

*This transcript was lightly edited for readability.*

## Introductory Remarks

### Moderator, RTI international

Welcome, everyone, and thank you for coming today. I'm **[Moderator]** from RTI International, and I also want to introduce my colleague, **[Secondary Moderator]**, you can see on the screen, and you may hear from her during the course of the conversation today. The Centers for Medicare & Medicaid Services, which we will refer to as CMS by the acronym throughout the conversation, is convening this patient-focused roundtable event, as well as others, as part of the Medicare Drug Price Negotiation Program. The information shared during these roundtable events will help CMS understand patients' experiences with the conditions and diseases treated by the selected drug, patients' experiences with the selected drugs themselves, and patients' experiences with other drugs that are used to treat the same conditions as the selected drug. The information that you share during these events will also help CMS identify other medications used to treat the conditions treated by the selected drugs, what matters most to patients in managing their conditions, and other important factors CMS may consider in negotiating Medicare pricing with the manufacturer of selected drugs.

The purpose of our time together in today's event is to hear from you, a group that may include patients, caregivers, and patient advocates, and we want to hear about your experiences with the conditions and diseases treated by Xolair, which includes severe persistent asthma, food allergies, chronic spontaneous urticaria [CSU], and chronic rhinosinusitis [CRS] with nasal polyps. We want to hear about Xolair itself, as well as other medications for the same conditions. I want to emphasize that our conversation today will be on the patient experience, so if you have other information that you wish to share related to the Drug Negotiation Program that's not directly focused on the patient experience, we ask that you send that input to [IRARebateAndNegotiation@cms.hhs.gov](mailto:IRARebateAndNegotiation@cms.hhs.gov) instead of using the time today to talk about it.

Your experience and perspectives are very important to us, and we genuinely appreciate the time that you've given us today. Along those lines, we have a brief welcome video from CMS Leadership so that you can hear directly from them about how much they value your time and input today.

## CMS Remarks

00:02:38

### Dr. Mehmet Oz, Administrator for the Centers for Medicare & Medicaid Services

Hi, everyone. I'm Dr. Mehmet Oz.

I'm the Administrator for the Centers for Medicare & Medicaid Services, also known as CMS. CMS is the Federal agency that oversees Medicare, which provides health care coverage for more than 69 million older Americans and people with disabilities. We also oversee the Medicaid program and the Health Insurance Marketplaces.

I wish I could join you today in person, but I want you to know I am eager to hear your feedback and am deeply grateful for your participation in today's discussion.

It is a crucial conversation.

No one in America should have to choose between buying groceries or paying for their medications. But many are forced to make this choice. It's a choice that comes with a personal cost in addition to a financial cost. I started my health care career as a cardiothoracic surgeon. So I know firsthand what happens when people can't get their medicine, like the ones that lower their cholesterol or blood pressure. Left unmanaged, these conditions can be dangerous.

CMS is doing incredible work reigning in the skyrocketing cost of prescription medications, and we need all of you to help us make real, lasting change.

Right now, we're working on the latest cycle of Medicare drug price negotiation.

We announced the drugs selected for this round earlier this year. Some of them are covered under Medicare Part D, and others are payable under Medicare Part B. For every drug, our priority is to reach an agreement with the manufacturer on a fair price for Medicare.

We are committed to being fair and transparent throughout the negotiation process. And that's where you all come in.

It's my goal to get input from people across the health care ecosystem. We want to hear your perspective about the drugs selected for the current cycle of negotiation and renegotiation.

Your input makes a difference – a big one. Thank you for taking the time to join us today. I'll turn it over now to our event moderator.

**00:04:28**

**Moderator, RTI international**

I want to also make you aware that we have staff from CMS who will be sitting in on this event so that they can hear about your experiences and opinions directly from you. So let me hand it over to them for a moment so that they can say hello. **[CMS Staff]**, I think you are sharing today.

**00:04:47**

**CMS Staff**

Yes, thank you, **[Moderator]**. Welcome, everyone. I'm **[CMS Staff]** from the CMS Drug Price Negotiation team. There are other CMS staff on the call today as well. We work on the policies for getting public input and for negotiating Medicare drug pricing. On behalf of CMS, and on behalf of my colleagues, I want to thank you for participating today. We are looking forward to hearing about your experiences during this roundtable discussion. We're going to go off-camera now so you can focus on the discussion, but we appreciate your time. Thanks.

## Housekeeping

**00:05:17**

**Moderator, RTI international**

Thank you, **[CMS Staff]**. We're almost to the discussion part, but we have a few additional housekeeping items and ground rules so that everyone knows what to expect from our conversation today.

Technical assistance, if you get disconnected, please attempt to rejoin. If you cannot connect, please reach out to the [IRADAPStechsupport@telligen.com](mailto:IRADAPStechsupport@telligen.com) that you see on the screen.

In terms of privacy, the discussion is not open to the press or public. We will use first names only to protect your privacy, and we also ask that you please do not share any unnecessary protected health information, such as your doctor's name, or the name of a medical facility where you receive care. Also, we ask that you don't add any unnecessarily personally identifying information, such as your employer's name, or the city you live in, or the schools you've attended. Following the event, CMS will prepare transcripts that have participant names and identifying information removed. These will be made available to the public.

On a related note, we are recording today's event. These recordings will not be shared publicly. Recordings will only be used for internal program documentation and to produce the redacted transcripts for public release, consistent with Federal privacy guidelines.

By participating today, you're agreeing to be recorded for these purposes. We hope that you will contribute your perspectives throughout the session. However, if questions arise that you don't wish to answer, that's totally fine.

We ask that you minimize background noise by silencing your cell phone or other devices if you have not already done so. Please mute yourself when you're not speaking.

Thank you in advance for keeping your video on throughout the discussion.

And then a bit on timing. We have reserved up to two hours for this session. However, it's possible that we may not need the full two hours, and we can let you go a little bit early. I have a discussion guide in front of me, you've seen my paper, to help keep us on track. We have a lot of topics to cover, and so every now and then, I may need to redirect the conversation, or cut conversations short at times, to make sure that we cover everything, and that all participants have ample opportunities to share their perspectives.

If you need a break during the session, you can briefly step away. Turn your camera and microphone off, and then rejoin when you're able. You don't have to tell me that you'll be going away from your computer, return to the discussion as soon as you can.

Please try to speak one at a time. Occasionally, I may interrupt folks if two or more people are talking in order to be sure that everyone can be heard, and that we can accurately record everyone's comments. Please use the raise hand feature in Zoom to indicate that you would like to speak, and this will help me know that someone would like to add to the discussion.

And in terms of chat, we are hoping that folks will focus on our oral conversation today, but you can also add comments in the chat if you don't get a chance to share them orally. This may be the case, for instance, if we don't get to hear from you before we need to move on, or if you think of something later that you want to add on later in the conversation. Be sure to take a note, like, "I'm referring back to the question about X, Y, or Z."

Unless anyone has any questions, I'd like to move into introductions. Hearing none, I would like to go around the virtual room and ask each of you to briefly introduce yourself. During these introductions if you could share your first name, whether you will be sharing experiences as a patient, a caregiver, or as a patient advocate, and if you wouldn't mind sharing what condition or conditions that you will be speaking on today. **[Participant 1]**, you are the first one on my screen, so I'm going to hand it to you.

## Discussion

00:09:43

### Participant 1 (registered as a representative of a patient advocacy organization)

Hi, I'm [Participant 1]. I'm from a patient advocacy organization, and I'm going to be here speaking about patient experience regarding asthma, CRS with nasal polyps, CSU, and food allergy.

00:09:59

### Moderator, RTI international

Got a little bit of everything, so as we go through the conversation today, please be sure to highlight which condition to talk about at any given time. Thank you, [Participant 1]. [Participant 2], I think you are next.

00:10:12

### Participant 2 (registered as a patient and representative of a patient advocacy organization)

Okay, hi everybody, I'm [Participant 2]. I'm here today as both a patient, I have chronic spontaneous urticaria that's in remission now, and I also lead a patient advocacy organization.

00:10:30

### Moderator, RTI international

Thank you, [Participant 2]. And [Participant 3].

00:10:36

### Participant 3 (registered as a patient)

Yep, I'm [Participant 3]. I'm also a patient, speaking on the CSU side of things.

00:10:45

### Moderator, RTI international

Thank you, [Participant 3]. We're glad to have you here. [Participant 4], do you mind going next?

00:10:49

### Participant 4 (registered as a representative of a patient advocacy organization)

Sure, hi, I'm [Participant 4] with a patient advocacy organization, and happy to speak to asthma, food allergies, CRS of nasal polyps, and CSU.

00:10:59

### Moderator, RTI international

All right, also the whole kit and caboodle. And [Participant 5], could you take it away next?

00:11:08

### Participant 5 (registered as a patient)

Yep. Hi, I'm [Participant 5]. I am a patient, and I've used Xolair for about four years now for my food allergies. I started using it for the chronic hives at first, and now the food allergies.

