Lipodystrophy United

Speaker 1: Maddie Pensiero

Good morning, everyone. Welcome. How's everyone doing? Amazing.
Welcome to the lipodystrophy Externally led patient focused drug development meeting. We are so glad that you're here. My name is Maddie Pensiero and I have the honor of serving as moderator today alongside my co-moderator and dear friend Andra Stratton.

Rowan McDonnell and Kate Stratton in the back will be our co-moderators supporting online participation. To every patient, carepartner and loved one of someone impacted by lipodystrophy who is with us today, thank you. If you are joining us virtually know that you are just as much a part of this room as everyone else. Your voice and comments carry the same weight as anything said from this stage. Thank you also to our sponsors, Regeneron and Entos whose support made this hybrid event possible, along with the many individual donors who supported our 25 stories for 25K campaign.

Before we begin, a few quick notes to help you feel comfortable and prepared. This is a long, meaningful day. Take breaks when you need them, ask for help anytime, and participate in whatever way feels right for you.

This is not a conference or a lecture. It's a town hall, and it belongs to the patients and care partners in this room. In a few minutes you'll hear brief remarks from the FDA and a clinical overview of the condition. After that, the drug developers, researchers, clinicians, and regulators here are here to listen. Patient and care partner stories are why this meeting exists and will comprise the majority of the content.

Why does this matter? Because individual experience, the parts of life that don't show up in a lab report or chart, the evidence this meeting is designed to capture. What a good day looks like, what a bad day looks like, what still feels hard even when labs look fine.

Everything shared today; spoken aloud, in the chat, written into the docket, or added afterward becomes part of the public record and will be built into the Voice of the Patient Report.

Comments added after today, again, carry just as much weight as anything said in this room.

Here's how the day will unfold. Kate Stratton, Lipodystrophy United's Executive Director, will kick things off, followed by remarks from Dr. Julie Golden of the FDA and Dr. Elif Oral with a short clinical overview. At 10:30, our first patient panel and open discussion begins focused on the burden of disease, along with a few polling questions to help us get to know who's in the room and online with us as well. We'll break for lunch at 12:15, which is a great opportunity to visit the gallery of posters outside, and also check out the storybooks at your tables. At 1:15, our

second panel turns to the burden of treatment, followed by another discussion, and we'll close by 3:30. A few logistics. AI translation is available. You can scan this QR code, pick your language, and follow along in real time. Just please be sure to keep your devices muted if you're here in this room with us. When you speak, please use the microphone, even if you think your voice is loud enough without it. Our zoom attendees and anyone hard of hearing, need it picked up as well for the recording. So please raise your hand, a mic runner will find you and wait to speak until you have the mic in your hand. If you're in the zoom chat, your comments can be read aloud, so please type what you'd like to share. We can't wait to hear from you.
On format, our questions are shaped by the core areas the FDA looks at in this type of meeting. So for each area, I'll open with a general question to the room and ask that you please keep responses to about 2 or 3 sentences, so we can hear from as many voices as possible.
Andra or I may ask a quick follow up, and if we step in to move things along, it's never personal. It just means making space for the next piece of the puzzle. If you're cut short, please put the rest in writing. Again, it carries the same weight. The most useful thing you can share is something specific and concrete. Numbers, frequency, what something actually costs you in time and energy, rather than a general statement. Please speak about your own experience, not on behalf of others. No two people with lipodystrophy live it the same way. And we ask that you step up and step back in these discussions. If you haven't shared yet, take the space. If you already have, consider making room for someone else. One last note today on scope. This meeting is focused on symptoms, daily impact, and treatment experience. Diagnosis journeys, insurance, and pricing are outside of what the FDA can act on here though we know how real those burdens are. We also want to name something honestly. Today is heavy, it's real, and it's raw. Counselors from Give an Hour are here throughout the day if you need support. There's Julie over there from the Give an Hour team.
Speaker 1: Maddie Pensiero
13:50:19
Thank you for being here. And between speakers, Andra and I are going to model, taking a brief, quiet pause before moving on. Just a breath to sit with what was shared. Feel free to join us in that pause if it feels right for you. And now I am delighted to introduce Kate Stratton, Executive Director of Lipodystrophy United.

Speaker 2: Kate Stratton
13:51:02
Hello everyone. Thank you very much for being here today. Before we begin, I would like to acknowledge something very important. Twenty-five years ago, our country experienced an unimaginable loss. Recognizing the weight of this day, I would like us to observe 25 seconds of silence in remembrance of the people whose lives were lost on September 11, 2001, and in honor of their families and loved ones.
[25 seconds of silence.]
Thank you.

Lipodystrophy United

Thank you all for being here today. It is truly an honor and a privilege to welcome you. Though I have been in my role at Lipodystrophy United for just three years, I have spent my entire life as a part of this community. My mom lives with Lipodystrophy. And growing up, I didn't always understand what that meant. Sometimes, I thought she was being dramatic. Sometimes, I was angry. Mostly, I was confused. But through my love for her, I've spent my life, and now my early career, dedicated to listening and helping with honesty, integrity and compassion. Today, you will have an opportunity to do the same.

There are symptoms you will hear about today that sound familiar. We all get hungry. We all get tired. We all experience pain. And that makes these symptoms incredibly easy to underestimate. I have spoken to hundreds of people in this community and I'm still learning everyday about what lipodystrophy takes from peoples' daily lives, and how treatment might be able to help. We are tasked with representing the broad spectrum of lipodystrophy in just a few hours. If anyone can do that, it's this community, who has waited years for an opportunity like this. I hope that by the end of today, you understand lipodystrophy differently, maybe completely differently, than you did when you walked into this room.

We went to great lengths to represent many different voices today, including visiting people's homes and helping them record their stories. We also conducted an IRB-approved study with cohorts of people with different types of lipodystrophy. You will see quotes from this study hung up around the room. At your table, you will also see a booklet with additional stories of folks living with lipodystrophy. If you are a member of the FDA, please engage with these during lunch, and if you are tuning in virtually, we will share a link to these resources.

During those months that people worked on their stories, 5 people dropped out, and we found others to share their story. One person who hoped to be here today is in too much pain to travel. Many days, she cannot even walk her dogs. Another person nearly lost her life because of severe cardiovascular complications. When another community member started a new job, the increased stress caused her metabolic numbers to go into mayhem. One person traveled with me for two days to work on her story, and then spent weeks recovering from a foot ulcer. Another member of our community spent nine months in the hospital for a double lung and heart transplant and has just returned home.

Importantly, there have also been wins. One person was finally healthy enough to travel to Mexico to visit her father after years of being too sick to make the trip. That's because she is now receiving a treatment that works for her.

That is lipodystrophy. It is incredibly up and down. I saw it growing up. My mom could seem great one day and be extremely sick the next.

I want to emphasize that the people who are sickest are sometimes the people least able to sit in a meeting like this and tell you how sick they are. So as you listen today, I ask you to remember not only the people whose voices you will hear, but also those who could not be heard.

Working with this community has been the greatest privilege of my life. It has also been heartbreaking. We lose members of this community far too often. Every loss reminds me that one day this disease will take my mom from me. I think about whether she will be here to see my

partner and I have children, and how many holidays we will have together. I wonder how long I will get to be with her, learn from her and work with her on incredible, impactful events like the one here today. I will never be untethered from the fact that my mom has lipodystrophy. But I also have to have hope.
There is a scientific possibility that did not exist before today. But possibility is not enough. Our community urgently needs more treatment options, and those treatments need to address the things that actually matter in people's lives.
Lipodystrophy has shaped my childhood. It has shaped my relationship with my mom. It has shaped the work I choose to do. And I hope that what you hear today shapes you, too, even just a little bit. When the day eventually comes that I lose my mom, I want to know that I did everything I could for her and for every family who navigates this disease. Today is part of that. You are all a part of that.
Thank you, and l would like to introduce Dr. Golden who is our wonderful FDA speaker.

Speaker 3: Dr. Julie Golden
Thank you for the introduction. Good morning. It is a pleasure to be here.
13:56:53
My name is Julie Golden. I'm a clinical reviewer in the Division of Diabetes, Lipid Disorders, and Obesity, also known as DDLO, within the Center for Drug Evaluation and Research at FDA. And it's great to see so many patients, caregivers, and advocates in the audience and attending virtually. Thank you all for being part of this meeting and sharing your experiences with us. I would also like to thank Lipodystrophy United and all the staff that were involved in this meeting and for inviting us to participate. We also welcome today other clinical experts and scientists at FDA and from elsewhere as part of this discussion. A little bit about me: I'm a board certified internal medicine physician and have been a reviewer at FDA for 22 years, evaluating drugs developed for a diverse array of metabolic diseases, including those associated with lipodystrophy. I was the primary reviewer and participated in the 2013 Advisory Committee meeting for metreleptin, which is now approved in the United States as an adjunct to diet as replacement therapy to treat the complications of leptin deficiency in patients with congenital or acquired generalized lipodystrophy. So Lipodystrophy is a rare and heterogeneous condition which can introduce unique challenges in drug development. With limited treatments available, we recognize that there is more work to be done to further advance the treatment and management of lipodystrophy. FDA protects and promotes public health by evaluating the safety, effectiveness, and quality of new products, but we do not develop medical products or conduct clinical trials. It is, however, FDA's responsibility to ensure that the benefits of a product outweigh its risks. Therefore, having this kind of dialogue is extremely valuable for us, because hearing what aspects of their condition patients care about can help us figure out how to best facilitate medical product development for Lipodystrophy and understand how patients view the benefits and risks of therapies for Lipodystrophy.

Patient-focused drug development, or PFDD, is a systematic approach to help ensure that patients' experiences, perspectives, needs, and priorities are captured and meaningfully incorporated into drug development and evaluation. As experts in what it's like to live with their condition, patients are uniquely positioned to inform the understanding of the therapeutic context for drug development and evaluation. PFDD meetings give FDA and other key stakeholders, including medical product developers, healthcare providers and other partners important opportunities to hear directly from patients, their families, caregivers, and patient advocates.

Due to growing interest in expanding efforts to gather patient input in support of drug development and evaluation, in 2015, FDA started welcoming patient organizations to identify and organize their own patient-focused collaborations. As with all PFDD meetings, these externally-led PFDD meetings provide an opportunity to hear directly from patients. We want to hear about the symptoms that matter most to patients, the impact the disease has on their daily lives, and the experiences with currently available treatments, and what patients are seeking in new treatments to manage their condition. This will help inform the focus of new drug development and future clinical trials. To date, over 60 EL-PFDD meetings have been held. While FDA is open to attending an Externally-Lead Patient Focused drug development meeting, the meeting and any resulting products like reports or surveys, are not considered FDA-sponsored or endorsed.

Meetings such as Externally-Lead PFDD meetings strengthen understanding of disease and treatment burden, what we call the therapeutic or clinical context. The patient input from meetings can support FDA staff as they conduct benefit-risk assessments for products under review or advise drug sponsors on their development programs. We also believe that patient input collected from these meetings can have value to drug development more broadly. But for example, by helping to identify areas of unmet need, such as an aspect of a patient's condition that is not currently being addressed in current therapies. This input may also help developers as they identify or create tools used to measure the benefit of potential therapies.

A summary report from an externally-led PFDD meeting can be shared on FDA's website. And there is a link here. And we have links to our FDA CDER patient focused drug development webpage, which provides more detail on the efforts currently ongoing at FDA. We look forward to incorporating what we learn today into the agency's thinking and understanding of how patients view benefits and risks of therapies for lipodystrophy. Your input and perspectives really help us, and will also help industry and academia to move the drug development process forward to ensure that new medications meet the needs of people living with lipodystrophy. Once again, we are all here today to hear the voice of the patient. So thank you for your participation. We are grateful to each of you for sharing your personal stories, experiences and perspectives.
Speaker 1: Maddie Pensiero
14:03:54

Thank you Dr. Golden, I would like to invite up Dr. Elif Oral.

Let me begin by thanking Lipodystrophy United for including me in this great program.
Speaker 4: Dr. Elif Oral
14:04:00
I do have a little bit of a daunting task of providing a clinical overview for a very heterogenous group of diseases and my job is to help you find some realities, shared realities, across many diseases, and many stories. I began working with lipodystrophy about 30 years ago, it may even be to the day, literally, it was a September Tuesday actually, that I met my first patient with lipodystrophy in 1996. It has been my distinct privilege and honor to develop my career with this community. You all have given me a purpose. You all have taught me a lot, so I am extremely grateful for all the experiences that I've had, and I'm speaking here today not only on my behalf but on half of our scientific advisory board on behalf of Lipodystrophy United. Their input also helped develop these slides. So the first thing I am going to tell you is what it means to live without healthy fat. So this is the common ground of lipodystrophy. And we have to remember that adipose tissue is an active organ, and its failure changes body shape, reprograms metabolism, causes inflammation, and all organs get impacted. Fat has two important jobs. One is to actually create a safe place for extra energy to be there and it can be tapped into at times of need. In addition, in its absence, you know, when this organ is absent, misplaced, or inflamed, patients have consequences that are visible and invisible at the same time, and it lasts and accumulates over a lifetime. Also, the other function of fat is to send out hormones and other factors to signal energy sufficiency to the brain and rest of the body.

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We began understanding this with the incredible discovery of leptin by Doctor Friedman, who's in the audience today and.

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The leptin is the most well known signal, and if those signals are absent or incorrect, the body will not know that there is optimal energy balance.

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And as a result, you know, in its absence or insufficiency, there's this relentless metabolic disease and hunger, painful and stigmatizing body changes and organ complications that accumulate over a lifetime. So this is how I met your disease. Actually, my patient, one, you know, she changed me forever, and I remember her dearly.

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As I walked into the room, it was very clear that she had paucity and deficiency of adipose tissue, she told me that it was from birth. She also had very severe insulin resistance and all the hallmarks of severe insulin resistance that I could see physically, the acanthosis nigricans (the darkening of the skin in the creases), hyperandrogenism and all of that. She had high levels of triglycerides. In fact, her triglycerides were in a couple of thousands for hemoglobin A1C was very high. She was on literally thousands of units of insulin and still had those numbers. She also described continuous hunger, excessive sweating after she ate and preoccupation with food and anxiety, depression. She felt very isolated and stigmatized. So that's the story, the classical story but there's not just one lipodystrophy. My patient that I met at that moment had congenital generalized lipodystrophy, but there are more than 30 rare disorders and we know different pathways, many of them converge on one problem, adipose dysfunction and insufficiency. And we have developed different ways of classifying the disease. We could sort of say generalized versus partial fat loss or we could say that the condition is genetic versus acquired. I've sort of taken a little bit of a different approach, put the classical forms versus more complex forms to remind us that sometimes we have to remember additional things in the background that we need to address as well. So I did put the classical forms as generalized versus partial and then I put the acquired immune forms into the more complex forms because there are additional concerns that we have to remember and then syndromic conditions that may affect a lot of different organs in different ways.

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And I've put in some of the slides that we've accumulated over time, but they represent and LU did a wonderful job collecting much better pictures and they're scattered in the hall. As I mentioned, the shared mechanism is that there's nowhere safe to store energy and failed signals from fat. And this cartoon that was developed by both a render and ChatGPT and my trainees, you know sort of depicts all of that. We start out with adipose failure. Then energy can't go anywhere, stays in the blood or goes to alternative organs, but also the signals are missing. And then that gives a sort of an important signal to the brain that more energy needs to come in. And with that, there's this vicious circle of bringing in more energy but not having a safe place to go. Then there's ectopic lipid accumulation and accumulation of organ damage.

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And that's not just in one place. It's classically in the liver, heart and kidneys, muscle, but they're also really no organs paired. So it does affect everywhere and it affects everyday function. So there's more than diabetes. You know, we endocrinologists meet these patients mostly because they have an unusual form of diabetes. But it's not just that. There's this hunger and food vigilance and anxiety around it.

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There's the triglycerides and the pancreatitis risk. There is this relentless liver disease and abdominal burden, abdominal pain, you know, both from the pancreatitis recurrent episodes in the past and the big liver. There's the heart and muscle limitations. Then there's the appearance stigma and procedures that come with it. And then there's these important considerations of fertility, puberty and pregnancy. So it's not just one domain. And the diagnosis, the same diagnosis doesn't mean that there is the same daily reality. You know, I've put in some things that may be different between the different forms, but you know, this doesn't really summarize everything. It's just like an attempt to make the point. In generalized forms, there's very low leptin, early severe metabolic disease and hunger may dominate the phenotype. In partial forms, there's this regional fat loss, variable lectin levels, trunk and face increased adiposity may coexist and the metabolic disease is still relentless. In acquired and immune forms there's inflammation, autoimmunity flares and unpredictable trajectories. And in the syndromic and complex forms, there is much earlier muscle and intrinsic muscle cell, heart, cell, bone abnormalities, neurological conditions and developmental complications that can shape the burden ahead of the metabolic diseases. So the big question is which burden should drug development measure. Within that, you know, I had the honor and the privilege and the luck to sort of be there for developing leptin. So we observed that patients with generalized lipodystrophy had low leptin. We had very sick patients. I was actually working on a project to look for causes of lipodystrophy, but the unmet medical needs sort of overshadowed

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the urgency to find causes and we had the opportunity to bring leptin therapy to our patients who were coming to see us and donating their blood for mechanistic studies. So then that experience changed us. It certainly changed me. I think it changed the field. It worked really well for patients with very severe forms of disease. And as I mentioned, for a trainee you know, looking for projects. You know, seeing that experience changes you forever, but I truly believe that it changed us as a community I'd say, and showed us that when the right biologies match to the right patient, treatment can be life changing. But I now know after working for 30 years on this disease that one biological pathway is not enough. Leptin is a key chapter, but it's not the whole book. We still have to deal with the energy storage issue, the immune system and organ protection differently and we have to look for additional pathways to address and hopefully this meeting will help us hear what biology matters most to patients. So again, this slide is just a big reminder that most forms carry biology beyond metabolism. It is a whole body disorder and every organ system is impacted. And as you see here, there's the inflammation and immune system activation or dysfunction. There's the muscle and heart disease to think about. There's the liver and kidney risk. There's also bone issues, growth and reproduction. Rebecca Brown, sitting in the audience, recently summarized all the bone data. And I encourage everyone to look at that. That's why our patients will describe fatigue, pain, flares, procedures, hospitalizations and fear of future procedures. And this burden changes across a lifetime. This 10 minute talk must make room for a lifetime. They will be discussing today's symptoms, but the hidden burden is the

forecast. What complications come next and can it be prevented? I think that's what needs to be in our mind. So in childhood, there's these physical changes, recognition, the hunger and the growth aspects in adolescence, the body image concerns puberty related issues and diabetes that creeps up in a young adult. Pregnancy, work, pancreatitis, life struggles and in midlife, liver, kidney, heart protection and survival, they all come to the forefront. We do have some data that doesn't show up very well over here. There's increased mortality and shorter life spans and different forms different carry different mortality risk, in a generalized forms, especially in remote areas where there's not great medical access, early infections is an important cause of early mortality and then cardiovascular disease as a cause of mortality takes over. But all of the things that I have highlighted need to be thought about and preventing the next complication should always be on the minds of clinicians. And the society really.

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Treatment burden is also part of the disease burden. There's always, you know, we talk about our patients, about dietary vigilance, insulin injections, devices. Leptin is an injection, too, and look at the picture. There's very little subcutaneous fat, and we're wanting big volumes in these little spaces multiple times a day. So a therapy that lowers lab values but adds major daily burden may not exactly feel like success.

