited treatment options that are often off-label or unproven therapies or drugs that have more side effects.

While the population of Huntington's disease includes a small number of people, they matter. The availability of safe, effective treatment for this population is important, not only for those living with Huntington's disease, but also for those at risk, who need to have some hope that we can address some of the symptoms until a way to prevent or slow the disease can be found.

Access to Austedo for this rare, fatal condition is critical to the quality of life of the families and the functioning and safety of our patients. Thank you for your time and attention.

00:46:15

Moderator, PhD, RTI International

And thank you for taking time. The third speaker is **[Speaker 3]**. This speaker has indicated that there is no conflict of interest.

00:46:31

Speaker 3 (registered as a healthcare provider)

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Thank you very much. My name is **[Speaker 3]**. I'm a clinical professor of psychiatry here in **[REDACTED]** California, and I've been practicing for about 35 years. My area of expertise is chronic mental illness. You've heard from the two previous speakers, Austedo has two indications for Huntington's chorea and tardive dyskinesia.

In my field, I utilize Austedo for tardive dyskinesia. Now over the last 50 years, many, many medications have come forward to treat these chronic mental illnesses, and we're moving forward with much better outcomes. When I started practice about 35 years ago, we had a few limited agents. And these agents would benefit patients, but unfortunately, they would have serious side effects, the primary one being tardive dyskinesia. As you've heard, it's a neuromuscular side effect that can be extremely troublesome. You see movements of the face, lips, eyes. The lips will be smacking. The tongue may be coming in and out. And you may have whole body movements.

The biggest challenge we have with chronic mental illness is compliance to treatment, so you can imagine if an individual who's getting better begins to develop a neuromuscular side effect, like tardive dyskinesia, it may immediately lead to not wanting to take that medication.

As we've continued to move forward with the development of other agents to treat schizophrenia, bipolar disorder, major depression, we have found that it's extremely important if people are going to stay well, we need to keep them on their medications. Now that we have two agents that are approved for tardive dyskinesia that have been thoroughly and rigorously tested and approved by the Food and Drug Administration, we can now give hope to patients. So, when I see a patient and I discuss the various treatment options, I can now tell them, if an adverse effect emerges, we can treat that. Unfortunately, tardive dyskinesia is quite common as an adverse effect when we're using antipsychotic medications based on the mechanism of how those agents work. Good efficacy, but we may have this side effect of movement disorder.

So, at the get-go, one of the biggest reasons individuals may be ambivalent about treatment, I can address that even before an adverse effect has occurred, letting them know we've got an agent that is well-tested and should be able to treat, if that adverse effect emerges. And again, I keep reiterating the point, the biggest challenge we have in illnesses like schizophrenia, bipolar disorder, even depression, is that once an individual is doing well, many don't want to stay on the treatment because of an adverse effect.

So, with Austedo, my patients can get better, that movement disorder can be decreased and hopefully eliminated, and it will keep them on their medications so they can return to work. They won't have the stigma of this movement disorder when they go out in public. They can connect with families, be engaged socially. We now have many studies that have come forward, showing the quality of life of individuals whose tardive dyskinesia is treated, they can now reenter society for an illness that they were being treated for that has severe stigma. So, it is critical, I've seen it in my practice with hundreds of patients.

00:50:50

Moderator, PhD, RTI International

Please take a moment to wrap up your remarks.

00:50:53

Speaker 3 (registered as a healthcare provider)

So, it is critical that we allow and have access to a medication like Austedo, as we only have two agents currently FDA-approved. The others are all off-label, that don't show the same efficacy and clearly have more side effects. Thank you.

00:51:13

Moderator, PhD, RTI International

Okay, thank you for your insights. The fourth speaker is **[Speaker 4]**. This speaker has indicated there is no conflict of interest.

00:51:24

Speaker 4 (registered as a representative of a patient advocacy organization)

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Thank you for your time today, and hearing from our important communities. My name is **[Speaker 4]**, and I'm **[REDACTED]** of the Huntington's Disease Youth Organization, a nonprofit supporting, educating, and empowering young people, 35 and under, impacted by HD. After learning more about a CMS renegotiation and what that means, there was some immediate concerns that came to mind. If this renegotiation impacted the out-of-pocket costs for HD families, we would have a different opinion on this process. However, it does not.

As mentioned previously, Huntington's disease is a terminal neurodegenerative genetic rare disease with no cure and no treatment to help slow or stop the progression of this disease. Families are turned upside down as they navigate the effects of living with a disease that mimics similar symptoms to ALS [amyotrophic lateral sclerosis], Alzheimer's, and Parkinson's all wrapped up into one. Caregivers watch their loved ones literally wither away over a decade or more who have choreatic symptoms.

For people with HD, this means direct impacts to quality of life while the disease is progressing, including swallowing challenges, maintaining jobs, independence, and more. I don't know if you've ever seen someone choking while you were helplessly watching and can't do anything. Or have your father who you've admired using a sippy cup in his thirties because he spills all the time. If you've held a loved one's hand as they uncontrollably shake in the hospital, only to feel an unfillable void

when the hand is gone. They're everyday things that the HD community faces because of chorea, and Austedo can help and does.

Putting any rare disease with little to no treatments or cure up for renegotiation has the potential of being extremely damaging to the future research and therapies. If you look at the list, HD is the only one that fits the description of a terminal rare disease. So, why is it included?

The only therapies we have in the U.S. to treat HD symptoms, only do that, and they mainly treat the psychiatric changes and challenges. As Dr. **[Speaker 2]** mentioned, the benefits of Austedo for families directly betters their lives of these HD patients in ways that the limited other therapies approved for chorea simply do not benefit.