00:11:28

Moderator, RTI international

Thank you, [Participant 5].

00:12:16

Moderator, RTI international

Thank you, everyone, for coming today. As you have heard during the introductions, we have folks representing all different conditions treated by Xolair, and we recognize those experiences may differ by these conditions. As I noted a moment ago, when you're speaking about your experience, if you wouldn't mind highlighting for the group which condition you're speaking about, or if you're speaking about multiple that would be useful to know as well. Let's get into patient experiences treated by Xolair. I want you to think for a moment about the different ways that the conditions treated by Xolair affect patients' lives. What would you say are the most important aspects of the conditions to have managed or treated? And these can be short-term aspects or long-term aspects. [Participant 1], get us started.

00:13:20

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

I'll get us started. Each of the conditions have a unique kind of thing that Xolair is effective for. For people with asthma, it's control of their asthma symptoms so they can live a normal life and try to maximize their quality of life. For CRS with nasal polyps, they can regain their ability to smell again, which is a lot more important than those of us who don't have that issue can appreciate, because it's a safety issue. They can't smell if a gas leak happens in their house. And they can't smell normal things that the rest of us smell, so it's a really important thing, not to mention the discomfort that they have with nasal polyps, as well as recurrent sinus issues. With food allergy, I can go on and on about that as a food allergy mom myself. But the fear of having a life-threatening anaphylactic reaction, being able to go to a restaurant and being able to eat food without the worry of having to go to an emergency room afterwards, being able to go on an airplane, to be able to travel internationally without worrying about a food reaction. All of those things have incredible safety issues and implications like that, but they also are incredible quality of life issues. And lastly, for people with CSU, the intense itch and living with that incredibly disfiguring condition keeps them from being out in their environment, the shame associated with it, the mental health issues of not being able to sleep, the family implications, all those things are so life-impairing in terms of that. And I want to mention that one of the startling things I found out in the past year was just published in the literature, that up to 33% of people with under-controlled CSU have suicidal ideations or have attempted suicide. Living with that condition is something those of us who don't have the condition can't appreciate, but it really does have incredible life implications for everyday living.

00:15:25

Moderator, RTI international

[Participant 1], thank you for that very comprehensive response. I'll go ahead and go to [Participant 2]. What would you like to add to the conversation?

00:15:33

Participant 2 (registered as a patient and representative of a patient advocacy organization)

For someone who does not have CSU, **[Participant 1]** really nailed it. I think itch is the big thing. It is excruciating, it is life-altering, and these sound like hyperbolic things and they're absolutely not. When I have a flare, it affects every aspect of my life. The itch is the worst, the angioedema, the swelling can be very painful for people. The presence of the hives, the fact that they're disfiguring can be really disturbing for people, but I think looking at how it translates, how does it impact quality of life, your sleep is really dramatically impacted when you have something like chronic spontaneous urticaria. Your emotional health, your mental health is impacted, and we know that comorbid depression and anxiety are very common. And we also know this impairs the ability to go to and to be present at work or school because it really effectively shuts down your life when you have a flare, it has ongoing control, I think, over your life.

**00:16:53**

**Moderator, RTI international**

Thank you, **[Participant 2]**, for adding that detail. **[Participant 3]**, I've seen you nodding very vigorously, that I feel like you have something important to add about the most important aspects to have managed.

**00:17:04**

**Participant 3 (registered as a patient)**

I think they both hit on some really important notes. I think the main ones for me are the isolation and the overall sentiment of danger and unknown and uncertainty with this, where it's especially if you don't have triggers that are easily identifiable and consistent, the spontaneous urticaria is doing a lot of work there, and that unpredictability makes your life so small. And for me, the only identifiable pattern was showers and exercise, and the full body hives from showers alone, I did not realize how heavily that was impacting my social life until it was no longer impacting my social life. I was realizing I was having to spend the whole day recovering from the showers, so I could never go out and feel put together and presentable, and then suddenly, when I was able to shower and not be bed-bound and bathing in Benadryl and trying to get through the day, I suddenly wanted to see my friends again, and I didn't feel so ashamed and gross with the hygiene limitations that CSU brings, and that freedom for both, being able to brave the unknown, and not really being able to predict your triggers, and also the ability to not self-isolate due to the debilitating symptoms. For me, it's a force multiplier across all my other comorbidities and disabilities, where if the CSU is acting up, everything else, the volume goes up, too. The Xolair has made a tremendous improvement in my hives, but I really was not expecting how much it has touched the other conditions and improved my quality of life in ways I didn't expect.

**00:18:48**

**Moderator, RTI international**

Thank you, **[Participant 3]**, for sharing your experience. **[Participant 5]**, what would you like to add? I believe you have food allergies?

**00:18:56**

**Participant 5 (registered as a patient)**

Yes, I was originally on Xolair for the urticaria. I have a condition, mast cell activation syndrome, and makes it so that I can be allergic to any food at any given point in time. And the Xolair is the only thing that's actually been successful in allowing me to keep my food down. I have developed eating

disorders as a result of my food allergies. I've had them since I was a child, and when you can't eat the same thing that everybody else does, you don't want to eat. Not only has the food allergies impacted my ability to function with something as small as eating, it affects my life in many ways, and it affects the life of those that are closest to me. I recently got married, and my husband and even for our wedding day, I always have to have a special meal prepared separately for me, and makes it very challenging for us to date and go out to restaurants. I had one injury that spiraled or increased the food allergies, or increased the mast cell activation, I guess, which then in turn increased my ability to not be able to eat many things at all. And there was a point where I was only able to tolerate three foods: chicken, rice, and broccoli, and they could not have salt or pepper, and they had to be cooked in olive oil. Fast forward to four years later, since being treated with Xolair, I can eat a variety of foods. I do still have reactions, but that's because of the mast cell, and without the Xolair I most likely would still have my eating disorder where I don't want to eat. And I was on the verge of being fed through a tube. Xolair has really made a difference in my life in the last four years, especially once we increased the dosage from the hives to the food allergy dosage.

**00:21:16**

**Moderator, RTI international**

Thank you, **[Participant 5]**. That sounds very difficult. I really appreciate you sharing that with us today. **[Participant 4]**, what would you like to add to the conversation?

**00:21:24**

**Participant 4 (registered as a representative of a patient advocacy organization)**

I think everyone has actually done an incredible job of describing the impact of all these diseases, and I really wanted to emphasize for each of these how it's not impacting part of your life, it's impacting your entire life. And so it's hard to say how will this improve, what would life be like without it? We hear stories about when they've lost coverage of Xolair, for instance, how incredibly devastating it is. Someone said, "I was so happy because I could", this is a patient with asthma, "I can go out walking, singing, dancing, and now I lost coverage, and I'm alone, I can't stop coughing, I can't breathe", and these are the kinds of stories that are really devastating. As we continue this conversation, we want to ensure that through this negotiation process, that access is also something that we talk about, and that is not at risk for the patients that are already on it.

**00:22:33**

**Moderator, RTI international**

Thank you, **[Participant 4]**, for bringing the importance of access up in the conversation. And I'll go to **[Participant 6]**.

**00:22:45**

**Participant 6 (registered as a patient)**

I think my story's pretty similar to everyone's. I was originally put on it for chronic hives and angioedema, and, similar to other people, my health had that element of being very out of control because with angioedema my big issue was swelling around my face and my neck. I have some other things going on, and I needed surgery, but nobody would do it because of the swelling and angioedema risk, which obviously is a smart choice because everyone was afraid of intubating me and not being able to extubate me because of the angioedema. And we had tried literally everything before someone finally suggested Xolair, and it was really difficult to get Xolair, and I'm sure other

people will talk about how hard it is to get coverage for Xolair, and I was finally able to get the coverage, and honestly, it was so life-changing. It was magic to be on it, and to not have swelling day after day after day, and to actually become stable. And the way that it changed my medical care because I'm now a patient that is not such a risk to everybody, doctors are now not afraid of me. It was like I became a different person to other people. Obviously, my experience as a patient, being in my own body changed, but the way other people treated me because I was no longer such a risk, I think it's really important to also look at how it changes your care. Now I'm stable so long as I can get Xolair. Other people are not afraid to do interventions. I was able to get the surgery that I needed. And so there is a sense, though, of always worrying about my access, like she said, to Xolair. It is something that I am concerned if I lose the access, I go back to being that patient that providers worry about.

**00:25:04**

**Moderator, RTI international**

Thank you, **[Participant 6]**. **[Participant 3]**, it looks like you want to weigh in a little bit more, too.