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There's all the devices to sort of monitor glucose when diabetes develops. Then there's the triglyceride number, liver management, there's procedures, imaging, monitoring. You go to a doctor's office and then the doctor gives you a whole list of referrals and tests to complete. And you know, you look at it and say, how, how am I going to fit all of this and life together? And then the big issue of insurance access you get in with. Can you get in with your doctor, will insurance pay? All of that uncertainty. So what should count as a meaningful benefit? So this slide sort of puts everything that I have mentioned and our patients, their testimonies will help us decide which of these benefits are most meaningful and when and what magnitude of change is worth the risk that we're about to impose with novel therapies. So I don't need to repeat everything over here. And I do want to mention because this I hear on a repeated basis from our patients, you know, we have all these medical goals that I have listed here, but also reducing the fatigue and also the pain should also come to the forefront as well. So today I urge you to listen for the differences and shared needs. The most important evidence comes from patients and families. And we, as the physicians and researchers who have given their heart to this disease, will never forget our why. So thank you again for giving us a purpose to work on.

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And thank you for giving me this time.

Maddie Pensiero:

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Thank you, Doctor Oral. I'd like to invite our first panelists speaking on Burden of Disease to join me on stage, and we'll begin with a video perspective shared from Kyleigh and Heidi.

Kyleigh: Hi, my name is Kyleigh and I was diagnosed with congenital generalized lipodystrophy around the age of 2 and this is my mom, **Heidi:** and I'm Heidi. So raising a child with lipodystrophy came with many different struggles. When she's hungry a lot, when your child has extreme fatigue for days on end, doctor's appointments, missing time from work even after a diagnosis. Even trying to find treatment from there was difficult. **Kyleigh:** Growing up with CGL, it was, for me I thought it was normal because as a kid it was Kyleigh's normal. But as I grew up and going into elementary school, I was getting tired faster than most kids, being hungry all the time, there's just, you can't concentrate. There's nothing that's fulfilling. You just eat, eat, eat and nothing is fulfilling that hunger. You can eat. **Heidi:** You're angry. **Kyleigh:** Yeah, angry. You're angry. And the more you eat, then my blood sugars were going up because it wasn't getting treated how it should have been. So anytime I was eating, my blood sugars would go up and then I'm still not getting full. So that's frustrating and it's angry and it was hard. It was hard growing up. **Heidi:** She ate different things than her older siblings because she had to get out there, but,

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As you know, eating healthy is expensive. **Kyleigh:** Socially it was really hard not fitting in getting those stares even when you're young as a kid or everyone's getting picked to do stuff and you're not getting picked. As that got older that turned into with not having the body fat, I had a bigger stomach with my liver issues and pancreas issues and all that. It then turned into.

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I looked pregnant and yes, I looked older, but I didn't look like an adult, so people questioned. Like why? Why is she pregnant? Why does she look pregnant? Then mom got judgment because her kid was quote on quote pregnant but I wasn't pregnant. **Heidi:** All of her appointments that she had, obviously that was. A hardship on the family. It was constantly back and forth to doctors, different surgeries, not knowing what was next. Saving all of your vacation time, all of your personal time, every year, year in and year out at work. Not to take vacations with your families, but because you might need that time

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for your daughter, which led into a as if we hadn't been through so much already in 2018, we almost lost her. We didn't know at the time it was pancreatitis, because she had written that out before. We had been to the hospital before. They give her an IV. She can't have anything to eat. Her triglycerides come down, her numbers come down.

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She goes home another month later, we're repeating the same process and she got used to just, you know, can I ride it out at home? Can I just drink lots of water? I'm just gonna lay here in bed. But as long as she's not vomiting, as long as you know, it's not too bad. She'd rather. I don't want you to take me. I just wanna lay here. And I was bringing her home from school and she told me that I should probably take her to the closest hospital. After so many times, you know there's only so much your body can take which turned into septic shock and respiratory failure. We almost lost her. Once you go back to work. Once life is quote back to normal if there ever was normal. It doesn't end there. Time to renew your leave where I could be able to take her to the doctor and it would be covered. I could not get the FMLA renewed so when I had to take her to appointments or things like that. I did not have that protection there, it was gone. So after almost 20 years with the same company, you have one day that you have to miss that you have no protection for and your job's gone. **Kyleigh:** When I feel like I'm the burden because.

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She's mom's always provided for us and then, you feel like you're, you are the reason you're the reason that she's not what that company anymore. You're the reason that we don't have a steady income anymore because I had to go to the doctor's. That's why it got so bad in 2018 is because I don't like to talk about when I'm hurting because I'm hurting all the time. But I don't want to, I don't want to talk about it. I don't want to burden anyone. I don't want to talk about my issues or that I hurt, so. **Heidi:** But she's not a burden. I mean, we, we all love her. And I try to tell her she's not, she's not a burden. It's, you know, the system, the disease, how it all moves in place together, the financial aspect of it. **Kyleigh:** Would any of this happen if we would have found out about my myalept sooner than when we did. **Heidi:** Maybe things could have been different. I don't know.

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Maddie Pensiero:
Thank you, Kyleigh and Heidi for sharing your testimony with us. Next, we'll hear from Julian.

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Julian Seida: I am Julian Seida and I have familial partial lipodystrophy type ACAA 2. So although I was only diagnosed five years ago, I've been living with this disease my entire life. Before my diagnosis, everyone called me an anomaly. When I was five months old, my parents noticed I was jaundiced. My pediatrician ran a liver panel and was shocked at how elevated the labs were. He repeated the test later that day. The next day I had an appointment with the head of the liver department in the Children's Hospital in Chicago. Over the next several years I went numerous needle biopsies and open liver and muscle biopsies. Despite running every test for diseases involving the liver, doctors in Chicago and Cincinnati could not figure out why my liver

continued to scar and become fatty. I was one stage away from cirrhosis, one doctor told my mom I had a liver of an old man as an infant.

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As a young child, I did not understand what was happening. Now that I am older, I often think about what my parents must have had to go through. And imagine being told that your child might not survive. Despite the uncertainty, my family never stopped fighting for me. It's been countless nights sleeping in the hospital so I could not be alone. They put their lives on the whole to take me wherever and thought the answer might be found. I spent so much time with my childhood traveling to hospitals. I became terrified of hotels. Never my family would pack for a trip. I soon had another hospital visit. I would cry and fight because I thought I was going to the hospital, but they didn't tell me what took me years to believe that they were actually not. Even as a child, I was very muscular. I also had asthma and EOE and used steroid medications. People would tell my mom that I was muscular steroids that she would be giving me. And no one realized the appearance would be of the disease. My brother used to joke around and call me the human garbage disposal because I would eat so much as a kid growing up compared to him as him being six years older. Even today people assume I am healthy because of how I look, but what they do not see is how difficult it could be going through a normal workout because sometimes my limbs will get sore and give out before I feel actually tired. During high school, my mental health also suffered severely because of this. I was very active and played two sports year round, but during my freshman year of high school that changed.

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I felt like I did not belong here anymore and the thoughts kind of kept staying with me for years. I eventually had to quit both sports because they're making my mental health even worse. That made me give up stuff that I have loved my whole life. And then 2021, after all the doctor visits for multiple years, I finally learned that the disease has been affecting my entire life. I felt happy and sad. I was happy because I had an actual answer of what was going on. But there was very little information because I was the very first person that had my variant. I was scared because I did not know what the future would hold for me.

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At the time I was in college living across the country from my family because of doctors who could have helped me were all so far away. I spent nearly every single break that you had from school going to multiple doctors visits in different states. Instead of having a real break from school, I was traveling for doctors tests and lab work. I was already exhausted from school and having to do this made it even worse. I even miss seeing all my friends graduate because I had to leave for the doctor visits the day that they graduate so I could make it to the appointments the next day.

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One of the most frustrating parts of living with lipodystrophy has been watching my disease progress while waiting for the quality for treatment. For years, I was told my numbers were not bad enough for additional treatment. I followed my treatment plan, monitored my health every day and did everything I could yet my disease continued to progress. Recently I developed diabetes. The hardest part is feeling like I must get sicker if I can actually get access to any treatment.

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By this time I qualify, some of the damage has already been done and it's irreversible. Living with lipodystrophy is not just about the abnormal lab values, diabetes, or liver disease. It's about the constant uncertainty about what comes next. It's the anxiety before every doctor appointment. It's watching your disease progress despite doing everything you could do. And it's knowing that every year without effective treatment can make everything worse.

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I'm sharing my story today because patients like me need better options. We need treatments that actually are available before all the reversible damage is actually done. We need the opportunity to slow the progression of the disease instead of waiting until the condition becomes worse. For actually qualify for help. I already have to live with this disease, I should not have to get sicker before I even need any treatment I need.

Maddie:
Thank you, Julian.

00:35:09.455
Next, we'll hear from Kristina.

00:35:20.951
Kristina:
I'd like to share a love letter to my husband, Eric. The one who weathers these storms with me.

00:35:28.370
Dear Eric. I'm sorry I've missed many mornings with you, gardening in the sun as our girls circle your feet. It's noon and I'm still in bed fighting to get up. As I opened my eyes it all starts. My body aches and my chest feels crushed by an 1000 pound weight. My arms and legs feel like lead, my joints are on fire and my head is throbbing. I wanna get up and be out there with you and the girls so badly, but my body has other agendas.

00:35:57.333

I try not to get mad at it but I'm already so exhausted. Finally, I muster enough strength to crawl over, pull the curtains open and collapse back into bed. At least from here I can watch the life we built, even if I'm stuck or trapped on the other side of the window. I'm sorry we can't go hiking or biking anymore. I miss tracking through the woods, hiking to the top of Bear Mountain and skiing the greatest snow on earth with you. I miss the feeling of freedom we had riding our bikes through the streets of New York, when we thought we could conquer the world.

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Those were our days. Now our adventure is a weekly walk around the block. And even that is a long shot. My legs and hips seize up and my lats are locked in a persistent tense spasm, like a bucking broncho trapped under my skin, kicking and kicking, kicking until I can't take the pain anymore. I'm sorry for the moodiness and irritability. Every second in my brain tells me I'm starving and dealing with this constant hunger is grueling combined with the weekly hours spent battling denials, researching treatments, finding doctors who have answers and fighting for survival is just too much and it's taken a toll on us. I'm sorry for all the debt and financial strain we bear. It kills me to see you tirelessly work at a job you hate, disregarding what it does to your own body and mind, just because we know the insurance policy will cover a fraction of what we need. I want to contribute and listen that weight you selfishly carry for us. I want to give you a break so you can pursue your dreams and we can start chasing ours.

00:37:37.907
But that's not possible because my body won't let it be. It stopped caring about keeping any kind of worker schedule a long time ago. And I know you don't want me to feel guilty about any of this. But I do. How can I not? Lipodystrophy doesn't just damage myself, it manifests in every aspect of our lives and slowly sucks the life and joy out of everything.

00:38:04.191
Out of me, out of you, out of us.

00:38:06.700
I can barely accept what it does to me. But I won't accept what it does to you and our family. But here we are again, with no treatment, fighting another storm. I'm 52 now so my symptoms will progress and my health will decline faster this time.

00:38:24.349
That means more pain, more fatigue, more hunger, more lipids kept around my organs. Our carefree sunny days once.

00:38:34.040

filled with fun and adventure will be replaced with rainy ones, filled with clouds of worry and guilt, where the overcast never seems to quite dissipate. So I'm sorry if our sunniest days are already behind us.

00:38:47.768
We know first hand this condition is so much more than a set of lab values or metabolic targets to reach. It is a slow, painful dismantling of our lives, our finances, our independence, our marriage. It is a relentless burden. An endless storm, continuously stealing life from a marriage that still has so much more love left to give. So many more sunny days to enjoy. A treatment could be our umbrella, our refuge to weather those storms, an anchor to give us hope, an opportunity to replace the string of apologies and guilt. With shared mornings and renewed dreams. I want to look forward.

00:39:34.535
I want to dream of our bright future. One where an umbrella, a treatment enables us to thrive together, not just survive. A future full of sunny days spent in the garden with you and the girls. And I will risk anything, anything to get some of those seeds back. And for that I am not sorry.

Maddie: Thank you, Kristina

00:40:06.984
Now welcome, Cori.

00:40:12.552
Cori:
I once heard a quote that deeply resonated with me. Being grateful and surviving doesn't mean I don't get to grieve what surviving cost me? Unknown. There have been many moments in my life this quote has applied, but one of the most impactful moments was when I woke up post surgery to my husband holding my hand and telling me he was so sorry with tears rolling down his face. It was in that moment I knew one of my biggest dreams was ripped away from me and one I still quietly agreed to this day.

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That dream of becoming pregnant again and getting to get my husband a child, Getting to be a mom. You see, we had gotten pregnant twice and lost both of our sweet babies due to miscarriages. We had tried. We had decided to try one more time with IVF for our rainbow baby. But due to all the extra stress and hormones that your body goes through during this process was too much for my lipodystrophy. My IVF treatment caused me to hemorrhage to the point I needed an emergency hysterectomy. And at that moment, post surgery, barely able to know where I was or what had happened, I knew. I knew after just looking at my husband's face that

my life was forever changed. That this disease took one of the most important things away from me again.

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And the only thing left was me feeling like I had failed. I wasn't enough. I wasn't a woman anymore. Heck, I didn't even have the parts of what made a woman a woman. It had won. Living with lipodystrophy can be overwhelming. Every day is a struggle, a balance to work, doctor's appointments, relationships, and the basic, basic life while running on an empty tank. Sometimes I feel paralyzed, by everything I have to manage in that single day. Some of the darkest parts of this journey have been the loss of personal relationships, the loss of my dream of becoming a mom, the loss of a career I loved. I had a job doing legal and contract work, and I loved it. I was good at it. I waited my whole life and I was so proud. But before my diagnosis with lipodystrophy, I was missing so much work for doctor's appointments that I eventually had to accept that a traditional nine to five in a major company might not be possible for me anymore.

00:42:25.878
That was another loss I had to grieve. Even when our numbers appear to be at their most stable, they can fluctuate rapidly and progress to a severe condition. And sometimes even do this silently without us knowing it's happening. On July 16th, 2026, this disease tried to take another victory lap. It tried to win again. On that fateful Thursday, I was being rushed into the OR to have emergency heart surgery. Unknown to myself, I was knocking on death’s door. You see I had been complaining for years 10 plus that my heart hurt. It really hurt. And that was my main symptom. I was even more tired than the normal lipodystrophy tired. And all my past cardiologists kept waving off saying it was PBCs or it's nothing, or you're too young to have heart problems. They never dug into my medical history, my lipodystrophy, or even my heavy family history of bad hearts. They kept telling me it was nothing

00:43:30.302
to worry about and to take this medication or that medication to control it. Or it was just PBCs. But it wasn't until a young PA really listened to me and looked into not only my medical history, my families, but also dug into a lipodystrophy can do to the body. And she ordered a test called CCTA. It's a very clear picture of your heart.

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And what they found out is that I was 99% blocked in my main left artery, AKA the widow maker. 90% blocked in my main right artery and 75% blocked in my back left. My heart was actively dying right there and then. As I was rushed into the OR lying on the OR table, my heart surgeon grabbed my hand, looked over. I looked over at him with tears rolling down my face, asking him to save me. Please don't let this disease win.

00:44:22.236
I can't let it win, he said. He said “I will do everything within my power to make sure that doesn't happen”. As of today, because of how long it went undiagnosed, my heart is now forever badly damaged. I will be facing constant lab work, testing. Nothing will be too small to be a red flag. And I will have to be on life saving medications for the rest of my life along with surgeries to come in the future. That is not an if. That is very much a when. On that July day, my life was forever changed once again, from facing the fact I was knocking on death’s door to having your doctor tell you your case humbles me as a doctor, but it also scares me as a doctor. To knowing my heart will be forever badly damaged and not knowing how I will have to move forward with the rest of my life and living out my days. Lipodystrophy is one of the cruelest diseases out there. Sometimes we know what we can expect

00:45:24.226
with it, and other times we don't. Sometimes this disease does and can win the ultimate prize, that being our life. Along with physical complications, I have constantly battled body image. No matter what anyone tells me, I've never truly felt beautiful. Before my diagnosis, I looked five months pregnant when I wasn't and knowing I can't be, it was deeply painful because it reminds me of what was taken from me. I have also dealt with the embarrassment of unwanted hair on my chin, upper lip and chest. I have even been asked if I was a boy just because my name is Cori. I will never forget being in Victoria's Secret shopping for a bra when a sales woman asked me how long I had been on hormone therapy and told me how brave I was for the journey of becoming a man.

00:46:11.335
I was mortified. I ran out of the store, got in my car, called my mom, and cried like I was three years old again. I felt disgusting, unlovable, ugly. That moment still lives rent free in my head at times. It still makes me question whether I'm beautiful, whether I'm worthy, whether people see me even as a woman. Sometimes it feels like there is not enough makeup or clothes to cover the things that make me feel different. I also lost personal relationships because of lipodystrophy. My ex-husband once looked at me and said I didn't marry you for you to be sick and deal with this for the rest of my life. I also lost friends who could not understand why I was too tired or had nothing left to give when I so badly wanted to. I became the unwanted sick friend and learned quickly to not talk about my medical journey.

00:46:58.497
People would ask how I could be so sick, but yet seems so normal. Between my medical life, private life, work, my current husband's back-to-back to back naval deployments, being far from family, and trying to manage a complex medical case alone, the pressure eventually led to a real and mental breakdown. I booked an emergency appointment with my therapist. After talking with me and seeing the state I was in, she asked if I would agree to a voluntary psychiatric hold.

I did. As I entered the ward and the thud of the ward door shutting behind me, I immediately went into an internal panic. What if everyone's right? What if it's all in my head? What if I'm being an uncompliant patient? What if I'm fighting for nothing? Even though I knew in my heart of hearts I was right, my body was yelling at me, heck, screaming at me for help, both physically, mentally, and medically. I knew I wasn't crazy. All I knew is that my body needed someone, anyone to listen to me and to take me seriously.

00:48:02.426
During those seven days in the psychiatric ward, I finally began to receive not only the medical help I needed, but also the emotional help I needed so badly for so long. If I can leave you with one message today, especially those of you in the medical and research worlds, it is this. You may be able to look at a patient and see their disease. You may be able to look at their labs like A1C, triglycerides, alts, LDL, CBC panel and even their scans like CT, MRI, and DEXA scans,

00:48:33.528
and get the clinical clinical information that you need. But there is so much more behind that patient standing in front of you. I still live in the balance every day, grateful to still be here, but quietly grieving what survival has cost me. But with the right treatments, the right care teams, and the right medical and research communities fighting for our future generations, my prayer is that this will cost them far less.

Maddie: Thank you, Cori.

00:49:12.354
Next, we'll hear from Becky.

00:49:18.161
Becky:
Hi, my name is Becky and my story is pretty different from many of the others. I wasn't born with this. I didn't have childhood trauma. I had a very normal childhood. Lots of friends, sports, college, marriage, a son. I was healthy and independent. And then in my 30's everything changed. When I was 29, after my divorce, I was diagnosed with aggressive rheumatoid arthritis. Over the next six years I cycled through multiple medications while relying on continuous steroids because nothing adequately controlled the disease. My final treatment was an immune suppressing infusion every three months. After a routine infusion, what seemed like the flu quickly became life threatening.