I have great fears that this renegotiation will halt the innovation of repurposing therapies from other diseases for the HD community. Because of the similarities and symptoms with other diseases, there's an emphasis in learning what therapies could be beneficial to help in the short-term, while very complicated long-term studies are focusing on modification approaches. This is what happened with Austedo after previously being approved for tardive dyskinesia. Because of that, we are now all grouped together for two very different diseases who happen to benefit from the same drug.

We have big questions that need to be answered. Why would a drug company focus on other repurposing trials for HD if they know this could cause them future renegotiation because of this precedent set for Austedo? How does this decision impact current and future trials looking at disease-modifying approaches for HD? Can renegotiations be placed on drugs for certain diseases, while sparing rare diseases? Austedo was innovative with its repurposing of this drug for HD. Their efforts have helped directly better the lives of people who are experiencing the devastating effects of HD. Please note this decision carries the weight of not only current therapies, but future therapies for this important HD community. Thank you for your time.

00:55:05

Moderator, PhD, RTI International

And thank you all for sharing your experiences and perspective about Austedo and Austedo XR. I have one follow-up question for you all. Could you please address or expand on what considerations drive treatment choice among Austedo and treatment alternatives? We have just a minute for your brief response, and we'll call on the first person to raise their hand. So go ahead, **[Speaker 2]**.

00:55:29

Speaker 2 (registered as a healthcare provider)

Great. So, I have this discussion with patients and their insurers all the time. I think that there are multiple considerations. I think that one of the most important is that tetrabenazine has many side effects. Over 20% of people have depression or anxiety, akathisia, which is an inner sense of restlessness that causes additional movement and agitation.

So, I tend not to use that medication anymore. It's also three-times daily dosing. It can be quite difficult. I choose Austedo, one, because it's the once-a-day in terms of XR, and the non-XR form has much more ability to titrate to really get to where we need to be and get people the medication dosing that they need while minimizing the side effect profile.

00:56:30

Speaker Remarks for Calquence

Moderator, PhD, RTI International

Okay. Thank you very much. We will now move on to Calquence with four speakers. Calquence is commonly used to treat chronic lymphocytic leukemia, small lymphocytic lymphoma, and mantle cell lymphoma.

The first speaker is **[Speaker 1]**. This speaker disclosed a potential conflict of interest, as shown on the slide. [Note: An error was made in the moderator's introduction. This speaker had indicated that there was no conflict of interest.]

00:56:57

Speaker 1 (registered as a healthcare provider)

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Hello, everybody. Thank you, **[MODERATOR]**, and thank you to CMS for hosting the Town Hall and facilitating this forum to collect public comments about the program. My name is **[Speaker 1]**. I am a hematologist oncologist in **[REDACTED]**, Washington, at the **[REDACTED]** and **[REDACTED]**. I treat patients with hematologic malignancies, also known as blood cancers. I would also like to share that I serve as **[REDACTED]** for the American Society of Hematology, also known as ASH. ASH represents more than 18,000 clinicians and scientists committed to studying and treating blood and blood-related diseases, including hematologic malignancy that are treated by a few of the drugs included on this year's list, including Calquence. ASH's mission is to foster high-quality care, transformative research, and innovative education to improve the lives of patients with blood and bone marrow disorders.

As part of this mission, the committee on practice focuses on health policies that improve access to care and support hematologist's ability to treat their patients. As **[REDACTED]**, I also review the society's comments responding to regulatory policies, including support for this program and for the federal government's ability to negotiate drug prices directly with the manufacturer.

Health care costs are a persistent barrier to care, especially when it comes to medications. The Part D program helps to manage some of the burden of these high drug costs by providing beneficiaries with coverage for both generic and brand name medications. But only 80% of patients eligible for

Part D coverage have enrolled in a prescription drug coverage plan. Additionally, more than one in four adults have reported difficulty affording their prescription drugs, even with Part D coverage. Further, about one in 10 adults say they have cut pills in half or skipped doses of medication in the last year due to cost.

While cost is a challenge across health care, it is particularly acute in hematology. Many of the oral treatment options available to patients are incredibly high-cost and present challenges for patients to pursue and continue their treatment plan. A colleague recently shared a story about an 85-year-old woman with a new diagnosis of chronic lymphocytic leukemia, that then progressed and required therapy. My colleague started this patient on acalabrutinib, which is the generic for Calquence, in which a standard dose is 100 milligrams by mouth twice daily. However, after a month or two on this therapy plan, the patient shared that her out-of-pocket cost for the drug was too high for her to afford. In order to save money, the patient self-managed her medication and reduced her dose to 100 milligrams every other day, which is a quarter of the recommended dose.

Adherence to acalabrutinib is important to slow and/or stop the progression of chronic lymphocytic leukemia. If a patient does not adhere to the treatment plan, CLL [chronic lymphocytic leukemia] is likely to progress sooner, and may be more resistant to other medications that are used in the future. Treatment with acalabrutinib is often initiated in CLL to improve symptoms, such as large, bulky lymph nodes or abnormal blood counts. Acalabrutinib has better efficacy and tolerability than other medications in the same class of BTK [Bruton's tyrosine kinase] inhibitors including a medication previously on this list, known as ibrutinib. Unfortunately, stories like the one I shared are common in hematology practice, but I hope the information provided today is helpful to understand the importance of access to acalabrutinib Calquence as part of this program. Thank you again for your time, and I'll pass it back to **[MODERATOR]**.

01:01:07

Moderator, PhD, RTI International

And thank you for your insights. The second speaker is **[Speaker 2]**. This speaker disclosed a potential conflict of interest, as shown on the slide.