**00:25:13**

**Participant 3 (registered as a patient)**

Thank you. The experience of other providers, doctors, being too scared to touch you has been a common trend, especially in the MCAS, the mast cell activation syndrome, and the EDS [Ehlers-Danlos syndrome]. I was able to get open abdominal surgery a week ago today, and I would have never been approved for that if I did not have my MCAS and CSU managed with the Xolair. And for what it's worth, I know the MCAS is not an indication for it at the moment. I think in the future, that could be a really great way to expand access. I know a lot of us with MCAS and CSU and all the other stuff see the CSU is more of the skin manifestation of MCAS, but I'm even seeing my dentist and my hygienist once I started Xolair, they were like, "What are you doing differently? This is a night and day difference from the mouth I saw six months ago," were thinking that I had periodontal EDS, it was really getting severe, and being on Xolair for several months, night and day difference in my mouth and the way surgeons treat me, being able to access other specialties that before I was too high risk to do. And I definitely sympathize with the feeling of, I'm doing great now, but it is contingent on access, and it is a monthly battle that I am scared to lose, and I know I can't get lucky all the time, so I'm hopeful that we can set up some safeguards in place to hopefully help protect access for folks who need it, because I definitely sympathize with everything **[Participant 6]** was saying about it being so precarious, especially with other conditions in the equation.

**00:26:52**

**Moderator, RTI international**

Thank you, **[Participant 3]**. **[Participant 5]**.

**00:26:58**

**Participant 5 (registered as a patient)**

I'm not sure if we're going to go into this later, so you can cut me off if we are, but I did want to piggyback onto the idea of access. Because, to give an example, I had breast cancer, and after, and needing radiology, because of the MCAS, had difficulties with, I couldn't find a plastic surgeon who was willing to do reconstruction. We had to do things very carefully and have different protocols for radiation, and even then, the doctors were still afraid to treat me. And I also said I got married a few

months ago, and so my insurance changed as of January first, and because of that I have not been able to get approved for my Xolair for the last few months. Not only did I spend the first month being married being sick in bed from not being able to tolerate food because I could not get my Xolair from getting married and changing insurances. Again, there's always the fear. Right now, I'm still waiting on the prior authorization, and I've had the insurance for four months now. There's always that constant fear, and all it does is trigger more and more anxiety about, am I going to be able to keep food down? Am I going to be able to be functional?

**00:28:31**

**Participant 4 (registered as a representative of a patient advocacy organization)**

I wanted to add that we hear the exact same stories of people who are aging into Medicare. When they lose their employer insurance, when they switch over to Medicare, they have these access issues. And that's at another critical life stage, that we have serious concerns about it.

**00:28:53**

**Moderator, RTI international**

Thank you, **[Participant 4]**. In terms of what's most important to patients to have treated and managed, I've heard the symptoms themselves, the impacts on the quality of life, impacts on safety and stability, interactions with health care providers or family and friends, and mental health impacts. Are there other things that are important for patients to have managed or treated when one has these conditions? Got a lot of comprehensive answers, but I want to make sure before we move on, we have everyone have the opportunity. Go ahead, **[Participant 2]**.

**00:29:29**

**Participant 2 (registered as a patient and representative of a patient advocacy organization)**

I want to make sure that you caught the part about employment or school being impacted. I don't know if you put that under quality of life or where it fits, but I think that's a really important one. Livelihood, and your pursuits of hobbies, all of it.

**00:29:44**

**Moderator, RTI international**

Thank you, **[Participant 2]**. I've lumped it together, but thank you for making sure that it was heard and highlighted. **[Participant 6]**, did you want to add another area?

**00:29:53**

**Participant 6] (registered as a patient)**

I'm not sure where all other people are able to physically access their medication, but I am lucky enough that I have my Xolair shipped to home, and I administer it at home. I know that Medicare is not always a fan of delivering medication to the home. And that can be a struggle. But for me, I work full-time, and I am capable of doing that. If I had to go to a clinic, sometimes you have to wait a long time, sometimes they're running behind. I take Xolair every other week, it would be really hard for me to make that work with my schedule, and therefore, that would be one of those instances where the treatment is interfering with me being able to work, but I need to work to be able to pay for it. So I think making sure that access also includes the delivery being accessible.

**00:30:58**

**Moderator, RTI international**

Thank you, [Participant 6]. And [Participant 4], why don't you take the final thought, and I'll move us to another domain.

**00:31:04**

**Participant 4 (registered as a representative of a patient advocacy organization)**

It's related to how if everything is not being manageable, and I'm particularly thinking of food allergy and asthma episodes and exacerbations. If it's not well managed, there are the costs if you do end up in a hospital. That is something that you might not be able to manage, particularly with some of the way our insurance is designed. The out-of-pocket costs could be astronomical, which, again, would then add to maybe your mental health struggles, your anxiety, the stress of everything. Now you have this hospital bill on top of everything else.

**Moderator, RTI international**

Thank you, everyone, for sharing. I'm really hearing how all these conditions touch so many aspects of one's life. So now, I'd like to turn to the experiences with Xolair and its therapeutic alternatives. Get your typing fingers ready, and we're going to go to the chat. I would like for folks to enter into the chat the answer to this question, what medications, if any, have you, your loved ones, or patients you advocate for taken for the conditions treated by Xolair? And this can be taken currently, or been taken in the past. And if you have a list of 20 or 30, you can choose a few of the more recent ones.

**00:32:38**

**Participant 2 (registered as a patient and representative of a patient advocacy organization)**

And these can include over-the-counter?

**00:32:41**

**Moderator, RTI international**

Yes.

**00:32:45**

**Moderator, RTI international**

I'll give folks a minute to think and type. What medications have you, your loved ones, or patients you advocate for taken for the conditions treated by Xolair? And thank you, [Participant 2], for noting which condition you're speaking to. Thank you, [Participant 1]. I'm going to give folks another about a minute, 35, 40 seconds to a minute to add. I see the blend of over-the-counter and prescription medications. Thank you, [Participant 4]. Yes, thank you, everyone, for sharing those medication names.

Now for the next few questions, I would like to hear about either your experiences, your loved ones, or patients you advocate for, what experience they have had with Xolair, as well as the medications we're referencing here in the chat. Again, as we go through, if you wouldn't mind pointing us back to what medication you're speaking about. What benefits have you, your loved ones, or patients that you work with experienced with the medications used for the conditions treated by Xolair? Benefits of the medications. What benefits have you experienced? [Participant 2].

**00:34:48**

**Participant 2 (registered as a patient and representative of a patient advocacy organization)**

I think that Xolair saved my life, and this was for CSU. I was at a really desperate point where I was researching whether I would have to go on disability at work. I truly did not know if I could survive it, and there was that suicidal ideation that **[Participant 1]** talked about. I never had plans for it, but I certainly understood why it happens, why people do it, because it's completely maddening. For me, it completely stopped my hives after six months. I've stopped taking it. I have not had them since. And it then enabled me to do all of the things in my life that, and when I say really every aspect, I can't remember who said that, but every aspect of your life being impacted, it might have been **[Participant 4]**. It's working, relationships, taking a bath, walking the dog, wearing makeup, wearing clothing, putting a wool blanket on you. It is every facet of your life. We could go on for hours, and I see people nodding. It gave me my life back.

**00:36:08**

**Moderator, RTI international**

Thank you, **[Participant 2]**. That's a very profound benefit in terms of quality of life.

**00:36:11**

**Participant 2 (registered as a patient and representative of a patient advocacy organization)**

Yeah.

**00:36:12**

**Moderator, RTI international**

Are there other benefits, like with dosage or administration, that you found with Xolair or the therapeutic alternatives?

**00:36:21**

**Participant 2 (registered as a patient and representative of a patient advocacy organization)**

I liked that Xolair was something that I had to just do once every month. That was a benefit to me. At the time that I took it, they were not doing it at-home administration, but I think that was a benefit. And the fact that it worked. I did not mind the other things that one might be concerned about, having to go to the allergist's office, it was worth it to me, even with the risk of anaphylaxis, the cost-benefit. Clearly the benefits outweighed it for me.

**00:36:59**

**Moderator, RTI international**

Vastly. **[Participant 1]**, looks like you had your hand up next, so what benefits would you like to comment on related to either Xolair or the medications, the therapeutic alternatives to Xolair?

**00:37:12**

**Participant 1 (registered as a representative of a patient advocacy organization)**

The one thing I want to mention, I don't know if there's going to be room for this somewhere else, is that all of these different diseases have a similar underlying mechanism of action, as some of the attendees here have said, it's treating more than one of these diseases, and so there's a multiplication of benefit for the folks who are dealing with more than one of these conditions. A

common one is asthma and food allergy, where the asthma gets under control, and then the food allergy is under control. And the important part of that is good asthma control is really essential for food allergy management, because reactions can become severe and life-threatening, and poor outcomes usually relate to asthma being under poor control for food allergic patients. I wanted to emphasize that in case it doesn't come up somewhere else.

00:38:06

Moderator, RTI international

That is an important benefit and thank you for bringing that one into the conversation. **[Participant 3]**, what benefits have you observed in your experience with these medications, whether it's Xolair or other medications used to treat your health conditions?