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Over the next six months, I developed diabetes, pancreatitis, meningitis, and severe fatty liver disease. I lost 74 pounds in four months and became so weak that I couldn't walk, eat, or even

crack an ice tray. I lost total hearing and required a feeding tube for six months. My best friend became my medical power of attorney because I couldn't manage my own medical care and my ex-husband even filed for full custody of our son because he thought I was dying. Although I survived the infections, my health never recovered. Years later, a nurse practitioner recognized the signs of lipodystrophy and after another year and a half of referrals, I was finally diagnosed with acquired generalized lipodystrophy. When I started researching the disease, I found very few answers. That was the first time that I was truly scared. I wondered, will this get worse? Will I be able to grow old? Is this going to kill me? Will I ever be able to care for myself? Everything I read said acquired generalized lipodystrophy rarely begins after age 30, yet my symptoms started at 35 and I wasn't actually diagnosed until I was 41.

00:51:08.683
Living with lipodystrophy effects every part of my day. Before accessing treatment, I was taking nearly 400 units of insulin every single day. While eating everything in sight just to try to maintain my weight, I'm constantly overheated, like living with a never ending hot flash because I have almost no temperature regulation. The loss of fat didn't just change how I look, it changed how I experienced my world. My skin feels paper thin when my foster dogs scratch me or even gently run their teeth across my hand while we're playing, it hurts far more than it should. I can't shave my legs with a normal razor because there isn't enough fat beneath the skin for the blade to glide smoothly and it just cuts me.

00:51:50.456
Every step I take on a hardwood floor feels like I'm walking on Legos because I have no cushioning on the bottom of my feet and sitting means sitting directly on my tailbone and kneeling on a hard floor is nearly impossible. The nerve damage in my legs that has made the nerve damage in my legs has made walking difficult. I can't lift my toes properly so I have to lift my knees higher to throw my feet forward with every step just to avoid tripping. If I lose my balance, I can't catch myself and often have to end up grabbing on to something or just falling. My calves also twitch constantly. It looks like bugs that are crawling beneath my skin. They cramp so severely that the pain is debilitating. And because my muscles never relax, neither do I. Months of physical therapy only provided daily temporary relief before everything tightened back up again. Eventually I was referred to a neurologist to discuss Botox injections and temporarily paralyze the muscles so they and I could finally rest. The fatigue is unlike anything I've ever experienced. Before lipodystrophy, I worked full time, cared for my son, kept up with the house in the yard, and still had plenty of energy to spend time with friends. I was the life of the party and five hours of sleep was plenty for me. Now I have to schedule entire days just to lie on the couch. I only get up to eat, use the bathroom, or let my dogs out. Sometimes I sleep all day and still sleep through the night.

00:53:20.085

And then my internal battery is only about 50 to 60% charged, even after a good night's sleep. I spread out activities across the week. If I don't, and if I don't finish something on the day I have it planned for, it may be weeks before I have enough energy to come back around to it.
My son now does much of the yard work and sometimes I have to hire someone because I simply don't have the energy. I cancel plans more often than I ever have before because I just can't keep up. I can no longer go on long walks or hikes because my legs fatigue much, much faster than the rest of my body. I also can't throw a few clothes into a backpack for a spontaneous weekend away anymore. Traveling now means counting out medications, packing refrigerated injections, protein powder and my glucose monitor. The cognitive burden has been just as frustrating. Multitasking is now a thing of the past.

00:54:12.997
The harder I have to concentrate, the more breaks I need because my brain becomes overwhelmed. It's incredibly frustrating because I know who I used to be. Then there's the hunger. I'm hungry all the time. I can eat a full meal and be hungry again 30 minutes later. Even on treatment, I carry snacks everywhere I go. Whenever someone invites me somewhere, my first question is whether food will be available so I know how to plan. When I was invited to this meeting, one of my first questions was, what's the snack situation gonna be? Lipodystrophy has also affected my reproductive health. I quickly went through all stages of menopause and later required surgery for two large ovarian cysts, a vaginal prolapse due to weakened muscles, and ultimately needed a hysterectomy. I still have night sweats so severe that I drench the bed every single night and no doctor has been able to tell me why or treat it. I could write a book on all the things that I can't do anymore, but I have a time limit here. Metreleptin has helped with my metabolic numbers, but it didn't give me my old life back. I still receive Botox injections in my legs. I still live with overwhelming fatigue, cognitive challenges, and constant hunger. New medical problems continue to appear with little warning, and they often escalate so quickly that I need emergency appointments or surgery within days, which also causes me to not be able to work anymore. What scares me most now is the uncertainty. Doctors often don't know what to do with me because there simply isn't enough research. I dread going to the emergency room because they see an ordinary patient, but my body is anything but ordinary. I still have more questions than answers. Will I continue getting worse? What happens next? Will anyone ever know how to treat all of these complications?

00:56:03.133
So I take life one day at a time, and every morning I wake up and thank God for another day. But still hoping that someday people living with lipodystrophy will finally have the answers, treatments, and research that we desperately need.

Maddie: Thank you, Becky, and thank you to all of our amazing panelists for sharing your stories, you may take your seats.

00:56:36.642
Before we begin our first discussion focused on the burden of disease, let's use some polling questions to get a sense of who's here in the room with us and joining via Zoom. Just to emphasize, polling questions are for patients, caregivers, and family members only. These first few questions are focused on demographic information. To get started, you can scan the QR code that you see on the screen or visit slido.com and input the code 91218369. This first question asks I am…

00:57:20.960 -
A person living with lipodystrophy, a caregiver, a family member or other. And folks joining us via Zoom, we encourage you to participate as well. And hope that you will. I'll give a few seconds for responses to come in. Great.

00:57:51.449
Thanks everyone. I see we have a few other respondents. Just as a reminder, polling is intended only for patients, caregivers and family members. Thank you. Let's go to our next poll. And these polls will remain open, so if you miss one or it's not loading, you can return to it throughout the day via the same link. Our next question asks, what type of lipodystrophy do you have? What is your or your loved ones diagnosis or subtype? I'll allow a few seconds.

00:58:32.642
Andra:
Yeah, these numbers, Maddie, look about right. We know that there's in terms of prevalence, there are significantly more individuals living with familial partial.

00:58:44.147
than generalized. So that feels representative. We're really excited to see that we've got representation from all types here and the unsure and other are also not uncommon. We're still learning about lipodystrophy, we're still identifying mutations and some patients are diagnosed but are never really entirely sure of their type. So these numbers make sense to me.

00:59:10.469
Thanks, Andra, and thanks all for participating. Our next question asks. What is your age or your loved one's age category? Again, we're asking about the person with lipodystrophy.

Andra: As these numbers are getting put in, I want to also add some comments. The 35 to 49 also really makes a lot of sense. Most types of familial partial are genetic and so therefore this isn't an adult disease but shows up often as an adult disease because folks take so long to get

diagnosed. This is a childhood disease that progresses over time and a lot of folks don't get diagnosed til a little bit later in life. Generalized often get caught

01:00:12.287 --> 01:00:20.048
younger diagnosed, earlier and I am really glad to see we've got some folks of that that surpass the age range here. So it's great.

Maddie:
Thank you. Our next question. Where do you live? Where are you joining us from today? Great.

01:00:38.392
Great to see so many folks joining us from all over, especially those in the West and internationally. Thank you for being up early with us today. We're grateful for it.

Andra: We are grateful for the AI transcription and thank you trans perfect for the translation. Hopefully that's working for you. Well, thanks for being here.

01:00:56.652 --> 01:01:02.065
Maddie:
Our next two poll questions are going to focus on setting the stage for this open discussion on the burden of disease. This is a little different. This next one we're asking you to input a word. One word. What is it like to live with lipodystrophy?

Andra: It's a tough one. There are lots of words. But we are looking for the thing that comes to mind the most. And if you've never used Slido before, I'm going to call out that exhausting is large because that means a few people are putting it there. So Slido is that ability to recognize the same word.

Maddie:
I see exhausting. Lonely. Debilitating. Overwhelming. Uncertainty. Tiring. frustrating. So many more. Hellish. Thank you all.

01:01:59.336
Our last polling question for this section of the discussion asks if you could wave a magic wand, what is one symptom you would remove from your disease? There's also an other option you can write in something if it's not captured among the options that you see on your device and on screen.

Andra: I know that you're all thinking all of the above, all of the above, but please, we'd love to hear just the first thing that comes to mind for you all. The one thing that impacts you the most.

01:02:37.234
Maddie:
Seeing a lot of engagement around extreme fatigue and lack of energy as well as pain, with a lot of range across these options. Thank you.

01:03:01.844
Andra:
As Maddie mentioned, the poll is still open and we will release the final results after the meeting. So if you skipped one and you need to get back to it, go ahead and do that while we move on to the discussion. Thanks, Maddie.

01:03:17.486
Maddie:
And we'll return to polling later in the day. So feel free to keep this link open. It's going to remain the same link as we continue the discussion. So now let's move into that discussion about the burden of disease. A few reminders. Please keep your responses specific and short to these questions. Two to three sentences. We know that's tight, but it'll enable as many voices as we can to participate. And Andra and I may ask follow-ups. If you're in the room, we ask that you use the microphone. Raise your hand for the mic runners. You'll see them along the edges. In Zoom chat, type the comments that you would like to be shared aloud. We ask that you start your response with your lipodystrophy type or your loved ones and where you're from.

01:03:57.678
And again, to our drug developers, researchers and clinicians, welcome and thank you again for listening and being here. Today's floor belongs to the patients, the care partners and the family members, and we hope we hear from you. Let's begin with our first question on the burden of disease.

01:04:17.478
What aspects of your lipodystrophy condition or your loved ones' condition do you find the most bothersome?

01:04:24.574
We have a hand out front.

01:04:29.983
Becky:
Hello FPLD 2, I'm from the UK. Before treatment my most bothersome aspect was hunger. The hyperphagia is absolutely unrelenting. For people in the West, starvation is not something that

they ever experienced. The level of hunger is not something they can comprehend, but it is starvation level hunger. No matter how much you eat, you're never satisfied, and it is all consuming. I'm fortunate enough to be on treatment that is massively helping with that, and so now my biggest difficulty is definitely fatigue. Again, it is massively impactful day-to-day activities. Mostly I work and sleep because I haven't got the energy to do anything else.

Andra: Becky, can I ask a follow-up question? Can you describe since you experienced that hunger before and now treatment is helping with that, can you give an example of what that starvation felt like and can you describe the burden you experienced in a typical day with that hunger?

Becky: Yes. So it's very difficult because growing up that was my normal. So I didn't realize that it wasn't normal, but as an example. I would go out and have a three course meal and on the way home, I'd stop somewhere and get a burger. I was still hungry. I'd feel so hungry. It felt like my insides were turning inside out. I'd feel sick, in severe pain. And what happens is it becomes an obsession because all you can think about is food. Where's my next meal coming from? Where's my next snack coming from? And you get kind of panic anxiety about having access to food. And as some of the panelists already mentioned, you know the first question is where's my next meal coming from? If there's an event that somebody's planning, what's the food situation? And it's, it's all consuming. It really does take over and you become very obsessed with food, which is actually really difficult because you're not enjoying eating, it's just that you're in pain. And it is, it's just, it's all consuming, yeah.

01:06:56.896
Maddie: Thank you. Thank you.

01:07:00.090
Let's go to the back.

01:07:08.288
Kenzo:
Hello OK I have CGL AGPAT2 so for me going off of what you said too, it's absolutely the hunger. Being younger my mom would always say we would eat her out of a house and a home because she would buy groceries and as soon as all the groceries were brought we were already eating them. I say we because I have a younger sibling who has it as well. She's not here. But even with being on treatment, while the treatment does help.

01:07:38.398
It's still very much. A real thing that hunger is still always there. Like I'm always hungry, always thinking about food. I can eat meals back-to-back and be completely fine until my stomach has

completely blown up and now I'm extremely bloated. So then it's painful. But it's also one of those things that I get angry. I'm one of those people like hangry that's me. Because like when I want the food and I can't get it, I am automatically upset because that my body is literally telling me that you need to eat, you want food right now. So that's been bothersome for my family and now even my partner hears me all the time. I'm hungry. What am I gonna eat? Like it's constant.

01:08:21.077
Maddie: Thank you.

01:08:23.375
Andra:
Yeah, my nickname was Tasmanian Devil growing up, and anyone in the room with lipodystrophy knows exactly what that means.

01:08:37.519
Shannon:
Hi, I'm Shannon. I live in Wisconsin. I have familial partial lipodystrophy and for me the most bothersome aspects are the intense pain and the chronic fatigue. And both of those battle each other in my body, so the only escape I get from pain is sleep. And yet I can't sleep, so it's a constant battle within myself and the pain is daily. By the afternoon my body is screaming on the inside and there's nothing I can do about it. I've had failed treatments and medications don't work for that either. So.

01:09:28.250
Andra:
So hunger is hard to manage, but pain, I think we all collectively know when you go to the hospital and they ask zero to ten, can you give us a number of a typical day?

01:09:39.277
Shannon:
Sure. So I wake up in pain probably at three-ish. By early afternoon I'm at like a six, and by the time I go to bed I'm probably close to an eight on most days. Usually by the time my husband gets home from work, I am too tired and in too much pain to do anything. I'm sitting on the couch. I can't have a conversation. I can't cook a meal.

01:10:04.273
Maddie:
Thank you.

01:10:12.071

Serj:
Hi I'm Serj, I have FPLD type 1.

The thing that I find most bothersome is, is also the extreme pain and, and the fatigue. Umm, I end up spending one time six months in the hospital and you know, months and months in the hospital all the time. Uh, and I end up in my house bed bound for weeks and months. I can't do anything. I have my partner here with me last night. I was so overwhelmed with coming here that I ended up having seizures and the pain was so severe. I mean, what do we do? As you were just saying, the only reprieve from that is to sleep. And sometimes the pain is just so severe. You wear yourself out to the point. And this is what happened last night for me is you wear yourself out to the point you're in a fugue state and you just pass out and you don't remember passing out. And then you wake up and you're still in pain and it's like it all just starts all over again. And I don't know, I guess I live at about a five. And again, you go and tell people that and they're like what? Like an average doctor and they think you're a liar. And I deal a lot with providers thinking I'm med seeking and it is. It has been such a disaster, such a disaster in my care since I was very young. The pain has been an issue since I was very young, like since I was 9 I started having complications with my gallbladder, but of course I'm a liar. You know, none of this is real and that's been devastating because how does one deal with that?

01:11:59.731
Andra:
We definitely hear that a lot about drug seeking. It's a really common experience across all the types. Thank you Serj for making the trip to see us. We know it is really hard on the bodies.

01:12:15.263
Lori:
For me as a mom, well, Julian's my son and he has partial lipodystrophy. For me, it's the stress and anxiety since he was five months old. Doing it as a mom and seeing your son suffer through severe liver diseases and a doctor told us he may not survive and seeing his condition progress. It's difficult to watch your young son, who's 25, spend days in bed sleeping or wanting to eat. And just the fatigue, he could sleep 12 hours and get up, eat breakfast, go back to bed and that's difficult.

01:12:57.899
Maddie:
Thank you.

01:13:00.059

I want to invite too. We saw a lot of different symptoms and complications on that survey and a lot of engagement with each. Does anyone want to share anything about a symptom that has not been mentioned yet or an aspect that has not been mentioned yet, yeah.

01:13:17.822
Christine:
Hi. I'm Christine, I've got Familial partial and neuropathy, the numbness in my legs. My intestines. It's catastrophic. A year and a half ago I dropped a 20 pound rock on my foot and I didn't feel it. I was barefoot. I had socks on. It wasn't until I pulled my socks off the next morning that I saw that my toes were all black and blue. The neuropathy is so bad it has caused extreme constipation that can go up to three weeks where no medicine has been able to help that. And if it does, I don't know when it's going to take effect, which is quite troublesome as well. You know, you, you kind of wanna know when that's gonna hit.

Maddie:
Christine. How does that affect? Like how has that particular aspect affected you? If you can give an example.

01:14:29.017
Christine: I don't wanna leave home, work. You know I work full time so I can be at work and bam, it hits and I've had to go home early because I've had accidents at work and it's embarrassing. You know, I, I can say it in front of all of you because I know a lot of you have the same issues, but work doesn't understand that.

01:14:55.595
Maddie: Thank you.

01:14:59.280 --> 01:15:00.842
Andra:
Rowan, do you have anything back there?

01:15:03.382
Maddie:
We are going to take a comment from our online audience.

01:15:06.294
Kate:
Hi, we have a lot of zoom interaction. So many people have shared that extreme hunger and pain have been very burdensome for them, including fatigue and tiredness. And then I'll, I'll read a couple fully. Nikki shared the inconsistency of symptoms makes it impossible to plan a life.

Keeping plans is impossible. Whenever I get rare bursts of energy, I can't trust that I'll get that energy again anytime soon. So I frantically try to get done whatever tasks I've not been able to do and then pay for it by being extremely exhausted to the point of uselessness for days or weeks after. Kyleigh shares that hunger. Kyleigh was the one that we saw her video. She shares that hunger before treatment and with treatment and the mental struggle has been really hard. Stress and anxiety causes havoc with everything metabolically. Dena shares as someone with atypical progeroid lipodystrophy, my symptoms differ from many with FPL and CGL. When metreleptin stopped working for me, there wasn't an opportunity for new drugs yet. Without working treatment, my A1C shot up, as did my triglycerides and cholesterol. I was diagnosed with coronary artery disease, artery disease and had a CTA scan that found Non obstructive blockage in my LAD and my CAC score was elevated to 109, normal for my age is 0. Being put on mibavademab was such a relief as my metabolic labs have returned to normal or at least safe range but it does not undo the damage done.

01:16:52.052
Maddie:
Thank you, Kate, and thank you everyone for joining us online and for sharing your perspectives here and in the room. We're going to move on to our next poll question. So hopefully you kept kept those devices close at hand. We'd like to know how much does your condition, or your loved one's condition as applicable, interfere with daily life, specifically in the last 12 months. Again, that same link slido.com code 9218369. Seeing a lot of responses in the significantly and extremely categories.

Andra: We do know like all rare diseases, there's a spectrum of disease and so seeing the spectrum from moderately to significant. I would like to call out that everyone is participating today. We haven't, we have 0% at not at all minimally.

01:17:57.848
Maddie:
So let's dig into this with our next discussion question.

01:18:01.934
Are there everyday activities or responsibilities that you have to do, but cannot do as fully or as easily as you would like because of your condition?

01:18:21.352
Kenzo: Hi, Kenzo again. So for me, my everyday activities, I was a person that was like always working out. I'm very active. I'm a PE teacher. So having that like taken away from me because of certain things that I'm dealing with on the lipodystrophy side, like my heart, for example, I

can't lift weights like I used to. I used to lift weights like daily and now I've had to cut that out altogether and keep with like body weight things, which that's not saying that it's something bad.

01:18:50.447
But when you really enjoy the lifting weights and then it gets taken away from you, it makes a difference. So that brought a mental turmoil for me because I've always done that. And so to have it just, we need to not do that anymore. It was detrimental.

Maddie: Thank you, Kenzo. Other responses.

01:19:09.650
And again, encourage those who haven't shared yet to join us in discussion.

01:19:18.516
Mallory:
Hello, my name is Mallory. I have familial partial lipodystrophy. My favorite activities to do are biking, hiking, swimming. But because I don't have any fat on my feet, recently I had a bone infection in my foot and I wasn't able to do any of the activities that I like and also have diabetic ulcers on my feet and I just can't be as active as I like and it's really hard for me to not because I just love being outside and doing things.

Maddie:
And Mallory, those activities when you can't do them, what does? What does that feel like?

01:19:57.640
Mallory:
It just feels like I'm not living my life. Like it just feels like I'm just here and I don't know. I just, it's, it's not fun at all. I'm bored out of my mind just sitting at home, but there's nothing I can do. So it's just, I'm stuck.

01:20:15.569
Maddie:
Thank you.