01:01:17

Speaker 2 (registered as a healthcare provider)

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Thank you. I'm Dr. **[Speaker 2]**, and I'm the **[REDACTED]** in the Division of Lymphoma at **[REDACTED]** and an associate professor of medicine at **[REDACTED]**. In addition to my role as a physician caring for patients with CLL and lymphomas, over the past 15 years my work as a clinical investigator has been dedicated to advancing our understanding and treatment of these diseases, particularly through my role as a key investigator in the development of BTK inhibitors and venetoclax. CLL is an incurable malignancy, and our primary goals in treating the disease are to prolong life and improve the quality of life for our patients.

These objectives guide every decision we make in selecting the most appropriate treatment strategies. When considering treatment options, it's crucial to evaluate the efficacy and safety of available drugs. For instance, while ibrutinib was a groundbreaking first-generation BTK inhibitor, it has been surpassed by second-generation BTK inhibitors, which offer improved outcomes and reduced side effects. This is why we are cautious about recommending ibrutinib when better alternatives are available.

Acalabrutinib Calquence, as well as zanabrutinib Brukinsa, are two such second-generation BTK inhibitors that we often recommend for our patients. While they are similar in terms of efficacy, patient-specific factors can influence our choice of treatment. For example, I've treated patients who required frontline BTK inhibitor therapy but suffered migraines. In this case, Calquence was not ideal due to its increased risk of headaches. So, we opted for Brukinsa, which has lower incidence of this side effect. Conversely, I might choose Calquence over Brukinsa in patients with significant hypertension given that Brukinsa may have a somewhat higher risk of that complication.

The key takeaway here is the importance of having easy access to both medications. Moreover, there are instances when patients exhibit intolerance to a BTK inhibitor early in the course of their treatment, necessitating a switch to another BTK inhibitor. In such cases, timely access to a different BTK inhibitor is crucial to prevent a dangerous flare of CLL disease that can occur when patients early in their course are taken off therapy even for just a few days.

Having flexibility with which drug to choose also allows us to tailor treatment to individual patient needs and preferences. Some patients prefer time-limited therapies with oral medications. The recent addition of Calquence plus venetoclax to the NCCN [National Comprehensive Cancer Network] guidelines provides a valuable option for these patients, offering the potential for prolonged progression-free survival with less total time on treatment compared to the strategy of continuous BTK inhibitor use. Such time-limited treatment approaches also allow the potential for intermittent therapy through retreatment strategies, and I'm currently leading a global trial exploring one such retreatment approach, which I'll be presenting the first data on at upcoming international hematology conferences in Europe.

In a somewhat similar but distinct disease known as mantle cell lymphoma, Calquence has until recently been more commonly used in the relapse setting. However, there is now a new approval by the FDA for its use in frontline setting in combination with bendamustine and rituximab. Having access to this regimen would be highly beneficial for patients with MCL, providing another valuable option in the fight against this difficult-to-treat disease.

In sum, our commitment to improving the lives of patients with CLL and lymphomas drives us to continually seek the most effective and personalized treatment strategies. By ensuring access to a range of therapeutic options, we can better meet the diverse needs of our patients and work towards a future where these diseases are even more manageable and less burdensome for patients and their caregivers. Thank you for giving me this opportunity to speak at the Town Hall meeting and thank you for your continued support in advancing cancer care.

01:05:03

Moderator, PhD, RTI International

Thank you for your insights. The third speaker is **[Speaker 3]**. This speaker disclosed a potential conflict of interest, as shown on the slide.

01:05:21

Speaker 3 (registered as a healthcare provider)

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Can you see me? Just want to make sure.

01:05:24

Moderator, PhD, RTI International

Yes, we can.

01:05:25

Speaker 3 (registered as a healthcare provider)

Okay, thank you. My name is Dr. **[Speaker 3]**. I appreciate the time to speak with you today. I am the **[REDACTED]** of the chronic lymphocytic leukemia program and professor of medicine at the **[REDACTED]** at **[REDACTED]** in New York. I also have worked as a CLL and leukemia physician for over 20 years, and similar to Dr. **[Speaker 2]**'s, my research focus has been dedicated not only to taking care of patients with this disease, but in developing better treatments for this incurable malignancy, therapies that continue to prolong the survival and quality of life for patients with this incurable condition.

I cannot stress enough that currently, this disease is chronic. Given this, it's important to have many options of therapy for these patients, so they not only can have alternative therapies when their disease actually progresses, but also that if they have a side effect to the therapy, they can receive another therapy that may have a different side effect profile. Currently, as we discussed, we have a class of therapy called BTK inhibitors, which have transformed the outcome for these patients.

The first-generation therapy, a drug called ibrutinib, was a game-changer for these folks. However, it's been associated with several side effects that sometimes make this agent intolerable for many of our patients. The newer generation BTK inhibitors, acalibrutinib or Calquence as we're

discussing, and zanabrutinib or Brukinsa are more targeted therapies that have less side effects and are really the newer standard of care of BTK inhibitors for patients with CLL and SLL [small lymphocytic lymphoma]. Even these two agents have a unique side effect profile that sometimes we, as physicians, may choose one agent over the other, depending upon their comorbidities or other medications that they're taking for their other conditions.

These agents can be given as a chronic continuous daily therapy, much as chronic medications given for high blood pressure or diabetes. And this often works well for our older patients who want a simpler treatment for their disease, and don't require a lot of medical monitoring or management. But these BTK inhibitors can also be given in combination with other therapies, such as a BCL2 [B-cell lymphoma 2] inhibitor named venetoclax, which is also an oral therapy, or with an intravenous monoclonal antibody therapy, such as rituximab or obinutuzumab.

In this fashion, these combination therapies with BTK inhibitors give a shorter duration to enable patients a time-off of treatment, so they get a treatment-free interval, and then when their disease progresses or returns, they can be retreated again. These combination therapies or time-limited therapies are often used for younger patients who will likely need more than one therapy during their long journey with CLL. And this also helps decrease resistance to these therapies, giving them a long treatment-free period, and so they can use these therapies again when they're needed.