00:38:20

Participant 3 (registered as a patient)

I was on about 20 medications going into it, and maxing out antihistamines and mast cell stabilizers. Prior to this, the cromolyn sodium ampules were what I was heavily reliant on, but the difficulty with those is it was four times a day dosing, 30 minutes dissolved into water before eating. And as my GI [gastrointestinal] symptoms and manifestations got worse and worse, I increasingly had to choose between drinking and eating. I couldn't do both. Even the volume of liquid, as those symptoms progressed, I became unable to even get that down.

When Xolair came into the equation, I was able to come off of cromolyn sodium. I ended up staying on the rest of the antihistamines and a couple mast cell stabilizers, but being able to eliminate the load that the cromolyn sodium brought to me of, I don't want to be the person at the restaurant who has to ask for a tiny glass of water to shoot an ampule into and drink four times a day. And, at best, it's inconvenient and a little embarrassing. At worst, it was simply impossible, and I lost around 65 pounds without trying, and the real wake-up call was being 25 and buying a life alert bracelet, because I was finding myself lying on the bathroom floor yelling for help.

I very much sympathize with **[Participant 2]**'s share about it really feeling like it saves your life in a literal sense. I think that we get to a very desperate place before we get to Xolair, and I hope that one day people won't have to suffer through as many of these, fail this first, step therapy approaches. Overall, I would say the self-injection, night and day difference in terms of the sheer volume of medication and the mental load and the stuff you carry around, flying with an EpiPen rather than an EpiPen and a small mobile pharmacy of everything I could possibly need, it is an incredible feeling of freedom that I've tried so many medications and rarely I don't know if any other medication has made as much of a difference in my life as Xolair. I have had many medications work for me, but I don't think I've ever had a medication work this well. It's been a change in my quality of life that I would say is comparable to when I got my power chair. It has revolutionized my access.

I think that the medication alternatives, there aren't a lot of great alternatives. The other one was the newer one, the remibrutinib, somebody listed it. I know that has bleeding and clotting risks, and my immunologist was really scared to put me on that one, so it feels like Xolair's our last man standing, and I would not be showing up to this roundtable a week after surgery for any other drug besides Xolair. It's truly been a night and day difference.

00:41:30

Moderator, RTI international

That's quite profound, [Participant 3], thank you. [Participant 1], it looks like you had something else you wanted to add into the conversation.

**00:41:35**

**Participant 1 (registered as a representative of a patient advocacy organization)**

I'll switch gears to CRS with nasal polyps. Sometimes people will go to an ENT [ear, nose, and throat doctor] first, and surgery will be the next step to control the nasal polyps. And they recur 40% of the time, whereas getting treated with an effective biologic like Xolair for nasal polyps can manage the disease without the needs for surgery and with lower risk of recurrence. Wanted to mention that as well.

**00:42:06**

**Moderator, RTI international**

Thank you, [Participant 1]. And [Participant 6], we saw you put something in the chat that things kind of worked, but then also had some consequences, so would you mind chiming in and adding anything you might want to about what worked and when it didn't so much.

**00:42:22**

**Participant 6] (registered as a patient)**

It was a long time coming trying every single antihistamine ever invented. And then trying them at up to four times the regular dose. Insurance drives that, part is doctor's suggestion, but part is the process of getting those prior authorizations, because you have to show that you maxed out, and you have to be able to describe why that's not going to work, and part of that is you have to have a negative side effect. Some of it was, Zyrtec, Xyzal, your tongue has to become actual sandpaper, which is super uncomfortable. And then you have to try different combinations of them, trying them different times a day, different nose sprays that they invent, even though it's like, that's actually a step down, in my opinion, is I had to try, I forget the name of this really ridiculous nose strip, but I had to try some of those, and then emergency Benadryl at monster doses. There was a meme going around Facebook, I don't know if any of you saw those, "you can't sneeze if you're unconscious." I felt like that really highlighted my experience. I had to show that I had tried all of those things before I was allowed to actually try Xolair. And I don't know if other insurances have this requirement, I actually still have to say that I'm on high doses of antihistamines in order to still be eligible to fill a Xolair prescription. I still have to say I'm taking Xyzal at four times the dose while being on Xolair, or else my insurance will say I'm not sick enough. So that's a little trick I'm playing.

**00:44:27**

**Moderator, RTI international**

[Participant 5], did you want to comment on food allergies and the benefits of any of the medications you've tried? Whether it's Xolair or others.

**00:44:35**

**Participant 5 (registered as a patient)**

I think everyone really touched on a lot of what I would have already said. The only thing I would add is that, Benadryl, I had gotten to a point where I was taking Benadryl too much, and Benadryl does cross the blood-brain barrier, and so there's concern of that, especially, again, when I've had food

allergies since I was young and have been taking Benadryl. Xolair allows me to not even have to take Benadryl as often. And yes, same thing, I have to be on all of the other four times the normal doses of all the other antihistamines that I've tried as well. And, the thing about the antihistamines is, I think we're all different, and so what works for one may not work for another, and so it really is trial and error, whereas Xolair worked right away for me, and it was the probably one medication that I can say actually worked the first time for me, because I don't usually tolerate too many medications. I usually have adverse reactions to them.

**00:45:47**

**Moderator, RTI international**

Thank you, **[Participant 5]**. Go ahead, **[Participant 3]**.

**00:45:50**

**Participant 3 (registered as a patient)**

I wanted to add about the necessity for insurance to be on high doses of antihistamine simultaneously. To me, it feels like that is like shooting itself in the foot, as one of the best benefits of Xolair is moving away from the polypharmacy of being on so many medications from so many providers for so many things, and having that be a requirement feels like it's almost limiting its own success when one of the biggest differences it makes is reducing the need for those medications across the board. I'm hopeful that requirement would maybe eventually be able to be lifted. I know it's tricky from insurance to insurance, but I think that that's a huge benefit of Xolair that we're not even really allowed to sing the praises of without worrying about getting booted from Xolair itself.

**00:46:43**

**Moderator, RTI international**

Thank you, **[Participant 3]**. And I know we've covered this a little bit in the conversation, but I want to make sure we open it up. It sounds like folks have been on more than one medication. Would you mind comparing and contrasting the benefits of the different medications that you've tried? Go ahead, **[Participant 5]**.

**00:47:07**

**Participant 5 (registered as a patient)**

The antihistamines may or may not work, and for me, at different times in my life, depending on what other health conditions I'm managing, that also affects how well the antihistamines work for me. For example, I had a hyperactive gallbladder and needed my gallbladder taken out, and could not keep many foods down. Even foods that I normally could tolerate, I could not keep them down. And then as soon as I had the gallbladder removed, I could tolerate all of those foods. And we were still on the same antihistamines that I had been on. We were trying a lot of different things. It really didn't matter, because of the other inflammation that was going on in my body. The only thing throughout all of that that still worked was the Xolair, and the only reason I know is because I actually came off the Xolair for about two months, by choice, because of the amount of medication I was on, and then came to realize that it was actually the Xolair that was helping, and not necessarily the other antihistamines. It's not that they didn't help at all, it's that they didn't help to the same degree as the Xolair.

**00:48:30**

**Moderator, RTI international**

Thank you for that, [Participant 5]. [Participant 6], please add in.

**00:48:36**

**Participant 6 (registered as a patient)**

I saw someone mention steroids, and I guess I forgot. Steroids have been a huge part of how my condition's been managed, and actually, they have had the most negative side effects, they have a really rough profile of things that are negative that they cause. Steroids work very quickly, and obviously manage angioedema and hives quickly. Antihistamines have a huge side effect profile as well. I don't think people realize, because they're over-the-counter, that they have such a severe side effect profile. Antihistamines cause a really bad rebound effect, with coming off of them when you lower the dose, like the insomnia, and they cause rebound itching. Even if you open the packaging insert for Benadryl or even Zyrtec, and then look at Xolair. The side effect profiles, Xolair is safer, so it's been really interesting to me that it was so hard for me to get Xolair, and when I asked doctors about it, because I campaigned for it, I had read about it, and I wanted to go on it before doctors suggested it, and I had to really advocate for myself, and then fight my insurance company. It was so interesting to me that it was so hard to get it when it is safer if we're talking about the side effect profiles, and then my direct experience of being on it, and what I experience, and it works so well, and I can take less of the medication less often. It's really a no-brainer for me as far as managing my medical condition. One drug is safer, I can take it at a lower dose. I've been on it for a long time, and I have not had to increase the dose or the frequency.

**00:50:41**

**Moderator, RTI international**

Thank you, [Participant 6], and you've actually led us into the next questions. We talked about the benefits and comparing and contrasting the benefits. I wanted to ask about the drawbacks or challenges that you, your loved ones, or patients you advocate for experienced with the medications used for conditions treated by Xolair. So benefits, drawbacks, and challenges. [Participant 3], go ahead and get us started.