01:20:24.625
Briana:
Hi, I'm Briana and I have familial partial lipodystrophy. I'm from Montana. I've always been incredibly an outdoors type person because you know you live in Montana. Due to pain, fatigue, losing the fat on my feet and the absolute fear of being out in the middle of nowhere, to not be able to get medical care because I have had four heart attacks. We don't do those things anymore.

And when we do, we have to be close to somewhere where I can get help. This is not something that we ever thought that was going to happen to us. And because of my fear, my children knew that I love this so much that they bought me a defibrillator so that I had it just in case, but it still doesn't take the complete anxiety away for being out there.

01:21:27.277
Maddie:
Thank you. Thank you. We'll take one more response.

01:21:33.458
Jennifer:
I'm Jennifer I from Pennsylvania, and I live with familial partial lipodystrophy. Like everyday tasks sometimes are hard, especially those ones like where you have to stand in one spot 'cause I get like debilitating leg cramps. Like if I'm standing at a sink doing dishes, I get cramped and can only do it for so long before I have to rest or take a walk around.

Maddie: Thank you. Our next question is gonna build on this.

01:22:04.176
How do symptoms affect your social life and relationships? Again, encouraging folks that haven't haven't heard from yet.

01:22:18.369
Kara:
Hi, I'm Kara and I have familial partial lipodystrophy. I would say it's the being self-conscious of being aware to also up the dark spots around your neck in your armpits. I avoid what I wear or I really look at it because I don't want people looking at me. It's making sure I have something flowy on so I don't get asked if I'm pregnant after I've had a hysterectomy. It's not wanting to have sex with my husband because I don't like how I look. So losing friendships because I don't understand what it's like to be tired all the time or not have the energy or having an outburst because you're just mad or whatever. It's also having to explain it over and over again and it gets tiring. So I try to avoid a lot of situations. I'm very much an extrovert, so I love being around people, but at the same time I also hate it.

01:23:46.660
Maddie:
Thank you.

01:23:50.531
We'll go to Crystal.

01:23:57.447
Crystal:
Hi, I'm Crystal. I have familial partial and I just in general keep people at an arms distance. I love meeting people, I love talking to people all the time, but, I will keep you at a distance because if I get too close to you then I might have to explain and there's just too much explaining. And it's exhausting.

01:24:27.464
Andra:
Crystal. Recognizing you don't want to explain, I'm going to ask you, if you could explain what are you avoiding explaining what explaining would you? What would you have to do?

01:24:43.461
Crystal:
So I love making friends but I can't hold a relationship because I always have to bail out. The inconsistencies and how I'm gonna feel is drastic. It's drastic and it can change from day-to-day or even hour to hour. I could wake up feeling really well and ready to conquer anything but then in two hours, I can't move, or I need to revert or I need to adjust. So if I keep you at arm's distance, then I don't hurt your feelings.

01:25:21.503
Andra:
Do you want to talk a little bit about your social life and the people you keep at a distance? Do you want to talk a little bit about your family and your marriage and how that's impacted that?

01:25:34.084
Crystal: My husband is very supportive and he is my rock but it got, at my worst state, it got to the point where he had to take vacations on his own and I had to stay back. And it's not because I didn't want to go, it's because I couldn't stop him from living his life.

01:26:01.493
Maddie:
Thank you. Do we have any responses from online? We'll take one.

01:26:14.536
Andra:
Kate, before you start, I'm going to remind folks we will put up in a little bit a QR code that goes to a docket. So if you're unable to get called on and you want to share you have something additional. We do have an open docket and there's no limit. So you can add to the docket

throughout the day. If you go home and you say, boy, I sure wish I'd said that or I thought about this, you can add to that docket. That docket will be open for a few weeks and then it will be included with the voice of the patient.

01:26:45.943
Thanks. Go ahead, Kate.

01:26:48.448
Rowan:
Yeah, we have a lot of responses online showing the same relation. Melody has shared that I no longer have any friends. I don't have the capability to be a good friend and hold resentment that others don't understand what I'm going through. And one more Trish sharing that my social time is best early in the day, my mood is low at times, I get quiet and my world is very small because of the same lack of understanding.

01:27:16.933
Maddie:
Thank you. We'll move to our next question.

01:27:22.159
When your lab values such as triglycerides, blood sugar or A1C are considered stable or well managed, what symptoms, limitations or day-to-day challenges do you still experience, if any?

01:27:39.744
Becky:
Thank you. FPLD2, I'm from the UK, so it's great when all your blood levels look good, but that's just scratching the surface. So for me mainly it's fatigue. So I really, really struggle with fatigue, as I mentioned earlier. But in addition to that and kind of expanding on what some other people already talked about, it's the body confidence, body image. So everybody is impacted by this, but it's particularly burdensome for women because to have muscles as a man is less unusual. And you know the number of times I've also been approached and asked if I'm trans. And it's really, really painful. And actually, you know, the same as any other woman, we want to be considered as a woman and I used to cover up, so I used to wear long trousers, wear long sleeves because it is easier than having to explain. And in fact, my mother, bless her, out of devotion to me because she didn't want me to be hurt would encourage me to cover up and actually that's the wrong thing because I shouldn't be ashamed of who I am and what I look like. And I've come to that realization. Sometimes I've got the confidence to wear what I want to wear and not care what people think, but it's still painful and sometimes I can't. I haven't got the kind of mental capacity to be able to deal with that. And so I will cover up but that is something that really bothers me on a day-to-day basis. And people are unkind, generally speaking, in society,

we have such a focus on the way that people look, which is not healthy generally. But for people who are suffering with lipodystrophy it's an extreme version of that. And people can be very, very unkind. And so I think the mental burden of this condition as well as dealing with all the medications, all the kind of lifestyle challenges, the kind of day-to-day management, your diet, your exercise, etc etc. On top of that, you know, grieving for who I could have been or feel like I could have been without this condition and what it's taken away from me, on top of feeling that I'm not feminine and I don't look like a woman. It's devastating.

Maddie: Thank you. Even when the labs are good. We'll go to Cori.

01:30:17.352
Cori:
So I know y'all just saw me, but even when my labs were at their most stable point probably than they've ever been like sticker worthy, I got to hang it on the fridge kind of labs. That's when I had my heart problem. And so this disease is cruel in the sense and day-to-day challenges of am I good? Am I not good? Like you say, I'm good on paper, but I obviously I was not. And then you're being rushed into the OR before you know it. This disease, I call it a thief in the night. It truly is a thief in the night disease. So I think that's one thing I want people to understand is, even though everything looks beautiful and they are sticker worthy and you can put them on the fridge, it doesn't mean that there is so much more happening behind those doors.

01:31:11.196
Maddie:
Cori, can you give an example of maybe what some of your numbers look like in some of these areas even as you know this was transpiring for you?

01:31:21.099
Cori: Before all this started, my triglycerides were in the thousands. A1C was 13, 14 like I was rocking that and then blood sugars were staying around two hundred and fifty close to three. Like I just couldn't get them to come down. And then on my last trip to NIH, I got multiple stickers from Dr. Brown and sticker worthy moments and they were at 218 triglycerides. A1C is 5.9 or you know, something below the average and sugars, I'm actually having too many lows now so even when I was at my best, there was so much happening behind those doors.

01:32:08.030
Maddie:
Thank you.

01:32:10.636
Andra:

I had no idea Dr. Brown gave out stickers and I'm going to be waiting for them. Back here.

01:32:19.321
Robin:
Hi, I'm Robin and this is my husband Russell and he has FPLD and he would speak but he'd cry so I'll do it. I would say his triglycerides have been under control since the very first dose of metreleptin. His triglycerides went from two thousand five hundred to three hundred in just two weeks. So the labs have been pretty stable since 2014, but the damage had already been done 10 years prior to all this. And so I think our challenges were so his kidneys did fail and he did have a kidney transplant. And so the fatigue and the hunger that's just there even when your labs are good. And the damage to the kidneys of course results in the transplant, which result in immune suppressant drugs, which limits you, you know, where you can go and things that you can do. You know, you can't eat a buffet. You can't really. We're scared to travel out of the country or too far from doctors, you know, that kind of thing. And then you know, there are certain things that you can't eat. And then of course, managing your blood sugar that you're, that's a constant battle between you know, being hungry all the time and eating too much and everything spiking up and coming back down. And so those are the limitations that he has from day-to-day.

01:33:44.529
Maddie:
Thank you. We're going to move to our next question. What does a good day with lipodystrophy look like? Alternatively, what does a bad day look like? And has your experience of good days and bad days changed over time? We recognize there's a few questions built in here, so whatever is speaking to you, we want to hear about.

01:34:08.546
Yeah, Serj

01:34:11.315
Hi Serj again, a good day for me is I'm able to get some sleep. And I wake up probably at noon. And uh, I was able to run my tube feed overnight and uh, was able to get at least half of it in and don't throw up in the morning and then I don't throw up throughout the day, hopefully umm and I remember to take my morning meds and I'm able to hold those down. But then I'm in bed after I drop my partner off at work and I'm able to pick up some stuff around the house but I am in bed. That is a good day. I am not able to do much. A bad day is I have been in the hospital for over a month already and I am on palliative care, copious amounts of comfort medication and hardly lucid, and uh, fully feeding intolerant. My jejunum will not allow me to feed into it at that point. The pain is excruciating and I feel like I'm dying at that point and at some days, I do come close. And it's scary.

01:35:39.207
Andra:
During Voices of LU, the research study before that we did in preparation for this, I remember one quote was “I'm at my best when I'm in bed”. That reminds me of that.

01:35:50.730
We've got. We've got a few hands that we've heard. There's anyone else that hasn't spoken yet that wants to share good day, bad day and then we'll go to. Sharon.

01:36:17.647
Hi, FPLD type one. For me a good day is being able to get 5-6 hours of sleep and then it takes me about an hour to get out of bed and this is a good day. In a bad day, just recovering from sleep, which sounds weird, takes a couple of hours just being in bed awake. Getting all the meds in and just starting the day and it's only getting worse and worse. I mean, the disease is progressing. It's harder and harder to start the day and get anything done. Not thinking about even going to work now. It's impossible for me and just thinking about the last question, it doesn't matter if my labs are good or not, the fatigue, the pain, the mental issues, the depression are always there. And this is something that no treatment can help with right now.

01:37:22.043
Maddie:
Thank you, Sharon.

01:37:26.650 -
Rowan, Kate, do we have anything that is different. And certainly if you feel the same, we want to hear it. You can add it to the docket. So we're not discouraging people explaining that they have those similar things, but looking also for a variety. Go ahead, Kate.

01:37:47.961
Kate:
Hi Billie says as a mom it is hard to know just how much she can endure her daughter I presume with lipodystrophy. When she comes home to visit she has her twin 9 year old niece and nephew who want all her attention. She spends her time with them. And then when she gets home to visit with me and dad, she sleeps. I never asked to do more because I need to be sure she has the energy to drive 4 hours back to her home.

01:38:16.680
Maddie:
Thank you. Crystal, did you have a hand? One second hold for the mic.

01:38:34.428
Crystal:
So currently a good day looks pretty amazing for me when I wake up, I see a little Unicorn on my meter with good glucose levels and all systems are ago and I'm ready to get out the door. But bad days can change because I don't know if I'm gonna have a good absorption day or adjusting to new treatments. So I don't, I don't know exactly how my body's responding. I've gotten better at judging it, engaging in what I can do, but sometimes I have to turn the car back around and go right back home. And I think with the changes in my treatment plan over time. This is the best I've ever felt. And I can do the most now.

01:39:35.994
Andra:
What does the most look like? What does that mean the most?

01:39:40.779
Crystal:
Anything I want to do? Getting up, going to the store. Then not having to worry to go back home quickly, maybe even stopping to see my mom. It's cool. It really is.

01:39:58.366
Maddie:
Thank you. We'll come to Gretchen.

01:40:02.893
Gretchen:
Hi, I'm Gretchen. I'm from Pittsburgh, Pennsylvania. Familial partial, I have no idea what type. My bad days usually happen after my good great days because you just spend so much energy doing what you love to do and being with the people you want to be with. You just spend everything you have and then recovery is days long and that's something I don't think people understand for us. I could do whatever you need me to do, but the recovery is what's difficult,

Maddie: Yeah. Can you say more about that recovery, what that looks like, how long it takes?

Gretchen:
It just really depends on how active, you know. My family tends to tease me because a drive to Michigan exhausts me. And they're like you you literally sat in a car. Yeah, I sat in a car. But I had to fight to keep up. And my neck hurts from this and my back hurts and yeah, so the more energy I spend one day, the longer it takes me to catch up. There's days after that I don't do anything.

01:41:13.986
Maddie:
Thank you, Gretchen. Other comments. Do we have any more online? No.

01:41:25.762
I do want to call attention to this last part of the question about how your experience has changed over time, Kristina, did you have something? Hold on. One second, one second, one second.

01:41:34.404
Kristina:
Thank you. I just wanted to kind of play off what Gretchen said. It's like some days I feel like there is a consequence to end everything you choose, right? Like if you choose to use your energy in do the things you love to do, you pay the price. So you either pay the price in days before or you have that burden of you know, disappointing the people you love, cancelling on your friends, not being able to go to work. So there's always a choice we have to make and it feels like either one always sucks.

Maddie:
Kristina, can you share about what that looked like for this meeting, for example, to be here today.

01:42:24.698
Kristina:
Ask my husband, he how long have you been doing everything? for the last two weeks 'cause it's. You have to prepare before to make sure your body is ready. You can have to do everything you can possibly do 'cause you know you have to do something. So it's the pressure, it's the unpredictability. I was panicking last night, 'cause I'm like, I'm hoping I don't feel like, like I don't have a headache tomorrow. I hope my body feels OK. Like you never know how you're gonna feel. So it's so unpredictable to plan anything or know how you're gonna feel because I feel like there's always some sort of disappointment there for someone.

Eric:
My name is Eric and I'm Kristy's husband. And just to add one point to that, these patients with lipodystrophy, they're wanting to give value. They want to work, they want to live, they want to play and twice now in the last five years I've seen Kristy step up to the plate at work, take on something. One was a new store opening for a company, in which she worked 80 hours a week for two weeks, and then got sick for three weeks. And was fired the day before Christmas.
One was last summer as well. Where Kristy stepped up, took on a project at work. And then had to miss two weeks. And also lost her job. I just wanted to put those numbers out there.

Maddie: Thank you. Thank you for adding that.

01:44:03.859
Andra: Maddie, before we move on, I'm going to do something I'm not going to do much during this day and that's to take off my moderator hat and put on my patient hat.

Maddie:
Please, Andra

Andra: I will share that. I want to emphasize what's changed over time. I would say when I was younger before treatment, I had very much the same experiences and I would be absolutely exhausted and an advocacy trip would require two to three weeks recovery and now on treatment while my disease has progressed, some of my numbers don't look as good. With the treatment, I'm able to take those big trips and then recognize I just need a day or two of rest and then continue on. I have a high pressure job that I love and I'm able to do those things and balance activities like this. So even though some of my numbers and metabolic has progressed. With treatment I'm actually able to do more.

01:45:11.125
Maddie:
Thanks, Andra. I'm gonna ask you a quick follow up. What does it look like when you're not able to get that rest, that recuperation?

01:45:19.415
Andra:
Well, one of my biggest challenges is my immune system. And so if I'm always on the edge and so if I'm not able to take the edge of of an illness, So if I'm unable to take the rest to work just a day after a day or two, or three, depending on how much energy I've put out, then I will absolutely end up with whatever virus is within 5 miles range of me. But I'm again able to stave those off much better on treatment than I did before. Yeah, it's not perfect, but it's a, it's a balance. And then a virus means, you know, a cold. I'm really, really sick, right? Or I'm in bed for several, several days. It takes a while to catch up. So it means a lot of missed work and missed family time, yeah.

01:46:11.048
Maddie:
Thank you. Do we have any comments online? We'll take one and then we'll come to you in pink.

01:46:22.929
Kate:
Zach is a husband with lipodystrophy. Yeah, husband with a wife with hypodystrophy. I see my wife's daily struggle every day, not just physically but mentally. I watch her everyday fight just to keep pushing forward when she has nothing left. I see the energy collapse daily, the fight to just keep it together in front of her children. I see her consistently missing out on events and normal things. I see her insecurities rule her life. This disease has taken so much for her, just to live a normal life that she deserves. Every day is a struggle for her, whether it is lack of energy, mental health struggle, or any of the other various struggles that this disease brings her way. It's hard as a husband to watch this defeat her, but to also know there's isn't anything I can do to help her from what she's struggling with.

01:47:14.164
Maddie:
Thank you so much for being part of the conversation. We'll take one more comment.

01:47:22.196
Ananda:
Hi my name is Ananda and I have a partial familial. So what a good day with lipodystrophy looks like for me is. Well, feeling like I'm satisfied. That what I'm waking up to to eat that morning is going to be actually helpful. And that I've slept throughout the night, actually slept throughout the night without waking up multiple times or having nightmares. Having to use a bathroom multiple times. And actually achieving. I mean I wrote down 8 hours of sleep. But I can't remember the last time I did that. Having my house clean. I do have three children. I am a single mother, but waking up to knowing that my home is clean and hearing from my children like this is wonderful. Oh my God, the house is so nice. Mommy did such a good job. That is really impactful. That makes me feel good. Seeing that my children are happy. And having snacks in the fridge that I've been able to make for them because I had the energy to do that because they're hungry too, that is really impactful to me. That's important. And waking up not feeling in incredible pain. The burning in my arms. Every day. I mean, this is almost like a magical day because like, I wake up every morning and just to brush my hair, my arms feel like they're on fire and the medicines that have been offered to me have failed. So I really live without many options even as supplements like most of the times that I when I'm able to afford all the things that I'm trying to take to help myself feel good.

Maddie: Thank you. We are coming to the next question. But again, encourage everyone. Someone who is sharing, who is not sharing to add additional comments into the docket. Again, we'll share that QR code coming up. Two questions left in this session. Thank you all for your contributions.

01:49:56.864
How has your lipodystrophy and its symptoms changed over time? Do you feel like the disease or symptoms have progressed or gotten worse? And again, encourage folks we haven't heard from yet, especially. OK. We'll go to Jennifer.

01:50:15.687
Jennifer:
I mean, for me, this is Jennifer again, I have FPLD type 2, I would say my lipodystrophy definitely progressed over time because I first started with insulin resistance and then as I kept going to doctors and doctors, a new diagnosis kept happening. First it was this then that, then it was high blood pressure, then liver disease. It continued on. And now there's something going on with my heart that doctors don't know what's going on because I'm like too young for it to be happening but so it's definitely gotten worse. From one diagnosis to another keeps getting added. Thank you.

01:50:55.719
Maddie:
We'll go to Serj.

01:51:00.228
Serj:
I'm going to speed run this but basically started from severe insulin resistance at like 2 to non-alcoholic fatty liver disease that was really severe at nine and then everything tumbled and, uh, everybody just said, Oh yeah, you're a fat kid, EH, even now we, that I have the diagnosis and, and we, we watched my body fat distribution really spiral out of control from the ages of like 10 to 15. I'm still losing chunks of fat from my extremities and not like the other one. I am experiencing weird wasting and then occasionally the visceral fat coming back and especially when I'm in flares, I'll go to the hospital and my triglycerides will be elevated. It's almost as if I have a viral hepatitis, but it's not a viral hepatitis. My liver will be all over the place. Things have spiraled and my cardiovascular health has gotten weird. The liver things since starting Monjaro has gotten slightly better, it's never gone, but the fat wasting is something that I've noticed is continuously getting worse. And I've started noticing that I'm wasting here under my eyes, which was something that I never had before. I've always had a very full face and it's upsetting to me because it is, I have no control. This disease is something that, you know, we never have control and that we never really have control in much of our lives. But, you know, I, I've suffered a lot with that control is something that I've been grasping for in every facet of my life since I was very young. And to still be at 24 years old, having no control, it makes me ill.