Through the research that myself and Dr. **[Speaker 2]** and other people have conducted and presented at national and international meetings, we have shown that we've improved both the quality and survival of patients with CLL. These therapies hope to achieve a functional cure, allowing the patients to hopefully live a normal lifespan with their chronic disease until we find a curative therapy for all. Future research is really focused on some of these combination strategies to improve the response and durability of response and hopefully decrease toxicity. So, we're looking for newer therapies as we unlock the biology of the disease.

Currently, the patients really haven't had a problem accessing these therapies and that's really, really important to have them be able to access all of these therapies from their payers and not have to have a problem. Many of our patients may have an out-of-pocket cap, which eliminates the concerns for increasing financial toxicity, and really lets us focus on being able to have access to all of these oncology drugs, including the many options in this class.

In summary, it's our continued commitment to treat our patients with the best possible current options of therapy that we consider now the standard of care for this disease. And, given our patients and their diverse needs and medical problems, which require multiple options to tailor to their current conditions, it's really imperative to provide access to all the BTK inhibitors given the different potential side effects and need for switching within the class, as well as have access to venetoclax and other therapies that are in combination with these BTK inhibitors available for all our CLL patients. I thank you for giving me the attention and opportunity to speak this morning and for your ongoing support for patients with cancer, particularly incurable diseases such as this one.

01:09:25

Moderator, PhD, RTI International

Thank you for your time. The fourth speaker is **[Speaker 4]**. This speaker disclosed a potential conflict of interest, as shown on the slide.

01:09:56

Speaker 4 (registered as a healthcare provider)

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Hi, my name is **[Speaker 4]**. I am a nurse practitioner at the **[REDACTED]** in **[REDACTED]** and associate clinical professor. For the past 30 years, I've been taking care of patients with blood cancers, particularly CLL and mantle cell lymphoma, and both of these blood cancers are unfortunately incurable, but they are treatable. And, the goal of our therapy is to help people live as long as possible, feeling as well as possible.

So, with CLL in particular, many patients are diagnosed when they are otherwise very healthy, and I can tell them that with modern therapy that they can live well with their CLL using a variety of different treatment options. It was an incredible advance in CLL when ibrutinib, the first BTK inhibitor, was approved more than 10 years ago. And people taking this medicine continuously can enjoy a remission of more than seven years feeling well. Ibrutinib has already been negotiated by CMS, but unfortunately, ibrutinib has a lot of side effects. It was first-generation, and there were side effects such as irregular heart rhythms, high blood pressure, joint pain, rash, diarrhea, and approximately 40% of patients had to discontinue this very effective therapy because the side effects were so intolerable.

Acalabrutinib or Calquence and zanabrutinib Brukinsa are two newer BTK inhibitors that work as well or better than ibrutinib, with far fewer side effects, and for this reason, we no longer use ibrutinib and prefer to use acalabrutinib or zanabrutinib. And patients treated with these medications can enjoy an excellent quality of life, with good disease control.

However, these medications need to be taken every day indefinitely, and this can be a big financial burden, because medications are so costly. For many of my patients, the out-of-pocket copay is $1,000 to $2,000 a month, and most patients are seniors on a fixed income. The out-of-pocket cap in Part D is a big help to Medicare patients, because affordability is such an issue.

And then, as the other speakers mentioned, building on the effectiveness of using a BTK inhibitor, such as acalabrutinib, as a single agent, we're now combining it with another medication called venetoclax, and acalabutinib and venetoclax work much better together than either alone, and can result in a remission so deep that patients can stop treatment for a period of time. Limiting time on treatment decreases side effects, minimizes cost, and decreases the chance of resistance. So, it is really important that patients have access to acalabrutinib and venetoclax, as well as zanabrutinib,

which they may need to switch to if they're having any side effects to acalabrutinib. My patients generally favor time-limited treatment, and since the acalabrutinib and venetoclax are in the NCCN guidelines already, we've already started using that regimen with patients.

And then, lastly, I would like to mention mantle cell lymphoma, which is a challenging lymphoma to treat as it's also incurable, but it's much more aggressive than CLL, and BTK inhibitors, such as acalabrutinib, are very active and are now used in combination with chemotherapy for mantle cell lymphoma, which has improved survival. And in addition to chemo, stem cell transplant was used for younger fit patients, but adding a BTK inhibitor to chemotherapy can make a stem cell transplant unnecessary, which spares patients an intense treatment that requires hospitalization and toxic side effects. So, I am hopeful that negotiating drug prices for patients and payers will continue to provide access to all of the medications that our patients with blood cancers need to offer them the best chance of survival with the fewest side effects. Thank you very much for the opportunity to speak today.

01:14:24

Moderator, PhD, RTI International

And thank you. And thank you all for sharing your experiences and perspectives about Calquence.

I have one follow-up question for you all. Could you please address or expand on what you consider to be a meaningful improvement used to assess improvement or treatment response for the indications for Calquence? Please raise your hand to respond. We have just a minute for your brief response. **[Speaker 2]**, go ahead.

01:14:52

Speaker 2 (registered as a healthcare provider)

Sure. So, we look at certainly a lot of different endpoints when assessing response both from an efficacy and a safety perspective. In CLL, primarily we use progression-free survival as one of our main metrics, because fortunately our patients do survive for a long time with this disease. So, overall survival is often challenging to capture in the span of a clinical trial. So, in addition to PFS [progression-free survival], we look at the depth of response, particularly with the combination regimens. When patients achieve complete response, or if they achieve undetectable minimal residual disease, these are very meaningful endpoints that suggest patients may be able to stop therapy for a period of time.