**00:51:04**

**Participant 3 (registered as a patient)**

For the medications used to treat this stuff, I think people really highlighted all of the drawbacks and consequences of the antihistamines and steroids. But, in terms of the side effects of the Xolair itself, I was not prepared for the "painsomnia" of the first month or two, I guess, where I have chronic pain and chronic joint pain, so that wasn't surprising to me, but the fact that it was amplified to the point where I wasn't able to get any sleep for the first week or so was really disturbing to me, but it was so effective at its job that I went to my doctor, and I was like, "I know it's not good practice to assign medications for side effects of other medications, but Xolair's giving me a 180, but I can't do this every month, in terms of a week of feeling really sore, and pain," and the more I talked to fellow patients with it, the more I was reassured this is super normal for month one. Month two was a little easier. By month three, I had no side effects. And in the last year, it's been smooth sailing. I had to change my dose a little bit, but much less than all of my other medications. So that's a huge benefit. I would say that maybe I didn't read my packet insert thoroughly enough, or got lost in the sauce of all the excitement, but I remember that that kind of blindsided me a little bit. I was willing to grit my teeth through it if that was what it took for the Xolair to work long-term. Super

grateful that that's not what happened, and that it did indeed drop off after the next month. But that's something that I, and people who I've talked to, have been kind of surprised by with the Xolair.

**00:52:56**

**Moderator, RTI international**

I think it's the first time I've heard the phrase, painsomnia. Thank you, **[Participant 6]**, **[Participant 3]**. **[Participant 4]**, I see you put some of the drawbacks of corticosteroids in the chat. Did you want to highlight any other drawbacks of corticosteroids or other medications?

**00:53:15**

**Participant 4 (registered as a representative of a patient advocacy organization)**

I think we all are aware that you don't want to be treated with corticosteroids. It is not good care. It does act quickly. Long-term side effects, when people are getting to Xolair, we've talked about step therapy, you have to do this because you are not getting relief on other medications. The general side effect of living with symptoms continues to be a downside. So I'll leave it there.

**00:53:51**

**Moderator, RTI international**

Thank you, **[Participant 4]**. And **[Participant 2]**, I'm going to ask you to weigh in on some of the drawbacks. I see you put some of the drawbacks in the chat, but if you wouldn't mind expanding on that.

**00:54:00**

**Participant 2] (registered as a patient and representative of a patient advocacy organization)**

To follow up on what **[Participant 4]** was saying, you want to avoid corticosteroids, but boy, even now, without having flares, I would do it again. I know all the risks and everything else, but in a heartbeat, I would take them again, because that's how miserable it is. People are forced into that when they don't have other options. I think I'll go to the antihistamines. They're so easy to get for the most part. If you can get to a pharmacy, you can get your hands on not just Benadryl, which is not standard of care for CSU, but you can get your hands on second-generation antihistamines that can help you. And then, if they're adding in the H2 blockers [histamine-2 receptor antagonists], it's really easy to get those too. You can get Pepcid or whatever else at your CVS, wherever you live. The drawback is if you're having this conversation like we are, those don't work for you. Even you're taking massive amounts of them, and they're still not working for you, but they're they are easy, accessible, and oral, which is a preference for a lot of people.

**00:55:17**

**Moderator, RTI international**

Thank you. **[Participant 5]**, **[Participant 1]**, or **[Participant 6]**, would you like to weigh in on the drawbacks of whether it's Xolair or any of the other medications you've taken?

**00:55:32**

**Participant 1 (registered as a representative of a patient advocacy organization)**

I was going to say everybody's covered it pretty well. **[Participant 5]**, go right ahead.

00:55:37

**Participant 5 (registered as a patient)**

The only thing I was going to add is the drowsiness. When you're already on just one antihistamine, it can cause you to be drowsy, but being on four times a dosage, and then it's multiple, it's been quite difficult playing around with different dosages so that I can have a few more hours to function in the day, as opposed to being knocked out the way that Benadryl does.

00:56:06

**Moderator, RTI international**

Thank you, **[Participant 5]**. I see some nods, and **[Participant 3]**, I wanted to ask, you had mentioned earlier about the reduction in polypharmacy and being able to be on one medication with Xolair. Would you mind also comparing and contrasting your experience with the drawbacks of the different medications you've tried?

00:56:26

**Participant 3 (registered as a patient)**

I would say with the antihistamines the biggest one was the drowsiness for me. I was literally having to leave my one-hour-long college class, lay down, set a timer for 10 minutes, take a tactical nap, and then go back for the last 25 minutes of my class. A profound level of drowsiness, like falling asleep eating food, it got to a point where I was waking up to eat and to go to the bathroom, and I was sleeping through the rest of the day, and couldn't enjoy time with my family or my friends, or even the two rooms that I was limited to in the worst of my disability, suddenly became one room, and I couldn't even keep my eyes open in it. That was the biggest life-limiting one for me, and then for the mast cell stabilizers, like the ketotifen, the cromolyn sodium, stuff like that, the low-dose naltrexone, cromolyn sodium is great, but like I was saying, the logistics of the intake can be really difficult. And then as far as ketotifen and low-dose naltrexone, also great, but they need to be compounded generally, and that gets expensive very quickly. And it can also be really difficult in terms of getting that through and into the patient's hands. For me, being able to get the Xolair shipped to my immunologist, or shipped to my house, and not have to use my one waking hour in the day to go to the pharmacy, made a tremendous difference. And then being able to cut back on a few of those medications really made taking the medications I am still on easier and more consistent. I was finding myself not sleeping through and missing doses anymore, and being able to be a more active participant in my life as a whole.

00:58:19

**Moderator, RTI international**

Tactical napping, okay. Thank you, **[Participant 3]**, for the phrasing today.

00:58:32

**Moderator, RTI international**

Since you're off mute, **[Participant 2]**, could I tap you to if you do any comparison, contrast of the drawbacks of the different medications you've tried for your condition?

00:58:42

**Participant 2 (registered as a patient and representative of a patient advocacy organization)**

Oh, yes. The drawbacks, like I said, I would still take these medications. The drawbacks for the corticosteroids would have been any side effects for long-term use. For short-term use, for me, the insomnia was kind of a nightmare. I did not have the painsomnia, but I was essentially hypomaniac. There's a reason you're up at 3 a.m. vacuuming, and it's called a corticosteroid. I think the drawbacks, I had stopped taking Benadryl, so I was not noticing as much drowsiness as the antihistamines, but the drawbacks to those is it's that it didn't work. It was nothing about the medications, because there were several of them themselves. It was the fact that they were insufficient to control my symptoms because they were so severe. Those were the things that I had tried. I think, too, that part of the issue with CSU is that even sometimes physicians think it's an allergy and you run into a lot of trouble with how you're treated because of that. It is almost never an allergy, and that makes it difficult, I think. People are running into difficulty in how they're treated because of that. But I think otherwise I've expressed everything else when it comes to the drawbacks and benefits. The big benefit of Xolair is that it worked, and I no longer I don't even want to call it live, live the life that I was at the time.

01:00:37

**Moderator, RTI international**

Thank you, **[Participant 2]**. **[Participant 1]**, I saw you put something in the chat. Would you mind expanding a little bit on this?

01:01:07

**Participant 1 (registered as a representative of a patient advocacy organization)**

We didn't mention the fact that for asthma and CRS with nasal polyps that there are other biologics available, but, for instance, dupilumab is based on the biomarker for eosinophils, rather than for IgE [Immunoglobulin E], which is an anti-IgE biologic. I wanted to mention that. There's tezepelumab for asthma as well. That's for even for low eosinophil count, and I'm trying to remember the other biologics. There's Nucala, there's a whole bunch of them. But, again, they're biomarker-driven. Thank you, **[Participant 4]**. Appreciate the checking on that. But there's different biomarkers that are run, and then based on that, it underscores the fact that you can't interchange biologics with another biologic for these conditions. You have to really look at the underlying biomarker and what the disease mechanism of action would be.

01:02:09

**Moderator, RTI international**

Excellent point, **[Participant 1]**. And **[Participant 2]**, why don't you take the closing thought on this question?

01:02:15

**Participant 2 (registered as a patient and representative of a patient advocacy organization)**

Sure, **[Participant 1]** reminded me, because I'm thinking about my own experience, but when I look at the larger CSU community, we have two medications that were just launched in the U.S. last year, one of them being dupilumab, which for many this is used for many conditions we're talking about, the other being remibrutinib. I think the big drawbacks to those medications, that issue is access.

People, their physicians knowing about it. Them knowing, patients knowing about it, so they can advocate to get it if they want to try something else. And because these are only essentially months old, when you think of the time that has passed since launch.

**01:02:57**

**Moderator, RTI international**

Thank you, **[Participant 2]**. Thank you, everyone. Overall, when considering potential medications for any of these conditions, what matters most? What factors matter to patients the most? What factors matter to patients the most when considering medications? Go ahead, **[Participant 2]**.

**01:03:26**

**Participant 2 (registered as a patient and representative of a patient advocacy organization)**

For me, and for many people that I see, or talk to, it's, does it do what it says it's going to do? First and foremost, can I expect relief from my symptoms? Obviously, I think safety is a huge consideration for people. What other things will happen to me as a result of taking it I would need to know about that. And access and cost would round it out for me.