Maddie: Thank you. Thank you, Serj.

Andra:
We hear that a lot. It's both the invisible and visible progression of the disease, right? We do see both the invisible and what's happening internally, and we continue to change through life with our fat distribution. So thank you for bringing that up, Serj.

01:53:36.490
Andra:
We'll go to Lori here, and then it looks like Rowan and Kate have some back there they'd like to call out.

Lori: Yeah, I've watched or I've seen Julian's liver or his disease progress from the unknown liver disease to lifelong hunger in a worsening fatigue in metabolic disease as a young adult. When he was diagnosed, he was metabolically stable and he looked healthy. So we were told to just wait till his numbers changed and while we waited, he became a diabetic. His triglycerides were increasing, his liver enzymes were back. They were up to a level we haven't seen since he was a little kid. So what frightens me is the damage that you can't see and you may not be able to reverse. And I feel he shouldn't have to wait for treatment. And and. If it's out there and he's suffering, so let's. I don't know.

01:54:41.023
Andra:
I know it's hard, but do you want to talk a little bit more about how that makes you feel as a care partner, as a mom knowing the disease is progressing or watching? Watching the disease progress without treatment.

Lori: It's hard because you expect your young kid to do things that other people are able to do, not have to go to different appointments throughout the year, not have to have 15 vials of blood taken at the doctor's office, and not to have to travel to different states for the best treatment for you see it taking an emotional toll on him as well as us as his parents. It's overwhelming and exhausting.

01:55:27.457
Maddie:
Thank you.

01:55:30.035 --> 01:55:57.648
Kate: Nikki says I feel like the classic lipodystrophy symptoms are vaguely stable for me now, but the fringe symptoms that we're just realizing are likely or definitely related to lipodystrophy are out of control. My A1C is mostly fine now, for example, but my immune system situation is

literally life threatening on a daily basis, and Miranda says I have to choose between working or living. I pour all my energy into my job.

01:55:57.648
Maddie:
Thank you.

01:55:58.901
Andra:
Before we go to the next, I think we've got someone who hasn't talked yet and then and then I know we've got about 10 minutes left.

01:56:06.278
Brian:
Hi, I'm Brian. I'm Cori's husband. She spoke earlier. I think a lot of people probably relate to this with the symptoms, whether or not they're getting better or worse, anything. You you kind of at a certain point hit a period where. You don't even care anymore if they're getting better or worse. You're kind of just on standby waiting for the next piece of bad news that's coming your way. And how am I gonna get through it as fast as possible or just try to keep moving forward? So I think that's, I would imagine a lot of people can relate to that.

01:56:37.831
Maddie:
Thank you, Brian.

01:56:41.493
Andra:
Ananda, did you want to comment on how it has changed over time, specifically how it has changed over time?

01:56:51.422
Ananda:
Hi, this is Ananda. So for my symptoms that have changed over time, it has been mentioned of course, but the wasting. Like actively watching my like whole body waste and become extraordinarily vascular and more painful and having now experiencing vascular pain that it's just extraordinary and I can't really tell why it's happening sometimes because sometimes it doesn't happen, sometimes it does, and then also the loss of memory and the ability to concentrate or keep track of things. That can cause a lot of frustration that I never experienced before and lack of motivation because of it. There's not a lot of. I personally don't feel like very supported or understood or. And I think that's just the progression of. Yeah, just having that as

the support, like the people who understand the awareness around me and understanding what I'm experiencing because it I truly feel invisible. It feels invisible.

01:58:15.455
Maddie:
Thank you. Thank you Ananda. We're going to move down to our last question for this session. What worries you most about your lipodystrophy now or for the future?

01:58:32.648
Andra:
Briana.

01:58:38.836
Briana:
Hi, I'm Briana again. The thing that worries me the most about my particular lipodystrophy is the fact that this ends in death. No matter what. And I don't know when my loved ones are going to be ready to let me go. And I don't like the idea that I'm going to hurt them. The other thing that scares the hell out of me, excuse my language, is the fact that I do have children who also have it, I also have grandchildren who also have it, and they may or may not get access to treatment unless I can advocate for them or other people can advocate for them. Because I don't believe that they're going to advocate for themselves at this point. And that is absolutely horrifying.

01:59:27.275
Maddie:
Kenzo.

01:59:29.974
Kenzo:
So one of the things that I think about with this is OK, like now I'm at like 6% body fat. What's gonna happen once the body fat is at 0? Does that mean I will still have a chance that I can live or is that gonna make things even worse than what they have been? So it's just like the not knowing once that body fat is actually all gone 'cause I barely have any now.

01:59:59.284
Maddie:
Thank you.

02:00:00.434
Mars:

Hi, I'm Mars. I'm Serj's partner and caretaker. One of the things that worries me the most about his lipodystrophy now and for the future is the fact that he's in so much pain all the time, to the point where I've almost lost him multiple times for different reasons. And, granted, our relationship is still pretty early and I haven't experienced a full lifetime of it. I've heard his stories and I've heard the stories from his mother and his sister and his father and and. I just. I got scared about his safety. About what is going to happen during the next hospitalization yeah 'cause he's been septic before and I mean, I've already been to the hospital with him twice now and it's scary seeing him in pain and my biggest worry is that like everyone, I'm going to lose him before I get to live my full life with him. The one that we dreamed of. That we talk about all the time.

02:01:23.875
Maddie:
Thank you. Thank you for sharing.

02:01:31.391
Ashley:
Hi everyone, I'm Ashley, I'm from Indiana and I have FPLD. What worried me years ago is different from what worries me now. In 2012 I had to have a heart transplant because of my lipodystrophy and that brought along a lot of different scary things, but I thought I was gonna be OK after that. But now though, the lipodystrophy is putting that on my newly transplanted heart and I didn't plan for that and I mean, we're doing everything we can about that, but I don't know what that means for my future. So that's just scary for me and my husband and my daughter.

02:02:21.252
Maddie:
Thank you. We'll go to Eileen. In the stripes, yeah.

02:02:28.689
Eileen:
Hi, I'm Eileen. I'm Meaghan's mom. Meaghan has partial lipodystrophy and we don't know what type and of course the future we worry about you know, longevity lack of it but I feel like what's gonna happen in between those times. Loss of independence. Meaghan is very proud to be able to keep taking care of her pets. She can no longer work but she can do some housework on some days she can go shopping and other days she can manage to order food in and, you know, have the delivery and then unpack the delivery and that'll be enough for that day. So the struggle. For the future, loss of independence, you know, will she be in a wheelchair? Will she have to move out of her house and live with someone else or live with me or move to another state? What's that going to do herself, esteem? And you're, you're feeling of yourself, of who you are. Like, I can do this. I can do this, but later on in life, I can't do it. And how do I feel about myself now? And just your pride and your, your dignity and your integrity.

Maddie: And how does that show up for you Eileen?

Eileen:
I mean, how do you feel? How do I feel about her losing her independence? I'm devastated, you know, feeling, you know, to see the child who was, you know, super independent and you know, that's something in our culture is very rewarded that you do things for yourself, and when you can't, it just feels like, oh, that's too bad, you know, that's brings on sympathy, but that's not what we're going for. We just want to support the person. But to accept that now I'm gonna be at a lesser. You know, more dependent on somebody, lesser independence and more dependence. It's really about, you know, there's it reminds me of the song in Rent. Like, will I lose my dignity? You know, will someone be there? And who's that person gonna be? And what is it gonna look like? And where is it gonna happen? Am I no longer gonna have a house where I can let my dogs out in the yard? I'll be in a tiny situation that I can manage physically, but it's not what I pictured my life to be.

02:04:49.874
Maddie:
Thank you, Eileen. We'll take one more comment before we close.

02:04:50.946
Jil:
All right. Hi, I'm Jil. I am the mother of a now 18 year old patient with congenital generalized lipodystrophy. I'm kind of going to switch the question on a little bit only because I think it brings a different context to. This particular Umm, questioning. What worried me most? When Raeya was a baby, was her standard of life, what life would she have? What would she be able to do, not do? I was fortunate enough to meet older patients who. For me, once you hit 18, you've lived your life like you've had experiences. She didn't have any, so I had to make decisions for her. As she grew, we were fortunate enough that she was given the best possible treatment for somebody with her condition, her disease, her type, everything. So she has had a life that. I don't even know how she can continue to have that life. And my biggest worry is that I've.

Andra: For those who don't know, can you clarify? A life like is that good or bad?

Jil:
OK, so she is a Oh my gosh. She competes in gymnastics.

02:06:14.004
This year in May, she competed on the national level. Oh my gosh. So she went to nationals and you know, for lipodystrophy, she scored a 39.4 out of 40. She won nationals. Like, girl walked up in there with the muscles, with everything and said this is who I am. Thank you. Thank you.

So yeah, she's lived a life, I guess. She also represents Jamaica on that national level and came fourth at the Pan American Games last year so. She doesn't have a lot of the what is the typical symptoms of lipodystrophy, Right now, Knock on wood and my biggest fear is that I get the call one day where her life comes crashing. And I hope that never happens, I really do. But you know, my biggest fear is that she doesn't, she's not able to maintain the life that she currently has.

02:07:19.570
Maddie:
Thank you. Thank you everyone for your engagement and responses. And as a reminder, the docket, which you can find here via the QR code will remain open for comments throughout the day today as well as following the meeting. So I know not everyone got the chance to share everything on their mind. And as additional ideas come up, we encourage you, please, to provide that input. We will now move to lunch to take a break. And look forward to seeing you back at 1:15. While you're on that lunch break, we encourage you to check out the storybooks on your tables right now, as well as the posters in the mezzanine.

02:07:58.646
Andra:
Thank you. Do what you need to do to take care of yourself. Certainly those of us who love food enjoy that. There is a wellness room. There's the stories and we have Give an Hour if you feel like you'd like some time with Give an Hour during break, please do that. And we will start sharp, sharp, sharp 1:15 see you soon. Thank you.

Burden of Treatment

03:04:29.409
Maddie:
Thanks folks, hope everybody had a lovely lunch. We're going to get started again in just about a minute, so grab those drinks. Grab those whatever you need. We'll get started in just a minute, about 30 seconds.

03:05:19.080
All right. Thank you so much everyone for a wonderful morning. Delighted to see so many of you back in the room as well as online. We are going to continue with our program moving into Session two, which will focus on the burden of treatment and that will begin with a panel. So panel speakers for Session two, burden of treatment. You can come and join me on stage, please. We'll get started, as you do, first with a video testimonial from some folks who could not be here with us in person, Daniela, Ricardo and Carminha. Thank you.

03:06:05.844
Daniela:
Hi, I'm Daniela.

Ricardo:
Hello, I'm Ricardo. And this is our daughter Carminha. She was diagnosed with syndrome Berardinelli-Seip. She's just a little girl of two and a half. Like any child her age, she loves to play, love, explore and discover the world around her. But there is one thing that makes her different. Every single day of her life begins with a fear. Because every morning she knows she will receive an injection. When we first learned about the treatment that could help the severe consequences, we felt hope, and that hope was real. It improved her metabolic values and helped control the overwhelming anger caused by her disease. We are deeply grateful that it exists. But there is another side of the story that is not so joyful. For the past six months, we have given our daughter an injection every single day. And six months later, the fear is still there. The tears are still there. There has been no adjustment, no acceptance. Every morning remains a battle.
Our daughter is still too young to understand why this is happening to her. We explain, we comfort, we reassure, but she cannot fully understand. What she

03:07:43.519
understands is that the people she trusts the most are the same people holding her still while she's being hurt. That reality has changed our relationship with her. It has created moments of fear, uncertainty and mistrust that no parent ever wants to see in their child. Administering the injection takes two adults, one person must hold her and the other must try to give the injection. Sometimes even that is not enough. There have been days when we have to attempt the injection

three times, not because anyone made a mistake. Not because we are careless, but because our fear is so overwhelming and their determination to escape is so strong, that keeping her still becomes almost impossible. The emotional burden does not begin when the needle appears. It starts much earlier, sometimes the night before, sometimes the moment she wakes up. The anxiety builds as she realizes what is coming. Fear becomes part of the routine and it affects our entire family. As parents, we know the treatment is helping her. We know it is important, but we are also the ones causing the pain. There is no emotional preparation for that.

03:09:14.940
The injection is only one part of living with this disease. Every meal requires planning. Every ingredient must be carefully considered. Every person must be weighed. We cannot simply leave home and eat wherever we happen to be. We must prepare food in advance and carry it with us. What is spontaneous for most families requires constant organization for us. Traveling brings additional challenges. Even a trip lasting more than one day requires careful planning to transport and store medication at the required temperature. There are no breaks from the disease, no holidays from the treatment, no days off. And while today's challenges are difficult, the future can feel even more frightening. Today she's too young to fully understand. Tomorrow she will ask questions. She will become aware of the difference between herself and other children. She will compare. She will wonder why her life is different.

03:10:23.809
And we do not yet know how difficult those conversations will be. Raising a child is hard. Raising a child with a rare disease is a thousand times harder. We know that some people listening may have lived through similar experiences. You know what it means to build your life around disease. We are so grateful for the current treatments. It has made a meaningful difference in our daughter's life. It has improved important clinical outcomes. It has helped control one of the most difficult aspects of her disease.

03:11:05.749
But people with lipodystrophy deserve more. They deserve treatments that are less painful, traumatic and invasive. Treatments that allow children to spend less time being patients and more time simply being children. Our greatest hope is that continued research and innovation will lead to new options for families like ours. Because no child should have to start every day with fear. Thank you.

03:11:45.553
Maddie:
Next, we'll hear from Gretchen.

03:11:58.017

Gretchen:
I remember sitting in my PCP office saying I feel so sick I think I might be dying. Three days later I was in Pittsburgh trauma ICU, maxed out on pain medication, screaming that my chest felt like someone was stabbing me.

03:12:14.465
I was diagnosed with severe pancreatitis, kidney failure, and respiratory failure. I begged the ICU physician to sedate me with whatever medication she had because the pain was unbearable. Thankfully, plasmapheresis saved me that night. Life never returned to normal after that. My body failed me again and again. Nine years later, lipodystrophy had destroyed so much of my body I had to leave the job I love and became disabled

03:12:45.445
before turning 50. Things that once took hours began taking days, and severe GI issues had me running to the bathroom dry heaving, hoping I could make it on time not to soil myself. The day I didn't make it was my very last day of work. I hated the person I was becoming while grieving the loss of the capable, dependable woman I had always been. For years, pancreatitis became part of my life, my triglyceride's routinely climbed into the thousands. Hospitalizations became a familiar routine. Crushing chest pain, swelling, days without eating, insulin drips, hourly glucose checks, X-rays, CT scans and long hours alone in a hospital room. The cycle finally broke when a medical student suggested I seek evaluation at the National Institute of Health. There, I spent three days undergoing extensive diagnostic testing

03:13:49.330
and was officially given a lipodystrophy diagnosis. I enrolled in a medication study soon after. During that year in the trial, I slowly found myself again. I could walk without feeling like I was carrying 700 extra pounds. I even returned to direct patient care, something I hadn't been able to do for years. I finally felt a little bit more confident and a lot happier. Then without warning, it all ended. Everything changed. I was told I could no longer continue the medication. No explanation, no worries, no answers. The emotional roller coaster of hope followed by disappointment was crushing. I became the mom who could barely find the energy to speak to my family. My husband quietly took over nearly everything and at times even our marriage suffered because I was too exhausted to give

03:14:48.908
any part of myself to anyone else. I held on to hope, only to face more unanswered questions and setbacks. For the next two years, I had no treatment. Mentally, I was more depressed than I had ever been. I cried every day for six months. I seriously considered stopping all of my treatments. I'm constantly deciding which medical problem needs attention first. Between 6 specialists, I see twice a year, my primary care visits, surgeries, testing, travel and copays, the cost of staying

alive and managing this disease is more than my monthly security payments. I carry heavy guilt for being a financial burden to my family.

03:15:35.891
In early 2025, my endocrinologist and I decided to no longer wait for a clinical trial of a GLP-1. I was so desperate I would try anything, do anything to feel better, no matter the cost, no matter the risk, even if it meant damaging my pancreas more. Six months later, my A1C dropped to a 5.9. My pancreas enzymes were discontinued. My diuretic medications were stopped. By the end of 2025, my dose was increased to lower the amount of insulin I use daily. Today I've gone more than eight months without using insulin. But, I'm scared this treatment will stop working. I'm scared I'll lose access to it. And most of all, I'm scared of becoming the woman I was just two years ago, the woman who spent more time surviving than living. I know how quickly everything can change because I lived it. I know this treatment is only for now, which makes the need for treatment options even more urgent to prevent further organ damage and to give us hope. I watched my father and brother suffer before they died, long before we knew they had lipodystrophy. I know what this disease can take from you and your family. That's why I will forever keep fighting for answers, for awareness, and for my own son.

03:17:17.918
Maddie:
Thank you, Gretchen. Next, we'll hear from Maryanna.

03:17:30.218
Maryanna:
Hi guys, I'm Maryanna and I have partial type two. When I was sixteen, my doctor sat down with me and explained what psychosomatic meant. He told me that while the collection of vague symptoms I was experiencing felt real to me, they were merely a creation of my brain. His opinion never changed, despite that I was sweating through four shirts every day. My periods were increasingly heavy and painful, my blood pressure was significantly elevated, and my triglycerides had gone over twelve hundred, landing me in the hospital with pancreatitis. My parents spent thousands of dollars on tests, procedures, and specialists throughout high school and college, none of which produced any answers.

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Many doctors ended up with one conclusion. I was just depressed, anxious. And told me I should see a psychiatrist. I was just a teenager when I learned the value of perfecting my mask. I learned to hide it all, the pain, the exhaustion and the misery. I became an EMT and then an emergency room nurse. In the ER, eing emotionless is power. No one saw my tears on the drive home from the pain in my legs or knew that I needed days to recover from shifts. I learned to shove everything down. People have sympathy when you have a visible or well known disease.

03:18:52.230
No one wants to be around someone who is always tired, moody and complaining about pain. In my mid twenties, my mom was going through breast cancer for the fourth time. She had become interested in a plant based diet and I agreed to try it with her. Trying it became a permanent lifestyle for me after I realized it made a large improvement in the way I felt, in addition to improving some of my lab work and blood pressure.

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Around that same time, I grudgingly started running with a coworker. Though I had never been into sports or exercising, I found that I loved this new pain of working out because it was pain I could control. Over the next decade, my training morphed from endurance sports, triathlons, running and mountain bike racing into strength sports, powerlifting, strongman, hybrid competitions. The heavy weightlifting was what really improved my metabolic profile and dimmed the constant obsession with food to a more manageable level.

03:19:53.242
Fitness and nutrition not only became my hobbies, they became my treatment. At twenty eight, I was finally diagnosed with lipodystrophy by a specialist at Mayo Clinic. Despite finding out that there was no cure and no real treatment, just knowing that my symptoms were not imaginary was a freedom for me. I've never been considered for a drug trial because I was always deemed not sick enough. That's hard to swallow when I've had to build my entire life around managing my disease through exercise, nutrition, adaptive clothes and equipment, and other self treatments. I found out how fragile my coping mechanisms were when my son was born at twenty six weeks and spent five months in the NICU. I could no longer spend hours exercising, or I could be out of the house and away from food. I gained twenty pounds in less than three months and became sicker than I'd ever been. My marriage disintegrated, my health spiraled, and I realized that without my carefully constructed coping mechanisms, the disease takes over very quickly. When I begged my doctor at Mayo Clinic to do something to help me stop eating, this one doctor that I had finally put my trust in told me that putting food in my mouth was a choice, and that I should see a psychiatrist about it. My lowest point came while watching my son play at a park when he was two.