And then safety, of course, is very important as well, both in the clinical trial setting, and increasingly, we have datasets from the real-world setting where we can understand better the safety profile of these drugs, which is critical for patients who are often on them for many years. Thank you.

01:15:46

Speaker Remarks for Ibrance

[No speakers provided commentary for this drug.]

01:15:46

Speaker Remarks for Linzess

Moderator, PhD, RTI International

Thank you.

No speakers are here to speak for Ibrance, so we will now move on to the next drug, Linzess. There is one speaker for Linzess. Linzess is commonly used to treat chronic idiopathic constipation and irritable bowel syndrome with constipation.

The speaker is **[Speaker 1]**. This speaker has indicated that there is no conflict of interest.

01:16:33

Speaker 1 (registered as a patient)

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Hello. Can you hear me? Hi.

01:16:35

Moderator, PhD, RTI International

Yes, we can.

01:16:37

Speaker 1 (registered as a patient)

Thank you for the opportunity to participate as a patient advocate for access to Linzess today. As **[MODERATOR]** indicated the indication, I won't repeat that, but I will explain to you my experience as a patient using Linzess, because I hope to inform the decision-making of this important committee regarding negotiation for this drug. Why and how this drug has helped me, and I consider it now an invaluable treatment in my daily routine, is that I personally suffered from this condition, and what it means in layman terms is an inability to have a bowel movement on a daily basis, which is a subject that I think all humans can relate to.

That is the normal outcome that this drug has helped me achieve. Previously, I had very slow, delayed process, and may have had five, six, sometimes seven days without having a bowel

movement, which was extremely painful, and really I think anybody could imagine what that might be like in terms of pain, discomfort, and the effect that it has on your ability to work and be productive.

I take this drug once a day. It's the only treatment that has successfully relieved my symptoms. I did try other options including lifestyle changes, dietary changes, which I continue to follow to maintain a healthy gut. And so, this drug I tried as a sample first to rule out whether or not I could tolerate it, or whether or not I might have an allergic reaction. I think that's a safe way to begin the therapy with 145 milligram dose. But I had to be moved up to double that dose to 290 to relieve my symptoms.

So, the goal is to get back to normal daily routine of having a bowel movement every day, and I'm very grateful for this drug. I think that with the aging population of American citizens, we need to be realistic and expect that many patients may have this condition as well. And CMS should, I would hope as a citizen using this drug, that you would negotiate aggressively on behalf of myself and the other patients who need to be able to afford this therapy, because we have perhaps other conditions. In my case, I also suffer from hypothyroidism and type 1 diabetes. So, lastly, I would just say that without this drug it could be devastating to the people who need it. I'm very grateful to CMS for advocating and negotiating on behalf of American citizens. So, thank you very much, and I remain available if you have any follow-up questions for me.

01:20:19

Moderator, PhD, RTI International

Thank you for sharing your experiences and perspectives about Linzess. I do have one follow-up question for you. Overall, how would you summarize the importance of Linzess for people with chronic idiopathic constipation or IBS with constipation? We have just a brief minute for your response.

01:20:36

Speaker 1 (registered as a patient)

Okay, sure. So, overall, I would summarize it by saying, being able to live a productive life, to be staying in the work world, and to feel comfortable in just living life. It's relieving pressure. It is useful in terms of just being able to function as a human being, and without it I'm not sure how long I could continue without having devastating effects and being admitted to the hospital or having real problems.

01:21:24

Moderator, PhD, RTI International

All right. Thank you. We appreciate your candor.

01:21:27

Speaker 1 (registered as a patient)

Thank you.

01:21:30

Speaker Remarks for Ofev

Moderator, PhD, RTI International

We will move on now to Ofev, with one speaker. Ofev is commonly used to treat idiopathic pulmonary fibrosis, progressive pulmonary fibrosis, and systemic sclerosis interstitial lung disease, or SSc-ILD. The speaker is **[Speaker 1]**. [Note: This speaker had indicated that there was no conflict of interest].

01:21:54

Speaker 1 (registered as a healthcare provider)

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Hi. My name is **[Speaker 1]**, and I'm a rheumatologist in **[REDACTED]**, Pennsylvania. And I do see patients with the three diseases that were commented on, and in particular, I look after patients with systemic sclerosis or scleroderma-related interstitial lung disease.

Ofev, which is also called nintedanib, is an antifibrotic drug which reduces scarring, and it works by blocking the tyrosine kinases cell pathways or signaling pathways in the lungs. What does it look like to have an idiopathic fibrosis or an interstitial lung disease? Well, that means progressive scarring inside the lungs, which manifests as worsening shortness of breath, trouble breathing, and eventually respiratory failure and death.

Who gets pulmonary fibrosis? IPF, or idiopathic pulmonary fibrosis, is seen in those patients 50 and older, and there are no major risk factors. Systemic sclerosis can happen to anybody, at any age, including children. And some subset of those individuals do get interstitial lung disease. There are actually no medications that have been found to cure these disease processes.

So, before Ofev was available, patients, particularly with IPF or under the idiopathic form, were treated basically with supportive measures, including lung dilators, oxygen for comfort, and finally, lung transplantation if they were in the select few that were able to obtain those. And for those with scleroderma-related interstitial lung disease, we manage those patients with immunosuppressive therapy, but again, generally progressive over time.

The goal of using Ofev and its sister drug, called pirfenidone, is basically to slow the progression, and there are good clinical trials which demonstrate that you could actually slow down the

progression of volume loss of the lung. So, in a normal person with IPF, you'll lose about 239 milliliters of oxygen-related capabilities in the lung. And, when you're on Ofev, that comes down to 114 milliliters per year.