**01:04:06**

**Moderator, RTI international**

Thank you, **[Participant 2]**. And **[Participant 3]**, what matters to patients most, or what matters to you the most as a patient?

**01:04:13**

**Participant 3 (registered as a patient)**

For me, of course, the biggest is will it work or not? That's always the end-all, be-all. And of course, the price and access make a difference, too. But for me, the method of administration made a big difference. I know for a lot of people with comorbidities, like gastroparesis, when they did an endoscopy, they found a bunch of pills in my stomach, and they told me try and switch every medication you have to an injection, a liquid, a patch, because we're not sure those pills are going anywhere. So the new medication, the remibrutinib I don't remember what that one's fully called yet, but that one, was ruled out, because the Xolair was an injectable, and that one was a pill, and they weren't sure that the pill was going to make it where it needed to go, so as much as many people dislike self-injections, as a method of administration, for me, that was a huge asset, and a big plus on the side of Xolair, and especially since you don't have to take it as often as many of the other oral equivalents, that's a bonus as well.

**01:05:18**

**Moderator, RTI international**

**[Participant 1]**, you had your hand up. What matters to patients most when selecting a medication for these conditions?

**01:05:24**

**Participant 1 (registered as a representative of a patient advocacy organization)**

I agree with **[Participant 3]** and **[Participant 2]** that it's going to be, does it work? But then, can I get it? And will I have to make a trade-off by having a less effective medication? Those are really front of

mind for the patients that I've spoken with regarding access to any kind of medication that they need for these conditions.

**01:05:47**

**Moderator, RTI international**

Thank you, **[Participant 1]**. And I think, **[Participant 4]**, I saw you nodding.

**01:05:51**

**Participant 4 (registered as a representative of a patient advocacy organization)**

I'll add to that. It's exactly that. Obviously, it matters if it works, but if it works, then you can't get it, and you can't afford it, what's the point of it? We did a few years ago a survey called *My Life with Asthma* where we surveyed people or caregivers with asthma, and cost was the number one barrier to taking medication as prescribed. I'll add that note.

Who comes up with the names of all of these drugs? Because that's why we...

**01:06:25**

**Moderator, RTI international**

Yeah, they are a bit of a mouthful those names. Let's see, **[Participant 5]** or **[Participant 6]**, would you like to chime in about what matters as a patient to you the most? Go ahead, **[Participant 6]**.

**01:06:43**

**Participant 6 (registered as a patient)**

In addition to what everybody's saying, does it work, can I access it, and is the treatment worse than the condition? Obviously, is it a seven-hour infusion that I have to do every two days, or is it going to be easy to actually do this? And it comes in a very nice little injectable needle that is easy for me to use, it is easy, I'm not a nurse, I'm not squeamish or anything, I'm on a couple different injectable meds. And Xolair is a lovely needle. It is not hard to do, it is not hard for me to handle, it's not horrific for me to order, or deal with, or receive. Like I said, I'm lucky enough to be able to get mine at home. I've been on other treatment regimens where I have to go to a clinic and, not their fault, but, one shot can turn into a four-hour affair, plus parking, plus getting validation, all of that. I can't be off every two weeks, I can't miss a full day of work every two weeks, so that does factor into it.

**01:07:58**

**Moderator, RTI international**

Thank you, **[Participant 6]**. And **[Participant 5]**, it looks like you had something that you would like to add in?

**01:08:03**

**Participant 5 (registered as a patient)**

The main thing is obviously, will it work? That's the number one thing. But in addition, the same things everyone else said, piggybacking onto what **[Participant 6]** just said, if I was still working, and I'm on disability full-time, I have been for the last six years, but if I was still working, I doubt that there would be a way that I would be able to manage, because I'm disabled and under 65, having

Medicare, you do have to go to the office to get the Xolair injection, every two weeks, and that can be really challenging, but everyone else, I think, encompassed everything pretty well.

**01:08:44**

**Moderator, RTI international**

And **[Participant 5]** if you wouldn't mind adding in a little bit about your experience in terms of Xolair in regards to asthma?

**01:08:59**

**Participant 5 (registered as a patient)**

Oh, so I wasn't ever using the Xolair for asthma, it was the hives initially. Is that what you mean, the urticaria?

**01:09:08**

**Moderator, RTI international**

Yeah.

**01:09:09**

**Participant 5 (registered as a patient)**

So what was the question again, then?

**01:09:12**

**Moderator, RTI international**

Oh, I wanted to see if you could comment a little bit on asthma and Xolair.

**01:09:22**

**Participant 5 (registered as a patient)**

Same thing, it manages the asthma enough that I don't even have to use the inhaler very often, maybe once or twice a year, so it's been a game changer in that aspect.

**01:09:34**

**Moderator, RTI international**

Thank you, **[Participant 5]**. And **[Participant 4]** or **[Participant 1]**, would you mind also sharing a little bit about the patients you advocate for, and Xolair for asthma? How it affects exacerbations, or steroid bursts, or hospitalization, avoiding steroid bursts and avoiding hospitalizations?

**01:09:54**

**Participant 4 (registered as a representative of a patient advocacy organization)**

That's exactly what we hear. On this medication, you're avoiding the ER [emergency room], you're not using your rescue inhalers as much, your quality of life goes up, you can sleep. It's real symptom management, and I have one patient I'm thinking of in particular, when she was getting ready to go on Xolair she did have to go through step therapy, so she was like, "I know that I'm going to have to be sick for the next few months, but I see light at the end of the tunnel, because I know this will work for me," and that's exactly what it did. And so she's incredibly happy that one of the

ways that she used to describe it is, “Unless you have asthma, you don’t think about breathing every minute of every day. We just breathe.” And, it’s everything. She was bringing her nebulizer to work; that’s really disruptive. She wants to appear quote-unquote normal. She didn’t want to be disruptive. She didn’t have her inhalers with her. That would give her anxiety. She would have to turn around, leave places. So it’s a huge impact on quality of life.

**01:11:17**

**Moderator, RTI international**

Thank you, **[Participant 4]**. And **[Participant 1]**, do you mind commenting on patient experiences with asthma and Xolair?

**01:11:23**

**Participant 1 (registered as a representative of a patient advocacy organization)**

Xolair is going to control the asthma if it’s the right drug for that patient, and that means the person won’t have uncontrolled asthma, which is very common in our population, even if you’re not on Xolair. Besides the symptom control, being able to sleep at night, not having symptoms in between, whatnot, it’s health care utilization that’s going to go down. There’s going to be less ER visits, urgent care visits, less hospitalizations, and most importantly, no deaths. So those are a really big advantage of having well-controlled asthma, and if Xolair is what is needed for patients, then it’s a really important drug for them to have access to.

**01:12:07**

**Moderator, RTI international**

Go ahead, **[Participant 4]**.

**01:12:08**

**Participant 4 (registered as a representative of a patient advocacy organization)**

To piggyback on that, exactly, I think people often think of asthma as being just asthma, but nine to eleven people still die every day from asthma. So it’s a very serious disease that, when you’re severe, uncontrolled, it is life-threatening, and this is an amazing drug, if it is the right biologic, but there are lots of options. But not for that. The person who needs it, they only need that one, to be clear.

**01:12:39**

**Moderator, RTI international**

Yes. And **[Participant 6]**, I saw your hand go up earlier. Did you have something to add related to Xolair and asthma?

**01:12:46**

**Participant 6 (registered as a patient)**

Not as much, but I guess I’ll throw in there, I do have asthma, and I got the added benefit that when I went on Xolair, I did not need a rescue inhaler anymore. It’s something that I didn’t even notice, because I really only filled it once every other month. But, my asthma was really triggered by cold air, I can’t breathe in cold air at all, and I think I started Xolair in October. It doesn’t get that cold where I live in October, but it definitely does by November, and I park in a garage, and I walk in the

city into work, and I was walking in November. And I forgot my scarf, which is something I wrap really tight around my face, and I forgot it, and I was like, oh no, and I was walking, and I went to go panic, and suddenly realized I could breathe, and it was cold, and it was one of those moments that I was like, oh yeah, I remember the commercial, Xolair was on TV, and I'm like, oh, and it helps with asthma, and I realized, oh, I haven't filled my inhaler, I have not had any issues. It's mid-November, I have been breathing, so it was one of those funny moments where I was like, oh, Xolair is not just helping in this way, it's helping in this way. And it's almost like I don't even have asthma anymore at all.

**01:14:24**

**Moderator, RTI international**

I think others have commented on those secondary benefits for their other chronic conditions. **[Participant 3]**, do you want to close us out with this section?