03:21:15.899
I barely had the energy to sit upright, much less go play with him. I just wanted to die. I wasn't the mom I wanted him to have or the wife I wanted to be to my husband. One of the hardest invisible complications of lipodystrophy is it steals moments with your loved ones when you are too exhausted to be fully engaged, and then crushes you with the guilt of feeling that you're letting everyone down, no matter how hard you try. That morning at the park, I vowed that I would make whatever sacrifices needed to regain the routines that had allowed me some

semblance of normalcy. Today I spend one to two hours in the gym four to six days a week. I spend another two to three hours a day preparing food and much more time planning meals and grocery shopping.

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It costs at least a thousand dollars in groceries each month for my family of three to eat the way that I need to. My schedule revolves around workouts, meal planning, and protecting the routine that keeps me functioning. A few days off because of vacation or injury can send me into a downward spiral physically and mentally. My family lives with these restrictions too. I keep the bare minimum of food in the house and absolutely no junk food.

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No one should have to construct their entire existence around just trying to stay functional or live in constant fear of an interruption in their routines. Even though I'm an elite athlete and look fit and healthy to the world, my goal is for people to understand that extraordinary effort does not eliminate lipodystrophy and I still suffer many quality of life burdens in silence. My improved lab values don't reflect the pain, crushing fatigue, and countless other symptoms I live with every day. I've qualified for nationals in five different sports, though many of these goals have been derailed by unexplained pain issues. My greatest fear in joining a drug trial would be experiencing what it feels like to feel normal.

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Having coped with my symptoms my whole life, I fear that I would never recover mentally if I experienced what it was like to feel great and then for some reason was no longer able to obtain that drug. My hope isn't simply for a cure someday. It's for treatments to be found that reduce the immense burden of living with this disease so that patients don't have to build their lives around managing it just to function at a baseline.

03:23:54.611
Maddie:
Thank you, Maryanna. Next, we'll hear from Sharon.

03:24:07.227
Sharon:
Hi, my name is Sharon. I'm 35 years old and I have familial partial lipodystrophy type 1.
I always knew I was different. From a young age, I wondered why I didn't look like the women around me. I had signs of insulin resistance by the time I was six years old, with the appearance of acanthosis nigricans, the dark patches of skin on the back of my neck. My mother took me to the best doctors she could find, but every test came back normal. So no diagnosis was ever made. I knew something was wrong because I looked different from the other kids around me and I had

health problems that no one had. For the next twenty seven years, doctors had no good explanations for me. They thought about Cushing's disease, PCOS, obesity and others, and when those didn't fit, they said you have no willpower. They told me I wasn't compliant with my diet, my exercise and my treatment. That wasn't true. I was doing everything they have asked for, but it was easier to blame me than to keep looking.

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When I was 28, already the head of a Cancer Research lab, I came to my emergency room at the hospital I worked with with a leg ulcer. I didn't know it was related to diabetes. I didn't know it was an ulcer but that day I was diagnosed with diabetes. That day I started treatment for diabetes. This was the start of years of complications and that eventually took my freedom to walk normally. All those years the message was the same, if you only lose the weight, everything will be fine, all your symptoms will go away. So I tried. I started a first generation GLP-1 agonist and it worked. I lost weight. My A1C came down and it lasted about ten months. After the ten months my blood pressure and my pulse began climbing out of control, although I lost seventy pounds.

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And from there everything became the same story over and over again. They started me on a medication for the problem. It worked for a few months and then stopped. They increased the dose or added the new medication. It worked for a few months and then stopped. This is for every medication, for every complication I had. After about a year and a half on the GLP-1 agonist, it stopped working and my A1C climbed higher and higher. I wasn't tolerating my dose well but they increased it anyway because there was no other option for me. Eventually I switched to a second GLP-1 agonist and it worked for about a year. And then the same cycle began. The medication stopped working, they increased the dose or switched me to a completely new medication and it stopped working again for every single medication they gave me. I was frustrated. I was eating well, exercising, taking all my meds as needed and still everything stopped working. I was doing everything right, but still had to work so much harder just to be stable, unlike some patients with just diabetes or just hypertension.

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Then, at the age of 33, I was diagnosed with lipodystrophy. I started the GIP/GLP-1 agonist and for the first time it felt different. It worked and it kept working. I reached my lowest weight and the best metabolic control I've ever had. I let myself believe that I had finally found something stable. And then one day my triglycerides started to climb again. My endocrinologist, who is genuinely the best person I ever met, asked me if I was taking my medication as she ordered. After everything, at my healthiest, the first question was still whether I was compliant with the treatment, not the disease progressing. And this is how deep it runs.

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Even with the best doctors it is always easier to question the patient than to accept that lipodystrophy simply does not stop. That this is the truth. This is the progressive nature of like audios for free. It doesn't stand still, and neither can my treatment. And this is my biggest fear right now. Not the injections, not the pills. My fear is the moment that my current medication stops working because I know I cannot increase the dose forever. And right now there is nothing

03:29:31.748
new waiting for me. There is no next option. So when I think about what is an ideal treatment for me, it is not about convenience. I will inject myself once a day, several times a day, take a fistful of pills. But I want a treatment that will last not for one month, not for six months, a year. I want a treatment that will last ten years, fifteen years, and maybe forever. What I need is a peace of mind. Again, not for a couple of months, for a long, long time. Something I can count on so that I can stop bracing for the moment it all falls apart again, and finally live.

03:30:25.646
Maddie:
Thank you, Sharon. Now we'll hear from Mallory.

03:30:38.833
Mallory:
Hello, my name is Mallory and I'm twenty four years old. I was officially diagnosed with partial lipodystrophy when I was twelve. My journey started long before that. It was the summer before 5th grade when I started paying attention to how my body looked. I could not understand why my legs were becoming thinner while my stomach was getting bigger. At first, I tried to ignore it. I was excited to be one of the oldest kids in my grade and wanted to focus on school instead of my body. Unfortunately, my body changed so much that I wasn't the only one who noticed. Kids started bullying me and I swore I would never show my legs again. My mom noticed the changes when I showed her my bathing suit for a school field trip. She thought I was dehydrated or not getting enough protein. One referral led me to another until we met Dr. Oral, who immediately recognized partial lipodystrophy. I remember feeling relieved that someone finally had an answer, until I realized that there wasn't really a treatment that could give me my childhood back.

03:31:45.492
I couldn't stop crying. All I wanted was to look like my friends again. I kept asking myself why did this happen to me? How do I get my healthy body back? I was fortunate to begin treatment early. I know many people with lipodystrophy aren't diagnosed until years later, after the disease has already caused severe damage. I'm incredibly grateful for that opportunity. The first treatment helped improve some of my internal health, but it didn't change the things that mattered most to me as a twelve year old girl. My stomach became a little smaller, but strangers

still asked if I was pregnant when I was thirteen. Kids at camp still left me out because I looked different.

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My mental health continued to decline because I still didn't feel, I still didn't feel like I belonged. Eventually my body developed antibodies against the treatment and it stopped working. It was bittersweet. I knew it had been helping my organs, but I also knew it had never fully helped me feel like the person I knew I could feel like. Over the next several years, I developed type 1 diabetes. I underwent I had a spinal fusion surgery for my scoliosis. I participated in another clinical trial that ultimately ended after I developed pancreatitis and spent years in and out of the hospital with chronic pancreatitis. I underwent weekly plasmapheresis treatments and had multiple ports placed in my chest. Every time a treatment stopped working, I had to start over again. Physically, that was exhausting. Emotionally, it was devastating because every new treatment brought hope that maybe this would finally be the one.

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In 2019, I became the first person with lipodystrophy to receive the treatment that I'm still taking today. Being the first person was scary. I knew there was risk, but I also knew what my life looked like without another option. I would absolutely make that decision again because hope was worth the risk. The treatment had made a huge difference. My liver is much smaller than it was when I was a teenager and I spend much less time in the hospital. But treatment hasn't given me a normal life. I still live with chronic pain every single day. Some days the pain is in my nervous system, some days it's my digestive tract. My joints hurt when I move and when I'm active all my muscles ache for a long time. Sometimes even eating a small meal causes severe stomach pain. There isn't one type of pain, there are many, and every day becomes a constant battle to figure out which pain that I'm managing that day. The fatigue never really goes away either. I constantly have to plan my life around the anticipation of pain and exhaustion. Compared to my friends and my roommates, I need significantly more sleep, more breaks, and more

03:35:02.763
recovery time just to get through the day. My mood, blood pressure and hormones are always fluctuating. I also work incredibly hard to manage this disease. I probably eat more vegetables than anyone I know. I follow a very healthy lifestyle because I know it helps keep my symptoms better under control. Under better control, but lifestyle alone is not enough. The treatment I'm currently on has helped my hunger tremendously, but I still inject three shots every week and the injections leave behind scar tissue that I have to work around. I still experience severe stomach cramping that sometimes feel like my organs are constantly pushing outward. On top of that, I take medications every day to manage diabetes, high triglycerides, kidney protection, chronic pain, muscle spasms, digestive issues, anxiety and depression.

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Every medication treats one piece of the disease, but none of them treat all of me. The medications I'm currently on, to name a few gabapentin, omeprazole, fenofibrate, Zoloft, Cymbalta, lisinopril, and Baclofen. I have a lidocaine infusion every month to help manage the pain as well. When people see my lab work improving and all the meds I'm on, they often assume I'm doing well. But my daily life tells a different story. My biggest hope for the future isn't simply smaller organs or better lab values. It's a treatment that helps manage my diabetes, reduces my chronic pain, and lets me live without constantly planning my life around fatigue, medication schedules, injections, and the possibility that my symptoms will flare.

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I'm incredibly grateful for the treatments I've had access to. They have changed and in many ways saved my life, but I'm also living proof that even with early diagnosis, multiple clinical trials, healthy habits and access to treatment, there's still a tremendous unmet need. I hope that someday people with lipodystrophy won't have to choose between surviving and truly living. Thank you.

03:37:28.404
Maddie:
Thank you, Mallory. Next we'll hear from Kenzo.

03:37:33.789
Kenzo:
Hello everybody. I'm Kenzo. I'm 31 years old. Here we go. So people often look at me and think I'm the picture of health. All right? I'm a physical education teacher, former collegiate athlete, bodybuilder, and if I'm not working out, I'm probably dancing somewhere. People see muscles and assume I won the genetic lottery. We all know that's not true. OK? What they don't realize is that my body isn't healthy because it's muscular. I look this way because I have lipodystrophy. I can’t store healthy functional fat the way that most people can. Instead, that is stored where it doesn't belong. Putting my heart, liver, pancreas, and the rest of my body at risk. I can look incredibly fit on the outside while dealing with serious health problems on the inside. Sometimes I even struggle to understand it in my 31 years. I look at myself and wonder how can I look so muscular and have almost no body fat, yet have so many health issues.

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What happens if I reach 0% body fat? Will I survive? Is working out going to make that happen faster? Do I need to stop? Those questions create a lot of mental turmoil. I've always worried about the uncertainties of this disease, and sometimes the way I cope is by trying not to think about it at all. My childhood with lipodystrophy stands out strongly in my memory because up

until I was about fourteen, it felt like we were just winging it with my care, getting tested for numbers and things like that. But no treatments or anything was going on at that time to my knowledge. I don't remember much about the journey to my diagnosis because my mom dealt with all of that. I ended up being diagnosed when I was about 6 months old and from six months up till 14, we were winging it until I ended up moving up to NIH where I found Dr. Brown and her team.

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So imagine being 5 or 6 years old, OK, wanting a Happy Meal and you're leaving a doctor's office and mom's like, no. You can't get it. We can't eat that stuff. You have to eat healthy. Now, of course I'm sitting in the back seat crying because what do you mean I can't eat that stuff? All the other kids are doing it. All right, but at home we had to be careful with food. Most of my meals were grilled or baked protein with vegetables, rice or potatoes, fried foods and pasta heavy meals, which were my favorite, they couldn't be regular things because they would send my triglycerides up and that would of course put me in a bad position. But the hardest part wasn't just that I wasn't allowed what I wasn't allowed to eat, it was how much I wanted to eat. I could keep eating and eating and eating with no stopping. We could have food in the house and it would be gone just as quick as it came in. I didn't understand why I was so hungry, but I just knew that I was, and I needed something to eat. Everything changed when I was 14. I started receiving treatment from NIH. I had started leptin there.

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I also started metformin and fenofibrate but at that age, I still did not fully understand why I needed all these medications. I looked healthy, I was athletic. I didn't really see a problem at the time. I didn't realize that the treatments that I was starting was going to be protecting me from complications that might end up happening in the future. Metreleptin made a significant difference in regulating my labs. It also helped my hunger slightly. I had a little more control, but I still thought about food all the time. Still think about food all the time.

03:41:08.169
It also affected other parts of my health, so starting treatment at 14 helped start my period. And although it was inconsistent for a while, it was finally there. I will be honest, I didn't want it to begin with. But, when it came to the treatment, it didn't fix everything. The hunger never truly disappeared. Neither did the fatigue or the pain. I experienced joint pain that can come on subtly and it can become paralyzing, especially in my elbow joints. When it happens, I have to stop whatever I'm doing and try and try and stabilize the joint. Typically I'll try to find some kind of wrap to put it in just to get a little relief from that pain. Sometimes that helps, sometimes it doesn't. When I'm in pain I tend to isolate myself.

03:41:56.283

I don't want to be a burden to anybody, I don't want to constantly be talking about it and then people don't want to be around me. I never know whether the pain is going to last for a few minutes or several hours, so sometimes it feels easier just to stay at home and not do anything. The fatigue affects me in a similar way. I've always loved dancing. I'm a joyful person and I love sharing my energy with other people. But some days it is really, really hard to show that part of myself because of the fatigue. Imagine sleeping 8 hours or more

03:42:26.808
and waking up feeling like you never slept. These are times where I would skip the gym, family gatherings, time with my friends simply because I'm too tired to function. When you cancel enough times, eventually people are going to stop inviting you and that's what hurts. Then there's the hunger once again that I still feel to this day. Most people get hungry before a meal. I can feel hungry all day. I can finish eating a meal and immediately start thinking about what the next meal is going to be. There have been times when I've eaten three meals back-to-back to back, because my body still believed that it was starving. I hadn't fed it. Today, exercise is another major part of how I manage my health. I'm a physical education teacher. So I'm active throughout the day. I usually get at least 5 miles of movement throughout the day. That's from walking, jogging sometimes. And then mentally I'm always pushing myself for more. My goal is often to get a little bit more than that. But I have this belief in the back of my mind that if I just keep on moving

03:43:29.234
consistently, that's going to help me keep my lab numbers good and it's going to keep my overall health looking good. Even when I know I'm active, I still feel like I'm not doing enough, though. Part of that comes from being a former collegiate athlete. I'm used to a certain level of training, but now I have left ventricular hypertrophy, so I can't lift weights the way that I used to, and I used to love lifting weights. I've had to change the way that I trained and mentally that has been difficult. Exercise helps fill in some of the gaps that medicine doesn't address, but exercise itself becomes a treatment burden. Spending hours every day managing your disease should not be considered a successful treatment strategy, especially because that level of activity isn't sustainable or even possible for everyone with lipodystrophy. One of the biggest reasons I take my health so seriously now is because of my younger sister. She has lipodystrophy too. She's not here with me today, but when I was younger there was a period in time when I was not taking

03:44:29.100
the medications the way that I was supposed to. Once again, I didn't fully understand everything, but I knew she watched me and she started following in my footsteps, so she was not taking her medication either. But then she ended up in the hospital with a few complications. That changed my perspective completely. I realized that I didn't just need to do better for myself, I needed to do better for her as well because she was looking up to me. It's always been me and her in this

together. We started at NIH together. I want both of us to be here for the long run. So today I'm participating in clinical trials for miba. I switched from metreleptin partly because instead of taking injections every day, I'll only have to take three injections one time a week. That convenience has been a major improvement. My blood sugars have improved enough that I've been able to stop taking metformin, and my labs have really never looked better at this point. But the experience has also taught me something important about successful treatment really means.

03:45:31.934
Toward the end of the week, usually around day 5, I can feel my hunger become more intense as I get closer to the next set of injections. Good lab numbers also don't necessarily mean that I feel good. My medications may be helping protect my future, but, they haven't eliminated the day-to-day burden of living with this disease. And because my current medication is still being studied in a clinical trial, there's another uncertainty. I don't know whether I will always have access to this. Access to treatment has been frightening

03:46:01.400
to me. I can look muscular and healthy to someone walking past me, while my medical record shares a completely different story. That disconnect is one of the hardest things about lipodystrophy. I want people to understand that living with lipodystrophy isn't just about abnormal lab values. It's about the constant work it takes to stay alive and healthy. At this point, I've done everything that has been asked of me. Strict diet, medications, constant exercise, daily injections, clinical trials. Those things have helped protect my metabolic health, but they still haven't relieved the daily burden of this disease. I don't want a treatment that solves one problem while creating another or putting my future at risk. I want to live a long, fulfilling life. Despite everything, I've learned to love myself and embrace who I am. So sharing my story online has actually connected me with some people, all right, with this condition, and it made me feel like I wasn't alone. And then that's one of the reasons why I'm sharing my story today, because my hope is that these future treatments won't just make our lab numbers better. They were actually going to help us live longer lives. Thank you.

03:47:20.216
Maddie:
Thank you, Kenzo, and thank you to all of our panelists. You can take your seats in the audience.

03:47:26.976
Maddie:
So as we begin our discussion around the burden of treatment, we're going to start with a polling question. And again, you can access this poll via the same link from earlier, which you can also find using the QR code on the screen or at slido.com with code 9218369. This first question: how

are you managing your disease? You or your loved one. Select all that apply. Lots of responses coming in. Thank you.

Andra:
The bottom line is changing depending on the percentage and it's a little, little cut off. But I, I think the, what I'm seeing here, Maddie, is that there's a lot of different ways, and since it's select all that apply, I'm guessing a lot of folks are selecting most or a few. Yeah, show of hands here in the room. How many folks are selecting multiple options?

03:48:51.377
Maddie:
OK, thank you.

03:48:55.370
So let's build on this. Let's start with our first discussion question and a few reminders to please keep responses specific and short, two to three answers. We can hear from as many people as we can. And Andra and I may follow up. Again, use the microphone if you're here in the room, hands up for the mic runner. In zoom chat, you can type your comments you'd like to be shared aloud. And please do share as you do your lipodystrophy type or your loved ones and where you're from.

03:49:22.078
Our first question. How well does your current treatment regimen treat the most significant symptoms of your disease? How well have these treatments worked for you as your condition has changed over time?

03:49:38.485
Go to Serj in the back.

03:49:43.177
Serj:
So I'm on a slew of medication, a whole cocktail as many of us are, and I would say not very well. As many of you guys have said it will work for a little bit and then it doesn't. And that's sort of what I'm dealing with now is that I'm, I'm realizing that I'm either gonna have to tweak some things or start something completely different. And this is what Mallory I think was saying is it's terrifying to have to start again. And it's, it's something, we got diagnosed around the same age and we're in a lot of the same treatment trials. It's so traumatizing. It's exhausting.

03:50:36.112
Maddie:

Thank you. We'll go to the back in the blue.