It's also been shown to reduce mortality over time, so fewer people die in the same timeframe when they're taking Ofev. And finally, Ofev but not it's sister drug pirfenidone, have been shown to prevent acute exacerbations, which means acute flare-ups of severe shortness of breath that can certainly lead to being hospitalized. There are three large clinical trials that show the efficacy in IPF in particular, and two for the systemic sclerosis or scleroderma-related diseases.

When is Ofev used? In IPF, Ofev is used in mild to moderate disease, and in moderate to severe disease if there is thought to be a benefit. Who gets to use this drug or should be on this drug in systemic sclerosis? ILD [interstitial lung disease] patients who've had progression of disease, despite immunosuppressive therapy. I also give Ofev to patients who have rapidly progressive disease. So, if I see that they're losing a massive amount of lung function even in a short six months, that would be an indicator.

How do we monitor this disease and the response to this drug? We use something called pulmonary function tests to look to make sure function is actually slowing down. And we also do CT scans. Finally, what other drugs are available other than its sister drug called pirfenidone, which doesn't have quite the outcomes? We don't have any other drugs that actually work for pulmonary fibrosis. We can use the lung transplantation, and that seems to be just an end-stage measure. So, in conclusion, Ofev is considered a significant therapeutic advance, and this has changed the lives of patients with IPF and scleroderma, or sclerosis-related ILD. Patients live longer and more importantly, more comfortably, and this is definitely fulfilling an unmet need within the field of medicine. Thank you for your time.

01:26:11

Moderator, PhD, RTI International

Okay. Thank you. Thank you for sharing your experiences and perspectives about Ofev.

I have one follow-up question for you. Could you please expand on the outcomes, such as clinical, functional, or patient-reported outcomes used to assess improvement or treatment response for idiopathic pulmonary fibrosis? We have just a minute for your brief response.

01:26:37

Speaker 1 (registered as a healthcare provider)

So subjectively, meaning our experience that we see or feel, or a patient sees or feels, patients have less shortness of breath and are more comfortable and able to do their daily activities and enjoy friends and family and continue working.

From an objective standpoint, we're able to see on pulmonary function tests, which is a way for us to understand lung capacity and good oxygenation. So, we're able to see on pulmonary function tests that patients have a slower progression of disease and can live longer with this disease.

01:27:12

Speaker Remarks for Otezla

Moderator, PhD, RTI International

All right. Thank you for your time. We'll move on now to Otezla with four speakers. Otezla is commonly used to treat oral ulcers in Behçet's disease, plaque psoriasis, and psoriatic arthritis. The first speaker is **[Speaker 1]**. This speaker has indicated that there is no conflict of interest.

01:27:38

Speaker 1 (registered as a healthcare provider)

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Thank you so much for inviting me. My name is **[Speaker 1]**. I'm a rheumatologist, and I am a physician clinical assistant professor at the University of **[REDACTED]**, **[REDACTED]** School of Medicine, and I am here to speak about Otezla, generic name, apremilast, which is a medication that is FDA-approved to treat three different autoimmune diseases, all which fall under my treatment.

One of them would be Behçet's disease, which causes oral ulcers that can be so painful, it is difficult to eat; plaque psoriasis, which causes itchy, sore skin rashes; and psoriatic arthritis, which causes stiff, tender, and swollen joints that can cause permanent joint damage and disability. These three diseases affect about 10 million Americans, and Otezla has unique value in treating each of these diseases. Three top reasons that Otezla has unique value is that, one, it is an oral medication. Patients may be needle-phobic, which means that it's hard for them to give themselves an injection or to receive an injection from someone else, or they can also have what's called an injection reaction, where their skin actually swells at the injection site. In patients with psoriasis who have extensive disease, it can actually be difficult to find a clear patch of skin to inject. And for patients with psoriatic arthritis, difficulty holding a pen to inject may be due to hand permanent deformities, hand pain, and while one could argue that simply they could go to an infusion center or a doctor's office for injections, this can be a hurdle for many of our patients who have either limited mobility due to their pain and swelling in their joints or their joint deformity, or they may live in a rural or an underserved area where it's a large distance to travel.

The second reason that Otezla has a unique place in treatment of these diseases is, it has a novel mechanism of action. It is what's called a phosphodiesterase-4 inhibitor. While it calms down the immune system that is attacking the mucosa of our mouth, our skin, and our joints, the effect is more of a potent anti-inflammatory agent. It does not lower the body's ability to fight infections as much as our other medications. For this reason, it can be used in combination with other medications, particularly biologic agents that more strongly suppress the immune system.

It can also be used very well in patients that are more prone to infections, which may be patients that are more elderly or specifically cancer patients. There's actually a class of chemotherapy agents called checkpoint inhibitors that can cause, as a side effect, flares of psoriasis psoriatic arthritis in people who already have the disease or even cause new autoimmune disease, and Otezla has as a unique ability that it may not suppress the immune system as much in these patients who are prone.

And, thirdly, Otezla has fairly mild side effects, diarrhea, nausea, headache, mild upper respiratory infection, and is better tolerated than other treatments. Otezla is effective. In Behçet's disease, 53 of our patients had resolution of their oral ulcers in three months. In psoriatic arthritis, 40% of people had a 20% improvement in their disease by three months, as measured by the ACR20 [American College of Rheumatology 20]. And in psoriasis, a third of patients had 75% improvement in their skin disease by four months, as measured by the psoriasis area and severity index 75, and 20% had normal or nearly normal skin by the Static Global Physician Assessment.

What's important to note as well in a subset of psoriasis patients is that they can get psoriasis of their scalp or their genitalia, and this is at a particularly difficult location to treat. It's hard to get cream on it, and it's a significant decreased reduction in quality of life.