**01:14:34**

**Participant 3 (registered as a patient)**

On the added asthma front, I suddenly got a ton less questions of, "**[Participant 3]**, why are you breathing so heavy? Are you okay? Why are you wheezing?" I then started to be more successful in PT [physical therapy] and using my inhaler less and less, and, especially with albuterol and how much it would raise my heart rate with, SVT [supraventricular tachycardia] and heart stuff in the equation, it was always a really tough cost-benefit analysis of, "I'll be able to breathe, but I might have to call the ER to cardiovert me," and not having to do a cost-benefit analysis in crisis, and now at all, honestly, because it's been so well controlled, is great, but, I think that the costs associated with using an inhaler sometimes were worse than the symptoms I was experiencing. But overall, the breathing in general has gotten a lot easier. My sleep is better. Yeah, there's no part of my life that has been untouched by Xolair. Every part of it has improved.

**01:15:38**

**Moderator, RTI international**

Thank you. I'm hearing some recurrent themes here. We've covered a lot of ground, and I know some of this is going to feel a little bit like we're coming back to some familiar ground, but I appreciate everything that folks have shared. Now I'd like to talk about how well Xolair and other treatments meet patients' medical needs. Thinking back about an hour or so ago, we asked you to reflect on the most important aspects of the conditions treated by Xolair to have managed or treated. Aside from the aspects you have already shared, what other medical needs related to these conditions are important to you, your loved ones, or patients you advocate for? What other medical needs are important? Go ahead, **[Participant 6]**.

**01:16:33**

**Participant 6 (registered as a patient)**

I think what I said, starting off. My main medical condition was the unpredictable swelling, which made it hard for me to get other needs met. Becoming stable medically was life-changing for me. Having a condition that is unpredictable and is life-threatening makes you have to live your entire life around being a patient. I went from being a patient first, a patient with a medical condition, to just being a person. That is life-changing in and of itself, to just be a person.

**01:17:14**

**Moderator, RTI international**

From being a patient to a person, that really encapsulates a lot, **[Participant 6]**, thank you. How about anyone else? Any other important needs related to specific symptoms, side effects, etc.? Go ahead, **[Participant 1]**.

**01:17:29**

**Participant 1 (registered as a representative of a patient advocacy organization)**

I'll bring up food allergy. The avoidance of food is the only option without having Xolair as a treatment to bring the whole IgE level down so that you don't react. That means that there's less likelihood of an allergic reaction, and again, back to the health care utilization, that means these individuals will not have to cycle through the emergency room or be at risk for a poor outcome, including death. Just like with asthma, there's not a huge number of people that die, but we don't want anybody to die, because with asthma and food allergy, all of those deaths virtually all are preventable. Those are really important things to keep in mind regarding the importance of Xolair for people for whom it's indicated.

**01:18:25**

**Moderator, RTI international**

Anyone else, other medical needs that you want to share or raise? Then, to what extent are the medical needs we've discussed being addressed by the treatments that are available? To what extent are the medical needs that we've discussed over the course of the conversation being addressed by the treatments that are available?

**01:19:04**

**Participant 1 (registered as a representative of a patient advocacy organization)**

I'll weigh in for CRS. The options for CRS are limited. Without a biologic, the option is going to be inhaled steroids, which may or may not work for the individual, or surgical repair and removal of nasal polyps which can recur. I wanted to put that out there for the CRS people, that they have few options that are really ideal.

**01:19:35**

**Moderator, RTI international**

**[Participant 2]**, do you want to go next about to what extent the medical needs are addressed by current treatments that are available?

**01:19:44**

**Participant 2 (registered as a patient and representative of a patient advocacy organization)**

It's such an interesting question. I think that the medicines that we now have, collectively are doing a much better job than where we were five years ago, even. But that's for the patients who can get that far. If you're misdiagnosed, if you're underdiagnosed, you're not getting there. So those medicines are not working for them because they can't even get to that point. Does that make sense? But if they do, I think it is not working for everyone. We obviously have not solved it for CSU.

But people are making their way through and finding benefit from one of them, if they can get there. If they can make it that far.

**01:20:36**

**Moderator, RTI international**

Bring back the notion of access.

**01:20:39**

**Participant 2 (registered as a patient and representative of a patient advocacy organization)**

Access and access in a different way. It's not just access to affording it or your doctor prescribing it, it's even being diagnosed with the right condition.

**01:20:53**

**Moderator, RTI international**

Okay, getting the right diagnosis. Yes, thank you, **[Participant 4]**. And then, to what extent are medical needs not being addressed? Are there still gaps in the available treatments?

**01:21:18**

**Participant 4 (registered as a representative of a patient advocacy organization)**

A gap in the treatments could be when you don't have access to them. Just harp on that, when you have an actual gap in your ability to access this medication. That continues to be a real issue.

**01:21:35**

**Moderator, RTI international**

We'll go to **[Participant 3]**, and then back to **[Participant 2]**.

**01:21:37**

**Participant 3 (registered as a patient)**

I think the access, big gap. I think the dosing, and timing, I feel like I'm getting to the max, according to my allergist-immunologist, and there is fear from people who've also been on Xolair for a while, a couple years of what's next if Xolair stops working? And I hope I don't have to entertain that question anytime soon, but now that I'm at the max dose and max frequency that my diagnosis will allow, there is that fear of, what comes after this? Whether that's, can I take Xolair every week instead of every other week, or do I do two pre-filled syringes instead of one, or what else? But the idea that there really isn't an equivalent that you could turn to as a potential next option if this one doesn't work is a scary thought. In the comparison between how well this meets medical needs versus past equivalents, to me, it's like moving from somewhere with one bus that came once a day to living in a place with a robust metro system, where it's like, technically it works for some people to get to some places, but it's a lot of waiting and a big uphill battle, and it's not convenient versus this drug is revolutionary. When you access it, it's easy, the side effect profile is pretty low, the risks are manageable. It doesn't even feel reasonable to compare them, because it's like comparing a horse to a Ford Mustang. It's different leagues, personally.

**01:23:22**

**Moderator, RTI international**

Thank you, **[Participant 3]**. **[Participant 2]**, we'll go to you, and then we'll go to **[Participant 6]**.

**01:23:26**

**Participant 2 (registered as a patient and representative of a patient advocacy organization)**

We are still seeing patients who have tried everything, meaning everything that is FDA [Food and Drug Administration] approved or recommended in the guidelines, who still do not have their symptoms adequately controlled. We know that there's still a shortfall there.

**01:23:47**

**Moderator, RTI international**

Go ahead, **[Participant 6]**.

**01:23:52**

**Participant 6 (registered as a patient)**

I think I'm one of those patients that still had a little bit of trouble. I ended up needing to add Dupixent to my treatment regimen, so I ended up still having chronic sinusitis. But, going on a second biologic, I joked that you thought maybe I was asking for phoenix feathers, or cocaine, even? I actually had to get my state attorney general involved in arguing with my insurance company, because they refused to respond to appeals, and I had to get the Department of Labor involved, and then they got exhausted, and it became this mammoth, 18-month process. And really, this is an issue of cost, it's not a safety issue to be taking these two medications together. As somebody pointed out, they are responding to different types of inflammation, and I obviously had two different types of inflammation because Xolair targets one, and Dupixent targets another one, and even though they do have overlapping FDA approvals, they are different drugs, or else they would be the same drug. So Xolair changed my life and got me working and got me access to so much, but I still had this one small, very specific problem, and there was another drug that's out there for it. And so after 18 months, I was able to get it, and I've been living large since then. And my insurance probably has a dartboard with my face on it, because they have to fill both of these drugs now, and I'm enemy number one. But I think it is worth mentioning that Xolair is a very specific, targeted drug that targets a very specific type of inflammation that we know causes specific conditions. And it's great that it continues to get expanded FDA approval, but there is a shortcoming for some people that have specific inflammation. But that was my experience.

**01:26:18**

**Moderator, RTI international**

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

We talked about the urticaria, we've talked about the rhinosinusitis. How about allergy and asthma? Any gaps that folks see remaining? **[Participant 5]**, if you don't mind me calling you, what are some of the gaps you're seeing in your experience with the food allergy?

**01:26:59**

**Participant 5 (registered as a patient)**

I still have to take multiple medications. I'm still doing allergy shots. I think I'm in year four of allergy shots. I think similar to **[Participant 6]**, that because there are still other symptoms that I'm experiencing from the mast cell, it's always the fine balance of what additional things that need to

be added. Especially for the food allergies, there are still times where I'll have to take extra antihistamines depending on the type of meal. I try to eat a low histamine diet as much as I possibly can, but that's really difficult sometimes. I occasionally will have to take additional medications to manage those or to prevent the flares.

**01:27:55**

**Moderator, RTI international**

Such a balancing act that you've described. **[Participant 1]**, do you have comments on food allergy or asthma you'd like to share?