03:50:41.580
Robin:
Hi, I'm Robin. I'm speaking for Russell again. He started as I said earlier, metreleptin in 2014 and it has worked like a charm ever since. All of his numbers, metabolic numbers have been in the normal range and the symptoms that he deals with now are symptoms that were, I guess, subject to damages that were done prior for the 10 or 15 years that he had the disorder prior to being diagnosed so. It's working great and I don't honestly don't know if he would be here today if it weren't for metreleptin.

03:51:21.716
Andra:
Can I ask a follow-up question on that? So when we get to how has the condition changed over the time in, in terms of pre-treatment to treatment now, can you talk a little bit about what happened before and, and what you see as an improvement

Robin:
In his college years when he went to donate blood and they called later and said we can't use your blood, your triglycerides and your cholesterol is off the charts. And so he didn't really know what that meant at the time. And in his 20's the disease progressed. And just like you all, the fatigue, major memory issues, the, the pain, sleeping all the time and then the damage to

03:52:08.557
his, the high triglycerides we had, I don't 3 to 4 pancreatitis attacks, you know, airlifted from campgrounds while we were on a family vacation. I mean, all kinds of things just like you guys. And so through his 20s and early 30s that progressed. And then in 2014 is when we had a friend come to us who was also in a trial and said, hey, your symptoms are similar to mine. And I, I think that you could benefit from calling Dr. Brown and so the moment he took that medication and all the numbers came back, you know came back down, the only damage really that was done was his kidneys and then had a transplant 2 years ago and has been doing good ever since so.

03:53:01.939
Andra:
There's always a fine line between we talk about the burden of the disease and then treatment. I think it would be really interesting if you would just give us maybe one sentence. About. So with treatment, the change has been positive over time.

Robin:

Absolutely positive overtime.

Andra:
What's a good day look like today now?

Robin:
I mean, well, a good day is a trip to Montana, fly fishing on the river, spending all day out camping, spending time with friends and family. Has totally changed how we live our lives.

03:53:39.932
Maddie:
Thank you.
We'll go to Crystal.

03:53:53.244
Crystal:
Ok, so my current treatment in my opinion is pretty amazing right now. It's what's worked the best. And it helps me use them as tools to implement other things so that I feel like I'm on the least amount of medication that I've ever been. So that I can do daily life changes, incorporate them. And have better quality of life that way. And over time, I hope that by reducing the amount of daily medications that I have to take later on when I need them, there's still options for me.

Andra:
Is your current treatment a clinical trial or an approved treatment?

Crystal:
I'm in open label medication and also GLP-1.

Andra:
Same thing. Let's go back just a little bit, but in terms of treatment, what are your concerns now if you were to lose access to this treatment?

Crystal:
It's always scary in the back of my head. What if something happens and I no longer have access to this life changing medication? What am I going to do? And I'm scared of reverting back to how bad it was.

Andra:
And I'm just going to refresh the room's memory and the memory of the room.

03:55:24.189
When you spoke earlier about that thing was really difficult to get yourself out of bed, is that is that what you're referring to?

03:55:33.661
Crystal:
Yes, absolutely it's really hard when you have to rely on your spouse to help you out of bed because you no longer have the strength to get up on your feet.

03:55:44.489
Andra:
Thank you, Crystal.

03:55:47.490
Maddie:
Thank you. We'll come here in the blue.

03:55:51.985
Laura:
Hi, I'm Laura. I have type 1 partial. So I am currently on tirzepatide and it has made a significant improvement in my diabetes and my fatty liver, my proteinuria, triglyceride control. So I feel like it's definitely made life changing improvements in those symptoms, but I'm always waiting for when it's gonna stop working. And so every pound I see the scale go up, I'm like, uhhh is this the end of it working for me? And so having progression of disease or of treatment development is important because it gives us new options when they stop working for us.

03:56:30.691
Maddie:
Yeah. Laura, I want to follow up. Thinking about your lipodystrophy today, would you describe it as well managed with your current treatment?

Laura:
I would definitely say it's well managed. Before I was on pump life, I was having trigs that were controlled, but with lots of interventions. But I feel controlled now.

03:56:52.499
Maddie:
Thank you.

Andra:

Jil right here.

03:57:00.966
Jil:
All right, so just a reminder, Jil, daughter with congenital generalized lipodystrophy. I was very fortunate that we got her diagnosis when she was five months. She was able to start. She was under medical care between five months and two years. She started her treatment on two years. We don't know what it's like to have lipodystrophy. We don't we, I say we 'cause I'm speaking for her as well. She has none of the comorbidities she does not like. The disease was not able to attack her. At all. So right now I would say her treatment works. It's doing more than working.

03:57:51.555
I mean like knowing that these are the things that could happen and also hearing and knowing like seeing in the back of my mind where, oh my gosh, like my kid is not going to have diabetes. She's not going to have. You name it, you've heard it. None of those things she possibly has the opportunity of not having because of her treatment. And then when you look at the life she has, yes, she's a gymnast, blah, blah blah blah blah. You know, to kind of brag a little bit about her because I can. She graduated high school with a 4.7 GPA. She's on a full ride, full academic ride at Stanford University. The end.

03:58:43.898
Andra:
Thanks, Jil. I know we want to. We've got a few hands and so definitely want to go here.

03:58:50.555
I want to, Maryanna, if you don't mind, I'm going to follow up, a question from you because also an elite athlete not on treatment, haven't had access to treatment that's beyond your, your diet and lifestyle. Can you talk to us just a little bit more about like that challenge of if you lose access to your treatment, you hurt yourself, you're able. Can you talk a little bit about what that looks like and if that has changed over time?

Maryanna:
Sure. I think as far as changed over time, I've learned how to handle it better. I've learned how to say no to things, to manage it better. But I think it's when I go on a trip or change my routine that I really realize how many things have to fit into place for me to feel OK. And that, you know, I might go day-to-day and think things aren't that bad, but when those puzzle pieces aren't in place? The exhaustion is overwhelming. The desire to eat is overwhelming. I don't even call it a hunger. It's more of just a constant fixation on getting food. And the more I eat, the more I want to eat. So those are the things that I think really affect me and I, I do fear everyday injury I.

Think about that. When I'm training and when I'm competing is if I injure myself and I can't do the things that I need to do. It really doesn't end well for me mentally.

04:00:32.530
Andra:
Thanks. Thanks. I was just thinking about the, there's several athletes in the room, but I was specifically thinking about your story. So thanks for letting me call on you.

04:00:42.453
Kristen:
Hi, I'm Kristen. I'm from California. I have familial partial lipodystrophy. I've now been on myalept for around 2 1/2 years now and it has helped me in several important ways. Before I was diagnosed and started treatment, I had spent about 23 years with intense hunger that I just never understood. I was always thinking about food. I would go to bed just anticipating the breakfast that I would have and planning out my lunch, dinner, snacks in between. And myalept completely changed that. I no longer eat as much. I don't have those cravings, the hunger anymore, the thoughts.

04:01:19.980
I also had fat concentrated around my abdomen. That never seemed to change no matter how much cardio or ab work I did. And the medication reduced that significantly. And now I feel comfortable, you know, wearing a bikini to the beach or crop tops, which I would have never done before because I would always feel pregnant or just not like my other peers. My fatty liver also improved and has continued to improve. My triglycerides also improved with the myalept and I'm up to the point where I have now been able to come off finofibrate, but at the same time my loop hasn't fixed everything. I still deal with extreme fatigue, muscle cramps and aches, and the psychological impact of how I see my body and myself.

04:02:05.245
Maddie:
Thank you. Thank you, Kristen. Kate.

04:02:10.357
Kate:
A couple of Zoom comments here from Jason. I'm a 2008 Olympian in the sport of weightlifting. My hobby probably saved my life due to the structure provided as an athlete. I had to get enough sleep, eat right and really take care of my body.

04:02:26.342
Maddie:

Thank you. We're gonna move on now to our next poll question so get those, get those phones back out. Question is would you define your condition today as well managed. Your condition or your loved ones' condition?

Andra:
Certainly my research brain is wondering the correlation between who are on treatment and who are answering yes or no. Those are numbers will, right? Those are numbers we'll look at on the back end for sure. I, Maddie, I think it's interesting that I think prominent is no and we've it's reflective of what we've heard in this room today, right? And then again, we could go dig a little deeper and be curious like, no, doesn't mean that the parts of the condition are well managed. I think it's just asking in general is it well managed.

Maddie:
Yeah. Well, let's dig into it more with our next question. How do you currently address the most burdensome aspects of lipodystrophy and that includes non-drug therapies such as diet modification and other approaches? How well do these strategies address the most burdensome aspects of your lipodystrophy? And again, that includes examples. Like exercise, non western medicine, pain management, etc etc. There's a lot here so I would love to hear whatever's speaking to you within these questions.

04:04:11.513
Becky:
Thank you Becky from the UK, FPLD2. So as I mentioned previously, the metreleptin treatment has really helped with my hyperphagia and so my biggest struggle at the moment is generally fatigue, which I really, really struggle with. I work full time but that means I spend my weekends sleeping in order to kind of recharge my batteries so that I can do another week of work. I come home after work and I almost always go straight to bed so all I do is eat, sleep, work, repeat which is very frustrating and I do feel like life is passing me by sometimes. I also suffer with a lot of pain, particularly in my back, mainly due to the extra muscle mass and the tension that's there. And I currently see a physio which I pay for privately once a week just to try and keep on top of the pain and the physio in the past has told me that I probably have about 50% extra muscle mass in my back than kind of a woman without lipodystrophy, and that's what's causing me so much pain. And that also impacts headaches because you get tension headaches because your muscles are so tense. I've had tears, gastrocnemius tears in my calf muscles on two or three different occasions. Again, it just comes from the tension in the muscles. So yeah, pain and fatigue are the things I struggle with.

04:06:01.142
Andra:

We haven't heard much from Briana, I see your hand up. We haven't heard much from people who feel like diabetes or triglycerides are the most burdensome. We've heard a lot about triglycerides and diabetes, but not the most burdensome. So I'd be curious, if there's anyone in the room, if there's not, that's OK. That has those as the most burdensome and then the second if you have strategies or treatments that impact that. Is it true?

04:06:33.019
We have Jennifer. We have Sharon.

04:06:36.491
Jennifer:
I would say right now with it most of my other stuff is taken care of but like my diabetes is still out of control even with taking multiple almost hundreds of at least a couple 100 units of insulin a day. It just doesn't seem to work in my body, but everything else is good. Like my triglycerides are good, my cholesterol is good, like all those other things are good. But my diabetes is the one thing I can't seem to get under control.

04:07:07.045
Maddie:
Thanks, Jennifer. Sharon.

04:07:12.831
Sharon:
Overall, I think that diabetes made it up there because it caused all kinds of complications so I can call my diabetes as one of my burdensome symptoms. My current treatment is managing my diabetes ok, but I still have to work really hard in order to keep it stable. I need to exercise a lot. I eat only twice a day, only veggies, only protein, and every time I eat outside of these meals when I eat some more carbs or sugar or I don't get to exercise as much, I immediately see it with the glucose levels and immediately see the A1C goes up. So I would say that my current medications are doing an OK job, but they still need to work really hard to keep it stable.

Andra:
Are you able to exercise all the time?

Sharon:
No, for the past 11 months, I wasn't able to exercise because I had a foot surgery and it got complicated so I had 6 surgeries after it including one infection. And I could see my A1C going up and up and eating 2 meals a day not exercising after surgery. It's really hard to keep those day-to-day management that you need with our disease.

04:08:39.084
Andra:
One more quick follow up. When you say exercise, can you tell us a little bit about what that means?

Sharon:
Yeah, I used to do seven days a week. I used to exercise seven days a week. Um, four times I would do weight lifting, heavy weight lifting and then three times at least cardio, at least 50 minutes each morning I would go. I have a personal trainer who was with me for the past seven years since I got diagnosis, the diagnosis of diabetes and, and yeah, that, that seems the only thing that can keep me stable.

04:09:19.811
Maddie:
Thank you. And we'll go to Rowan, who has a comment from online.

04:09:28.246
Rowan:
Regarding diabetes management online, Antonise shared medication management for diabetes has been really hard, especially during the pregnancy for me. When I was on a GLP-1, it was going great, but I had to stop and start insulin which was very hard to manage daily. I was taking six injections daily.

04:09:46.705
Maddie:
Thank you. Briana, did you have something?

04:09:58.861
Briana:
So one of my biggest things is pain and then exhaustion. So the unfortunate thing is your pain medications cause drowsiness, cause you not to be completely and totally aware in your head. So that causes more exhaustion and then it's a whole cycle. So one of the things that I do is I use cannabis cream because I have to. The problem is I can't take that on a plane because it's not legal everywhere so I have to try to manage my pain without it, which makes me groggy, which gives me exhaustion, which gives me pain, and it's just a whole horrible cycle.

04:10:39.895
Maddie:
Thank you. Is there a hand over here?

04:10:50.385
Robin:
I will clarify that Russell's biggest burden is diabetes and he does take a GLP 1 and 500 U 500 in his pump every other day in addition to the leptin, the metreleptin. So it takes all three of those things plus a handful of medications to manage all the symptoms, but I think the the strategy that's burdensome is when you're hungry all the time, it's hard to manage your sugar and that's probably pretty obvious to everyone, but it is difficult when you eat so much to manage high sugar. Thank you.

04:11:43.660
Maddie:
Other comments? Yea, one second, one second. Thank you.

04:11:48.061
Kristina:
I'm going off treatment so we've been trying to find different ways to treat this and I find it so difficult because you can have everything planned out, plan your food. I'm going to exercise, do all this, but if you can't get out of bed it's kind of hard to maintain that. So you just get stuck in that cycle because even if you have the best intentions, if you can't get out of bed, it just throws everything off. That's difficult, I think to, when you're feeling fatigue and pain and all this stuff to just, yeah, I'm gonna go exercise, I'm gonna do this, I'm gonna do that. Oh, yeah, yeah. And it's just it's very hard to actually manage all of that when you're feeling the way we do.

04:12:38.274
Maddie:
Thank you.

04:12:45.582
Cori:
So to address the triglyceride side. It becomes a mental game because what do I put in my mouth that isn't going to affect a triglyceride? What is the? Do I eat this or do I eat this but then it almost becomes a, you have an unreal healthy relationship with food because you're terrified, with the triglyceride side of things. So for me personally, I do not have a healthy relationship with food at all. I could care less if I eat. I'm one of those patients that I don't feel hungry all the time. Now that my treatments are working, I don't feel hungry all the time. So like even if you haven’t eaten lunch. I didn't wanna eat everything because I'm like, if I eat all of this, triglycerides are gonna go here. That'll affect my heart. So it's become a battle of mentally like healthy food, I still don't care. I rather just not eat because that's healthier than putting something in my mouth in my mind.

04:13:56.536
Maddie:
Thanks.

04:13:57.702
Andra:
Cori, yeah, that really, yeah. That resonates with me deeply. Yeah, deeply. Go ahead.

Maddie:
I guess related to that, how do you, when there are trade-offs between, you know, eating or managing those numbers, how do you make those decisions when things are seemingly in conflict with each other?

04:14:17.223
Cori:
Right now, not great to be completely honest. I've actually had to go back to therapy because I'm headed down a very dangerous path of anorexia. I only weigh 97 pounds. And so, umm, some of that's due to medication, don't get me wrong, but a lot of it's due to the fact that I don't, I'm terrified to eat. It is literally every day a mental battle of like, you gotta eat to live, to keep your diabetes up, to keep your body going. But. I just, no dietitian, no doctor, nobody can just tell me like you can have this, you can have that. No, it's a mental battle and it's a struggle. And so yes, I'm seeking the correct help, but it's, it will probably be a battle for the rest of my life unless we can come up with a solution for triglycerides. So.

04:15:12.424
Maddie:
Thank you. Thank you, Cori.

04:15:15.042
Andra:
Yeah. We're hearing specific stories and we're focused on those. But I do want to say that there is a significant amount of eating disorders that we hear about in the lipodystrophy community for that reason, it's that the relationship with the food noise is really challenging. Yeah.

Maddie:
Thank you for sharing. We'll take one more comment from Eileen.

04:15:36.127
Eileen:

Hi, thank you. And I completely understand that scientifically the question would be worded, you know, the most burdensome aspect and so on, but. Ok, but in Meaghan's case the Monjaro and working with Dr. Brown at NIH has been life altering, miraculous as Meaghan reports. Helped with the food noise and the weight loss and Dr. Brown worded it was her diabetes in remission so in terms of the therapy working. That's miraculous. But the pain I cannot say that. That OK, check off #1 and #2 is pain and #3 is gastro problems and #4 is mental health. They're not in a row, you know they're equally burdensome, so I don't want it to sound like. OK, you got rid of the most burdensome one, so let's now address the next or less burdensome or the least burdensome. They're all equally burdensome. It's just some things can be treated and others can't. Her pain is completely out of control and unmanageable. Muscle pain, bone pain, pain from exercising. And she takes Remicade infusions. And that's a burden to go get, you know, she travels an hour to every doctor in all different directions. So she has to choose. Well, I'll have to cancel the cardiologist this month because I have to get my injection, my infusion, and then I'll pick that up next month. So I know it has to be reported back as most and least, but they're all just as bad.

04:17:30.283
Maddie:
Thank you, Eileen.

Andra:
Thank you for calling that out. It's really difficult I think in any disease, but a multi-system disease like this, it's particularly challenging. Thanks.

Maddie:
Absolutely. We're going to move now to our next poll, which is asking us to think ahead about potential new treatments and associated side effects specifically. What degree of side effects from a new treatment would you or your loved one be willing to accept if the treatment addressed an important aspect of your condition?

04:18:26.003
So roughly 65, 68, 60 percent around high. 30 ish percent around moderate. 5 to 10-ish percent, 10% around low. But a range and representation across each of these areas. So this poll explores side effects. This next question for the group, it sounds similar, but we're specifically focused on safety risk. So what degree of safety risk from a new treatment would you be willing to accept if the treatment addressed an important aspect of your condition? Yeah, Kara.

04:19:15.237
Kara:

So Kara again and familial partial, I would accept any because I am not on any treatment because I'm quote on quote not sick enough. So, honestly anything and everything my husband will tell you different. But I'm willing to accept anything that is available at this point just to get some type of relief in a normal life. Also, I'm doing this also because I am one of the lucky ones that have three beautiful children and God forbid that one of them have this. I'm willing to take on the risk so that maybe in the future, if one of them do have it, they don't have to go through what I had to go through.

Maddie:
Thank you. Yeah.

04:20:12.424
Christine:
Hi, Christine with FPLD2. I started getting eye injections for diabetic macular edema four months ago. I get them once a month. Because if I did not, I have cysts, fatty cysts on the back of my eyes along with inflammation, so the risk of taking the injections is blindness. But so is the risk of not taking the injection. So I'm taking the injections.

04:20:55.890
Maddie:
Thank you.

04:21:06.536
Briana:
Hi, Briana again. So the degree of safety risk that I would take for a new treatment would be absolutely anything because I have children and grandchildren with this and when I first started this journey, if that's what you want to call it, I was told by my son that he did not want me to take any treatment, that was, that he didn't know about. He didn't want me to have those side effects. He didn't want me to possibly die. He now has children, so he now understands that, you know. You're when you have your children, you are willing to do whatever it takes. And that's exactly where I'm at. And he now understands that.

04:21:52.066
Maddie:
Thank you. We'll come to Gretchen. In second round.

04:21:54.660
Gretchen:

I think for some of us that have seen family members pass. I was my father's caregiver. Then we thought I was the lucky 1 because I didn't have the kidney issues, but knowing and seeing what they went through that could be me and also my son, so, anything's worth it for my son.

04:22:25.100
Maddie:
Thank you.