So, in summary improvements in psoriasis, psoriatic arthritis on Otezla is comparable for other medications, maybe not quite as much as biologic agents like Skyrizi or Taltz, but we should remember that Otezla has a unique benefit of being better accessed due to being a pill. It can be used in combination therapy with other medications, and for people with access or difficulty with access to needle injections or infusion centers. Thank you.

01:31:54

Moderator, PhD, RTI International

Thank you. The second speaker is **[Speaker 2]**. This speaker disclosed a potential conflict of interest, as shown on the slide.

01:32:05

Speaker 2 (registered as a representative of a patient advocacy organization)

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Thank you. As **[REDACTED]** of AiArthritis, the only nonprofit in the world focusing on the two dozen autoimmune and autoinflammatory arthritis diseases and also a person living with spondyloarthritis myself, I appreciate this opportunity.

Behçet's disease, psoriatic arthritis, plaque psoriasis, all autoimmune, autoinflammatory arthritis diseases are treated by Otezla. They're full-body diseases impacting joints, tendons, organs such as eyes, intestines, and skin, cause fatigue and severe cognitive impairment. Given the window of opportunity to treat these diseases to achieve the highest rate of remission is six months from onset, yet diagnosis can take years, and given the treatments themselves may take weeks or months to show efficacy, it is vital that the patient and doctor work together in a process called shared decision-making and a treat-to-target approach to consider patient complexity, as well as when they started in their disease journey, to match them to the best therapy, if there is a choice, to avoid disease progression and increase the chance for remission.

Keeping in mind both psoriatic arthritis and plaque psoriasis have other treatment options on market, often referred to as therapeutic alternatives, it must be stressed that these diseases are heterogeneous, so what works for one, is their miracle drug, will not work at all for the other.

This is for a variety of factors, including uncontrolled inflammation, causing comorbidities in which 50% of all patients will likely develop these due to uncontrolled inflammation and not finding the right treatment for them at the right time. This also raises risk for heart and lung disease, cancer, dual-, triple-AI arthritis diagnosis, as they will also age, have a higher risk for Alzheimer's and dementia. It not only adds to the cost for patients, but also to the cost for the care system. Considering age of onset and these diseases is 20 to 40 in adults and any age of children, the cost for the system should be highly focused on. Other considerations, many subgroups. There is from severity of disease, mild, mediate, to severe, with severe disease carrying the highest risk for poor outcomes and comorbidities. There's also the age of onset, elderly onset psoriatic arthritis, which is a newer diagnosis. The year the person was diagnosed, if there were no therapeutic options at that time, specifically before the 1990s.

For those living with Behçet's disease, Otezla was the first treatment ever approved to treat oral ulcers, which are primary symptom in poor quality of life. It only occurred six years ago. With a patient population with few options, this has offered them promise and hope for a better today and tomorrow. There are other options for treating ulcers, including colchicine; however, unlike Otezla, cannot address other multifaceted symptoms, like fatigue, joint pain, etc.

There are pill form benefits, as mentioned by previous speakers. In addition to the needle application complications that I will not repeat, there are also the issue of fearful to advance to injection or infusion therapy. Patients find that a pill form is not a big jump. This is particularly of high impact for adolescents and children, which Otezla is also indicated to treat psoriatic disease who have met with the right treatment, could achieve remission and lead normal healthy lives.

So, cost must include also the probability of adherence. Like many other treatments taken weekly, biweekly, or monthly, patients do not have any days or weeks, where the drug wears off. Other drugs in pill form of JAK [Janus kinase] inhibitors have an FDA black box warning finding this class of medications was associated with an excess risk for serious heart-related events, cancer, blood clots, and death. While we support the use of JAK inhibitors in patients, there's a subset of patients where this type of pill form therapies are considered dangerous. Otezla does not have these same safety profiles.

Overall, Otezla has proven through research it differentiates itself from other pill form therapies on market, only treatment for Behçet's mouth ulcers, has high efficacy in psoriatic subgroups, including scalp and private area impact. In my opinion, there is no other alternative for Otezla if it's the right treatment at the right time for the patient who needs it. Thank you very much.

01:36:07

Moderator, PhD, RTI International

Thank you for your insights. The third speaker is **[Speaker 3]**. This speaker has indicated there is no conflict of interest.

01:36:16

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

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Excellent. Thank you for the time today. My name is **[Speaker 3]**, and I am a pharmacist by training, **[REDACTED]** of Haystack Project, and a rare disease mom. Haystack Project represents over 140 rare and ultra-rare patient advocacy organizations. Otezla is an excellent example of repurposing both in Behçet's and in pediatric psoriasis. The pediatric indications, for example, weren't approved until about 10 years after the first approval. If, for example, the price negotiations had been implemented in year nine of Otezla being on the market, would investing in a pediatric indication be a sound business decision? Probably not. And this issue is hugely important for Haystack's patient and caregiver communities, since we already struggle to get things on label and can't obtain access for off-label uses in diseases that are too rare to gain the attention of compendia.

We also believe that for treatments that are indicated in conditions that are sufficiently diverse to require a new NDA [New Drug Application], a simpler solution could be implemented, and that is to interpret qualified single source drug or QSSD the way Congress wrote it in the statute, by reference

to an NDA or BLA [Biologic License Application] rather than an active ingredient or moiety. We think the last administration defined QSSD incorrectly and now, it can be made right. This administration is interested in protecting innovation, and this can be done without any additional authorities or legislation to solve the problem. This would go a long way toward preserving innovation, especially in rare diseases that otherwise won't get on label.

I think we can all agree that post-approval research is extremely important, and these refinements in the negotiation process to target manufacturer behaviors that they were designed to reduce. As a pharmacist, I understand what those behaviors are. However, we must maintain incentives for behaviors that are aligned with the goal of ensuring that treatments are available even for the rarest of diseases. Also, we need the launch prices to be such that families do not have them set high and kept high to ensure recoupment of costs before negotiation occurs. There are things already under research for Otezla, for example, and other drugs on this list that aren't even on the radar for agencies' negotiators. Even I had to go find some pediatric Behçet's indications on my own, as it relates to research and development.