**01:28:05**

**Participant 1 (registered as a representative of a patient advocacy organization)**

The important thing to know about Xolair and the use of food allergy is you can't eat the food freely, it's something to prevent a small exposure and not that resulting in a severe allergic reaction. We're still looking for new therapies that will allow for people to be able to live with their food allergy and eat food more freely than this, but Xolair is the best we have right now in trying to help people who really could benefit from it. I wanted to make sure everybody was aware of that, that there's still more research that needs to be done, and new therapies that need to be conceived for people who have food allergies, and there's no cure.

**01:28:57**

**Moderator, RTI international**

You took the words out of my mouth, **[Participant 4]**. And **[Participant 4]**, do you mind if I call on you to talk about asthma? Gaps in treatment for asthma.

**01:29:06**

**Participant 4 (registered as a representative of a patient advocacy organization)**

I think we were talking about this a bit with the seven different kinds of asthma biologics. Asthma's a heterogeneous disease, one person's asthma is one person's asthma. If Xolair is the right biologic, and this is part of the patient experience, though. You've already been through a long experience, generally, if you're getting access to a biologic, and unfortunately, it shouldn't be a full-time job to get access to the medication that your doctor has prescribed, and that you have already been through quite extensive rounds of different types of therapies that are out there.

**01:29:52**

**Moderator, RTI international**

Thank you, **[Participant 4]**. All right, we're beginning to wrap up. The event's nearly over, and before we part ways, I'd like to go around the virtual room and see if anyone has any closing thoughts on Xolair, or raise any topics that you feel we didn't adequately cover during the conversation today. Do you have any final thoughts on Xolair, conditions treated by Xolair, or any of the medications used to treat the same conditions that that you feel are important to share with CMS? **[Participant 1]**, you're at the top of my screen. I'm going to see if you have any closing thoughts.

**01:30:26**

**Participant 1 (registered as a representative of a patient advocacy organization)**

Our mantra is, affordability does not mean access, because sometimes if a drug is priced so low it will disappear from formularies that become available to patients, so I want to mention that without going too far into the weeds, that access and affordability are separate and distinct issues.

**01:30:48**

**Moderator, RTI international**

Thank you, **[Participant 1]**. And **[Participant 3]**, you're next. Leave us with some amazing phrases.

**01:30:54**

**Participant 3 (registered as a patient)**

Oh, that's a high bar. I wanted to amplify, I think it was something that **[Participant 4]** noted about how paying for these therapies up front can save a lot of costs in terms of back-end emergencies that happen when you're not able to prevent these things at the outset. There's more and more research establishing mast cell activation as connected to Long COVID [post-acute sequelae of COVID-19], so I'm predicting that as much as Long COVID is a problem now, it's only going to become more and more important, and this drug is going to become more and more impactful. And super agree with what **[Participant 6]** was saying about it's a specific therapy for a specific mechanism of action, and it excels at that. And I have seen so many people get their lives back in so many different ways, and I think the mast cell activation syndrome is highly under-acknowledged in the medical community, and Xolair is at the forefront of those patient discussion groups. Every single day, people are asking questions and talking about Xolair, and I think with the trajectory of how things are going overall public health, that could definitely be a long-term cost-saving measure by having this more available up front before people deal with the really advanced consequences of Long COVID and uncontrolled inflammation and all this stuff.

**01:32:14**

**Moderator, RTI international**

Thank you, **[Participant 3]**. **[Participant 4]**, why don't you go.

**01:32:45**

**Participant 4 (registered as a representative of a patient advocacy organization)**

I would reiterate what everyone has said—this drug is really important to people for whom it works. We are obviously concerned with cost and appreciate CMS going into these negotiations, but we want to ensure that, as **[Participant 1]** said, lower cost doesn't necessarily translate to the person at the pharmacy counter. Making sure that cost-sharing schemes don't change because of where if there's lower prices, we're still getting saddled with higher costs. Ensuring that as this moves forward, we continue to monitor how this is impacting patient access and cost, at the same time.

**01:33:41**

**Moderator, RTI international**

And **[Participant 5]**, any closing thoughts?

**01:33:45**

**Participant 5 (registered as a patient)**

I think everybody pretty much wrapped it up. I will touch on again how much being able to actually access the medication at different times has been able to help me. And right now, dealing with waiting for prior authorizations for months and months, I'm thankful that my allergist office has samples of Xolair that they've been able to provide me with, but insurance doesn't know that, so it's really important to patients like me that literally have food allergies that cause all kinds of other comorbidities, like eating disorder and things of that nature.

**01:34:34**

**Moderator, RTI international**

Thank you, **[Participant 5]**. **[Participant 2]**, I think you get the last word.

**01:34:38**

**Participant 2 (registered as a patient and representative of a patient advocacy organization)**

One of the themes that we have heard repeatedly here was that people are not saying that this is a medication that takes a sting away, or makes it so that their skin looks better. That's not what people are saying. People are saying that it gives them their lives back. What can you say that about otherwise? It's such a profound and important thing that I think that's the sub header for all of this. This is a medication that can potentially give people their entire lives back, and I can't imagine anything more important.

**01:35:21**

**Moderator, RTI international**

Thank you, **[Participant 2]**. **[Participant 1]**.

**01:35:24**

**Participant 1 (registered as a representative of a patient advocacy organization)**

Since we're talking about a Medicare population, and we've been talking about quality of life. I'm thinking of elderly people who may not be able to advocate for themselves, or communicate what kind of things they're feeling, like intense itch, or asthma symptoms where they can't breathe. I want to underscore that we need to be thinking about these really vulnerable populations, and how this kind of drug can keep things at a status quo so that their conditions are not going out of control.

## Closing Remarks

**01:36:02**

**Moderator, RTI international**

Thank you, **[Participant 1]**. Thank you, everyone, for participating and giving us well over an hour and a half of your time today, and participating in today's event. We appreciate you taking the time to talk with us and share your experience. Your experience and input were very valuable, and they will help inform CMS' negotiations for Medicare pricing for Xolair. CMS staff want to say a few closing words and they will bring your perspective back to their team. **[CMS Staff]**, do you want to close this out?

**01:36:47**

**CMS Staff**

Yes, thank you. On behalf of CMS and my colleagues attending this event, who are now on screen, thank you very much for taking the time to share your experiences and perspectives with us. I especially want to thank the patients on the call, **[Participant 3]**, **[Participant 2]**, **[Participant 6]**, and **[Participant 5]**. We appreciate you sharing your personal stories with us today. Each of you has given us a lot of valuable information to consider, and for that, we are genuinely grateful.

**01:37:12**

**Moderator, RTI international**

Thank you, **[CMS Staff]**. And, as a closing note, if you have any questions following today's session, you can submit them to the mailbox at [IRARebateAndNegotiation@cms.hhs.gov](mailto:IRARebateAndNegotiation@cms.hhs.gov), is what's on the screen, and you can use the subject line Public Engagement Events. Thank you, everyone, so much for spending this time with us today.

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

For a list of the drugs selected for the current 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-2028.pdf>

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

## Appendix

Participant 1: Registered as a representative of a patient advocacy organization

<b>Declared Conflicts of Interest</b>	
No	Receipt of financial payments (e.g., gifts, funding, research support, honoraria, travel, or other expenses) from companies with direct/indirect interest in the Negotiation Program (e.g., drug companies, health plans) in excess of \$10,000 by you, your spouse, or an immediate family member.
No	Direct assistance preparing your remarks from someone who is NOT a family member, caregiver, friend, or your health care provider.
No	You, your spouse, or an immediate family member is employed by or holds equity interest (stock or ownership interest) in excess of \$10,000 in companies or related associations with direct or indirect interest in the Negotiation Program (e.g., drug companies, health plans).
No	Any other personal or professional relationships or interactions with companies or related associations with direct or indirect interest in the Negotiation Program (e.g., drug companies, health plans) that may be considered a financial COI.

Participant 2: Registered as a patient who has experience with the selected drug or the conditions treated by the selected drug; representative of a patient advocacy organization

<b>Declared Conflicts of Interest</b>	
No	Receipt of financial payments (e.g., gifts, funding, research support, honoraria, travel, or other expenses) from companies with direct/indirect interest in the Negotiation Program (e.g., drug companies, health plans) in excess of \$10,000 by you, your spouse, or an immediate family member.
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Participant 3: Registered as a patient who has experience with the selected drug or the conditions treated by the selected drug

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Participant 4: Registered as a representative of a patient advocacy organization

<b>Declared Conflicts of Interest</b>	
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Participant 5: Registered as a patient who has experience with the selected drug, the conditions treated by the selected drug, or other treatment(s) or drug(s) similar to the selected drug for those conditions

<b>Declared Conflicts of Interest</b>	
No	Receipt of financial payments (e.g., gifts, funding, research support, honoraria, travel, or other expenses) from companies with direct/indirect interest in the Negotiation Program (e.g., drug companies, health plans) in excess of \$10,000 by you, your spouse, or an immediate family member.
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Participant 6: Registered as a patient who has experience with the selected drug, the conditions treated by the selected drug, or other treatment(s) or drug(s) similar to the selected drug for those conditions

<b>Declared Conflicts of Interest</b>	
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