Also, related to cost, another concern that our communities have is that the negotiated prices in Medicare could have downstream impacts on formulary inclusion and out-of-pocket costs outside of Medicare. Rare diseases disproportionately affect children with about a 50/50 split between those covered commercially and by Medicaid who do not qualify for Medicare yet.

We read over and over that the air will be let out of the balloon and companies will increase costs elsewhere. Commercial payers don't have an out-of-pocket cap like Medicare does. And so, how will you track how the first year goes with the initial round of negotiated drugs in terms of the impact outside of Medicare? And how will those findings inform what you do before the 2027 prices come out? I would love to have broader discussions with you on these issues and rare disease, as there's not sufficient time to do so today. I do appreciate your time and the opportunity to speak on behalf of our rare disease community today. Thank you.

01:40:09

Moderator, PhD, RTI International

And thank you for your insights. The fourth speaker is **[Speaker 4]**. This speaker has indicated that there is no conflict of interest.

01:40:18

Speaker 4 (registered as a healthcare provider)

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Hi. Thank you again for listening to me for a second drug. I appreciate your time. So, Dr. **[Speaker 1]** already did a fabulous job of talking to you about the utility of this medication in certain settings, and I'm going to focus just on a couple points.

First of all, it is very useful in this setting of psoriasis, psoriatic arthritis, and Behçet's disease in particular. It is a unique oral drug. Most of our drugs that are used to treat moderate to severe disease are either IV [intravenous] or subcutaneous, which can have host of concerns, including access to the medication if you live in a rural area, difficulty if you're not able to inject yourself for a variety of reasons, or cost of medication, so it being an oral drug is really, really impactful. It's also not a biologic drug, and so all the biologic drugs have side effect profile of increased risk of infection and also sometimes reactivity, so the fact that this is not a biologic drug is also profoundly impactful.

Thirdly, we don't really need to monitor this drug. There's not even a CBC [complete blood count], which is a blood count, or a CMP [comprehensive metabolic panel], which is a liver kidney panel that needs to be done when we're following these patients. Again, how is that helpful? Cost. Access. So, if you live in the middle of Idaho, and you don't have access to a local laboratory, this may be a good medication for you.

And it's unique; this actually fills a niche in a very complicated field of autoimmune diseases where patients have a lot of disability and dysfunction. They have painful joints. They may have sores. They may actually have inflammation of their blood vessel. They may have skin that's raw and open. And, this provides a unique opportunity to help control disease, so these patients can have some semblance of a normal life. The problem is the cost is very high.

I've had a patient recently that I'd like to tell you about in the last minute or so. I had a patient who has a history of psoriasis, but doesn't have a lot financial wherewithal, doesn't have a lot of resources. And he was admitted to the hospital with a unique presentation called keratoderma blennorrhagicum, which is like psoriasis that is so thick and encasing that his feet look like the Hobbit's, and he's not able to walk and his joints were also very painful. He required high dose corticosteroids, which have a lot of side effects. We were able to get him on a biologic drug and tried second, third, and fourth biologic drugs, and he remains on those. Then we added Otezla and Otezla made a profound difference, such that he's able to go back to his construction job and he's able to actually have a semblance of a life. So, this drug is powerful, doesn't require monitoring. It doesn't make you immunosuppressed, and actually really is impactful in patients who have terrible disease. Thank you.

01:43:26

Moderator, PhD, RTI International

And thank you for your insights. Thank you all for sharing your experiences and perspectives about Otezla. I have one follow-up question for you all. Overall, how would you summarize the importance of Otezla with oral ulcers, and Behçet's disease, plaque psoriasis, or psoriatic arthritis. Please raise your hand to respond. We have a minute for your brief response. I saw a hand briefly. **[Speaker 3]**, go ahead.

01:44:09

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

Yeah, I will say, from the perspective of oral ulcers for Behçet's, our patients tell us that the biggest benefits are that it's an oral pill formulation versus an injection, as well as the fact that there is no black box warning on that particular medication as there is for others. Also of note, it is the only FDA-approved treatment for oral ulcers, which is important as well. Thank you.

01:44:37

Moderator, PhD, RTI International

Okay, so we have a few more, little bit more time. So, **[Speaker 1]**?

01:44:45

Speaker 1 (registered as a healthcare provider)

Thank you. Thank you so much, **[Speaker 3]**, for pointing that out that it is the only FDA-approved medication for the oral ulcers, and this is a very difficult manifestation of this disease. I'd like to share a story about another patient that, **[Speaker 1]**, thank you again, **[Speaker 1]**'s beautiful comments reminded me of a patient suffering from melanoma. And she developed very painful swelling in her joints to the point that she was having difficulty walking, driving, getting dressed, and when I said, I have medications that can improve your quality of life, I can get you back to your life, what do you want to start? The first thing she said was, whatever doesn't get in the way of my melanoma. I'm so scared of dying from my melanoma. And here's someone who's fighting for the last maybe months or hopefully years of her life, and she can't function, her quality of life is terrible, and this is a type of medication where we can say it's going to have a lower risk of side effects for you. This is a really unique medication, and we just really hope that cost won't be the barrier for our patients. Thank you.

01:45:50

Moderator, PhD, RTI International

Okay. Thank you. That brings us to the end of this Town Hall session. I would like to thank all our speakers for joining us today and providing important input for the Medicare Drug Price Negotiation Program. You can find additional information and news about the Negotiation Program on the CMS website listed on this slide. If you have questions, please contact CMS using the email listed on this slide. Thank you again.

=== 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