04:22:35.027
Crystal:
I could have never seen myself being part of a clinical trial. But being drastically ill, just never ending sickness and feeling like absolutely nothing was working made me really self assess and say OK, nothing else is working and I need to take this jump. I need to try something and hope and pray that it works. And I'm very fortunate that it has. But I would have never seen myself in a clinical trial. It took me getting deathly ill, feeling like I was going to die or I needed to try something different.

04:23:22.190
Andra:
Maddie I'm going to take my second card, I'm gonna pull up my second card, take off my moderator and answer this and wanna reflect on something Maryanna was saying about the worry of trying something and then losing access to it, feeling good. I very openly am a patient with partial and have had expanded access to metreleptin for 11 years and my number one burden growing up from 4, 2, 3, 4, on was hunger and I would take significant risk if I had to go back to that level of starvation. I have absolutely no interest in existing with that level of starvation, it's that severe. So yeah, I would. I would risk losing 10 years of my life if I knew that the years until then were without starvation.

04:24:24.053
Maddie:
Thank you, Andra. We're going to move to our next question. We're back. We were thinking ahead, we were thinking about potential future treatments. Let's come back to current treatments. What are the most significant downsides to your current treatments and how do they affect your daily life? Examples could include going to the hospital or clinic for treatment, time devoted, restrictions on driving, things like that. There's a real range here. Let's go to Ananda in the back.

04:25:00.962
Ananda:
Hi, this is Ananda. I have a partial familial and I'm from Massachusetts. I would say from my experience, the most significant downside to my current treatments is the lack of. There's not

enough opportunities for me to try things or ways that I can positively, from an educated place, from my doctors, from a clinical perspective to help me positively affect my daily life. And I don't believe that I can't get better. And that's why I'm here today. I absolutely believe in this entirely. Thank you.

04:25:53.231
Maddie:
Yeah, Serj.

04:26:01.317
Serj:
So I do an Immunoglobulin infusion called hyzentra through colisis and it's supposed to help with my pain and mast cell activation syndrome and dysautonomia, and it does for a little bit. But the entirety of the week basically is also plagued with the side effects of the actual infusion, which is, oh, you're going to feel so much worse and then you'll get a day of maybe not passing out. And then you have to take the infusion again. And then I'm also with a GJ tube, which I suppose would be part of my treatment for my gastroparesis. That thing lands me in the hospital so much. It falls out it gets, you know, comes out my throat because I throw up so much it gets displaced. I have to get it replaced all the time and so it has some of these treatments while absolutely necessary, and I would, maybe not the Hycentra, but that would make me miserable without it. And I have been living for a while without it. But the, uh, the GJ tube, I would die so it, without it, I would be miserable or dead and, with it, I'm miserable and in the hospital.

04:27:29.583
Maddie:
Thank you. Yeah, we'll go to Maryanna.

04:27:36.453
Maryanna:
I thought of a little better answer to what I said earlier. So one of the downsides to exercising a lot is in June I had breast cancer removed and the surgeon recommended that I take two weeks off the gym. I took one day off because of fear of not and how I would end up if I didn't. And I also wouldn't accept help with meals or anything like that because of what people might give me and I didn't want to seem ungrateful and not able to accept other things so I felt like I really couldn't do a standard recovery, even though I might want to.

Maddie:
Thank you. We'll come here, Kristen.

04:28:24.094

Kristen:
I'm here, Kristen. So one of the hardest parts of the treatment for me is the daily injection. Even if it only takes a few minutes, mentally it still feels like a lot because I know it hurts due to the lack of fat that I have in my stomach area and it just over time gets tiring doing that every single day. And then the past few years I've had three elbow surgeries and because of that, I couldn't reliably inject myself. So I had my roommate help me for weeks, but obviously she wasn't always home. Umm, so sometimes I try on my own and fail, sometimes I considered calling another friend, and sometimes I would have to just wait until my roommate got back, which could delay my dose by hours. I was really grateful for her help of course, but I hated feeling dependent on someone else and knowing that my treatment could also affect her schedule.

04:29:14.576
Maddie:
Thank you. Crystal.

04:29:18.123
Crystal: The newest problem that I have been facing is sugar level lows. I've never had lows ever in my life and with this new treatment, I've gotten to experience a severe low to where I need help and I can't do it for myself.

04:29:39.128
Andra:
Crystal, can you explain what you mean by low?

04:29:42.611
Crystal:
Where it's kind of like ski sloping. So I may be like in the lower hundreds and then all of a sudden I feel like I'm dipping.

Andra:
So you're talking about your blood sugar?

Crystal:
My blood sugar, my glucose level, yes. Managing how much activity I can do and eating because I am on a GLP-1 and leptin helps me as well not wanna eat as much. The newfound activity with the less amount of eating and it's scary thinking about being caught and not being able to help myself because my sugar goes too low.

04:30:23.144
Maddie:

Thank you. We'll come to an online response.

Andra:
Oh, great online response. And then do we have time for Becky? Yeah, OK, great. Great.

04:30:36.115
Kate:
Nikki says I'm so tired of every treatment being gut centered with its side effects. The nausea, vomiting, diarrhea, constipation and in my case ulcer are just as isolating and burdensome as untreated lipodystrophy has been for me historically. Rachel says, very time and energy consuming, large financial burden, social cost of restricted diet makes traveling difficult. Lack of availability for spontaneity and social opportunities and one more. Managing the prescriptions of numerous daily pills, having to swallow 9 pills with a meal, traveling with multiple daily pills, managing multiple doctor's appointments, labs, etc. All of this has had an impact on daily life and requires high level organization and time.

04:31:19.131
Maddie:
Thank you and thanks for the folks sticking with us on Zoom. We know this is a long day to look at the screen. We're so glad you're here. Becky.

Becky:
Thank you. Yes, Becky, FPLD2. So managing this condition is a full time job. You never get a day off. It is absolutely exhausting. As other people have already said, you can't be spontaneous. You can't stay away from home. You know, on a whim. You have to plan everything meticulously. When I travel, half of my suitcase is taken up by meds or the medical paraphernalia associated with being able to take those meds. I'm on metreleptin, so that needs to be kept cold. So travelling, particularly if you're flying, trying to keep things cold is really difficult. And you know what happens, I can't, I can't put my medication in my suitcase if it's going to be checked because what would happen if they lost my bag? And so you're kind of carrying all this stuff with you. And often, you know, they'll say when you get to the check-in desk. Oh, well, we want to stow your bag. Well you can't stow my bag. You can't take my medication off me. I need it with me. And so it just makes everything so much more difficult. And as everybody said, you know, the amount of pills you're taking, fistfuls of pills a day, you're taking multiple injections, it takes time. It takes a lot of time to do these things. Several years ago I had bilateral carpal tunnel surgery and couldn't inject myself and my mum came to stay with me for a few weeks and she had to do it for me. Now I was diagnosed at 17, so just before I went off to university. So she never had to deal with it when I was a child and she was shocked at how much time it took and everything that I had to do just to keep surviving on a daily basis.

04:33:10.937
Maddie:
Thank you. We're going to move now to our last poll question. We're back to thinking about new treatment options. Clinical trials have come up a couple times, but let's get some data through this poll. Of the following, which is the biggest barrier preventing you from participating in a clinical trial? And we recognize multiple of these might apply, but we're asking you or your care partner to pick the one that's the biggest.

04:34:02.680
Andra:
We've heard a few times in the room today the inclusion,exclusion criteria. Folks would like to participate but do not qualify. They're either not sick enough or too sick, however we define sick.

Maddie:
So let's dig into this. And I'm going to call an audible and go a little bit out of order, let's talk about. Let's keep talking about clinical trials. Have you ever been screened for a clinical trial? If yes, were you enrolled and what factors influenced the outcome? If you've been waiting to join the discussion, this is the 3rd to last question that we have, so we hope to hear from you.

04:34:53.016
Kenzo.

04:34:56.815
Kenzo:
Hi Kenzo, CGL, so I've actually been a part of two clinical trials now. The first one I did was with myalept. I was on leptin before myalept was approved, that worked out very well and then I switched over to miba, but which is what I'm currently on, three injections once a week. The factors that influenced it is with the leptin, hearing from Dr. Brown and the team about how it was supposed to work and how it should do to my body. Really made me want to try it because at that point I was 14, was dealing with a lot of different things health wise, and I'm just like, do something that works. So I jumped into that. And I will say at the beginning, I wasn't fully bought in because I had the medicine, I wasn't taking it correctly. But once I actually saw that it was working, then I got on the roll. And then with this new one, miba, the one thing that really influenced this one was the fact that it was once a week, still three injections, but once a week.

04:35:54.930
And honestly, they have, it has done just as well as Myalept did for me. The one thing about that one, it's just like, since you're only doing it once a week, I feel like usually by day 5 is where you really start to feel like, hey, the medicine's not really there anymore. But yeah.

04:36:12.119
Maddie:
Thank you.

04:36:18.199
Andra:
Just confirming. It was the convenience, right? The second time was the convenience.

Kenzo:
Yeah, absolutely.

Andra:
Just want to make sure I heard that right. Thanks.

04:36:27.721
Maddie:
Jennifer.

04:36:32.339
Jennifer:
So I've had participated in two clinical trials and I just, I used to be anti-clinical trial. I didn't want to be a test subject, but my aunt had passed and right after that I knew something had to change or it was gonna be me. So I reached out, started a clinical trial and I was enrolled and did participate in them. And the factors that would have influenced the outcome was everything was paid for. The travel and everything cause I travel several hours away for a clinical trial and if that wasn't paid for I wouldn't be able to do it.

04:37:06.167
Maddie:
Thanks, Jennifer. We'll go to Serj in the back.

Serj:
So I've been in one, enrolled in two. The one that I, I actually had success in was with Dr. Oral, the one that Mallory was talking about. And I also developed antibodies and that was devastating. But the other one was I was too young for and too sick for and that was what we were just discussing with the, you know, not allowed, and that was also devastating.

04:37:47.254
Maddie:
Thank you. Crystal.

Crystal:
So shortly after my diagnosis, about a month or two after my diagnosis, I got screened for a clinical trial. But I was doing so well I thought that I would live my best life on a GLP-1 until I lost access to that GLP-1. And all it took was missing three doses for me to completely spiral out which led to multiple hospitalizations and it was like four months continuous back-to-back. I got very, very ill. And felt like I needed something and I was able to get well enough again to be able to try to get into a clinical trial again and that's when I made the cut and from where I'm at now.

04:38:48.729
Maddie:
Thank you.

04:38:53.002
Andra:
Does anyone have any thoughts about placebo? Or not placebo. I know that was one of the considerations on the last poll, I was just curious.

04:39:05.611
Kate, is there anything or Rowan, is there anything online about placebo or not placebo and influence? Kara.

04:39:16.810
Kara:
For me, I wouldn't care if it was placebo or not placebo. I would do it because my ultimate goal, like I said before, is to find something that the people that come after me don't have to do this. So, I'm willing, literally willing to do anything to help further medication on lipodystrophy. I was in one of the clinical trials where doctor Br. don't kill me, I don't remember what it was, but I didn't care if it was like the real drug or not, like I just wanted to be a part of something that's bigger than who I am.

04:39:55.078
Maddie:
Thank you, Kara. One other way to think about this question, especially if you haven't disqualified for a clinical trial to date, is that if you theoretically did, think about what factors would influence your decision to participate and what concerns might make you decide not to participate, if that's helpful. Yeah, we've got someone in the back. Great.

04:40:16.458
Shannon:

Hi, this is Shannon, I'm FPL and from Wisconsin. I would not care if it was a placebo or not. Best case scenario you have something. I guess worst case scenario is that you know, you feel the same way that you do now. And the best case scenario would be that you get relief of your symptoms and help with your health, but then there's that fear of, what if I'm able to get on this medication and then it does something for me and then I have no access to it afterwards? So that's a huge fear also.

04:41:04.484
Maddie:
Thank you.

04:41:06.274
Andra:
We've got Jil over here.

04:41:12.074
Jil:
Ok, so, we were screened for two, enrolled in two, but I'm only going to focus on the 1st. We're screened. It was great. I'm glad you can, but, at this moment you don't qualify and I know a lot of patients have been like, ok well, I don't qualify. I'm just like, well, sorry, sad, walk away. But unfortunately I won't call the doctor's name because she knows who I am. I'm more of the ok, well, I'm gonna challenge it. Today I'm not qualified, but what do I need to do to get qualified? Yes, she doesn't meet the medical requirements to be in the protocol but is there a way that we can change a narrative so that she doesn't have to wait until something goes wrong to then be involved. Fast forward, it took us a minute. Well, it took a year and a half for her to get in, but. I guess if you want to say what influenced the outcome, I did. But at the same time, what forced me is to make sure that at the end of the day, my kid had the best possible option out there.

04:42:31.206
Andra:
Yeah. Thanks Jil. Before we call on you Becky, I want to comment on that because I think there is a difference in the rare disease community, but also, you know, lipodystrophy is whether or not you're advocating for yourself or you have someone advocating for you. I think we hear that quite a bit. It can be really difficult to advocate for yourself and having a Mama bear or a Papa Bear, someone advocating for you is really helpful. We're gonna go to Becky and then you, Mal.

04:43:05.826
Becky:

Yeah, I think it's very difficult because people are desperate because there aren't enough options available and the ones that are available are not specific to lipodystrophy and we need more options. Unfortunately, in my experience, some of the trials that have been available to people in the UK have not been designed with the patient in mind. So they've been designed by researchers and clinicians who mean very well, but it's not talked to the patients to see what is suitable and what is not. And one particular trial I'm thinking of is a meal replacement trial. So basically a milkshake 800 calories a day. So you're asking patients that have extreme hyperphasia to not eat solid food and only get 800 calories a day. Now to me, it's absolutely insane. And if they asked patients about that, in the beginning of the design stage of those trials, it probably never would have got to a real trial. I think the results have been mixed, again because the compliance issues with that are significant and frankly

04:44:26.223
the misery of dealing with hyperphasia and then not being able to eat is not worth the burden in this instance. For most of the things I would try anything. Particularly at the risk of losing something that's currently working, the thought of going back to what I was pre-treatment is unfathomable and terrifying and to be able to have multiple options and for future generations is so important that I would try almost anything.

Maddie:
Thank you, Becky. We'll go to Mallory.

04:45:04.450
Mallory:
Hi, I'm Mallory. I've been on a few different clinical trials. The first one was leptin and I created antibodies against it, so I had to go off of it. But I was 12 years old when that started and I didn't really understand, all I wanted, all I focused on was my legs and my arms were too skinny and my stomach was big. My body image was the only thing I was thinking of when I was 12 years old. And so I really have to thank my mom for advocating for me and Dr. Oral, my mom and Dr. Oral, they're like both my mom's. Without them, I wouldn't be alive today and the advocacy that my mom has done for me, I cannot thank her enough. She's amazing. Yea, thank you mom.

04:45:58.744
Maddie:
Thank you, Mallory. OK, we're gonna move.

Andra:
Yeah, I'm gonna make one comment about that, just following on my last comment. And we're not here to talk about diagnosis, but if most people are not diagnosed til they're much later, they don't have their mom to advocate for them and help them guide through those decisions. And I

think that's something that is a big factor is really this is a disease that impacts children who don't really know about it until later. So that is tricky. We're so thankful for your mom too.

04:46:31.860
Maddie:
Awesome.

04:46:33.554
Thank you all.
OK. Second to last question. What worries you most about the safety, side effects or long term impact of the treatments you currently use or have used for lipodystrophy or related conditions, including any treatments that you had to stop because of these concerns? And again, this applies to you and your loved ones. Yeah, I'll start with Serj in the back.

04:47:02.562
Serj:
Sorry to keep hogging the microphone, I'm worried I hear bits and pieces about GLP-1's wasting muscle and I already deal with that in the sense of fat being removed from my muscle and then that wasting. And I don't want to lose any more from my limbs because I'm already weak enough. Like I'm strong in some ways but some days I am like I can't walk. So I, that worries me. I don't know if anybody else here knows anymore about that, I'd be interested to hear, come find me, but that scares me a bit.

04:47:50.095
Maddie:
Thank you.

04:48:02.076
Ananda:
So as far as safety is concerned. My biggest, something that I've always had a concern of is my coordinated care. I also have a child that has lipodystrophy. I was blessed to be able to be diagnosed and find some really beautiful people. Very well educated people, but I'm always concerned about lack of data. I wish that I was studied more. I wish that I had the ability to wear more monitors and more scaling and I was able to show up more and be more proactive. I do believe in the science. I do believe that I can feel better.

04:48:54.975
Andra:
By monitors, do you mean wearables?

Ananda:
Yea, like wearable monitors or having like a visiting nurse to check my sugars for me and, you know, healthcare, health insurance that I'm able to access these things through because I'm it's debilitating, I'm exhausted, and it's progressive.

And being able to understand the right language for my symptoms and being able to communicate and be able to be educated as well. And not just like show up to my doctors because I get forgetful. You know, there's a, there's a whole part of lipodystrophy in my case that I have asked my doctors about in regards to like my memory loss and lack of study and how it affects my brain. Like I am definitely willing if you know there are clinical studies requesting and the awareness of that, like I am here for a reason and I have a child and there's a generation that's ahead of me and like I definitely am concerned about that, that part of yeah, my treatments. I think that I could show up more.

Maddie:
Thank you. Thank you so much. We'll take Becky in the front row.

04:50:17.614
Becky:
Thank you. Yeah. So I think that like most people here. The alternative, I know treatment is far worse. And so I'm willing to put up with a lot of things. And that's not to say that they're not burdensome, but the comparison, you know, compared to not having treatment, it's, it's something I'm willing to sacrifice. What really concerns me going forward is either having the current treatment that I'm on that is currently working, stop working and there not to be an alternative. And at the moment those alternative options are extremely scarce. But I'm also concerned and again, this might be slightly different depending on what country you're in, but for a long time, like lots of people have worked really hard to try and keep my levels under control and every time I see the specialist doctors they tell me how well I'm doing and I always say to them, but can't it be better? What can we do to make it better? Because, you know, when I was diagnosed at 17 and when I was diabetic by 21 and they told me I was under good control and they said, yeah, but I'm only 21. What's going to happen when I'm 30, when I'm 40, when I'm fifty? I've got a lifetime to live with this condition and to develop the side effects, the comorbidities that come with this disease. And so just managing well, it's not frankly not good enough. I want to do better and at least the way that we are treated in the UK, it's, and I think it sounds like it's the same in the US. You know, if you're not sick enough, we're not going to do anything about it until you are sick enough and by which point it's too late. And actually it's much more cost effective to treat these things upfront in advance to prevent the development of some of the worst comorbidities than it is to treat that once it's developed.

04:52:20.995

And about the quality of life that comes with that, you know, managing it in advance means that I don't have to live for 20, 30 years with, you know, organ disease, damage, failure and everything that comes with that. So my biggest concern is the availability of treatments and then the access to those treatments and they need to be multiple because as if you're not everything works for everybody and even if they do, they stop working sometimes.

04:52:50.113
Maddie:
Thank you. We're going to go to Rowan in the front row with a comment from our online community.

04:52:58.510
Rowan:
Two comments from the zoom Melody mentioning I think my biggest worry is losing the GLP-1, losing control of my A1C and being hungry all the time again. And I can't worry about what might happen in the future because of treatments because I'm too busy dealing with the current ones. Also mentioning dealing with memory loss as well. And then Wakiko referring to her child with lipodystrophy, I was worried about the unknown long term impact of starting leptin at age 8 months old and continuing it potentially for the rest of my child's life there is no one else who had started that young and I just had to trust that it may work and we are making the right choice for the baby because no treatment was worse.

04:53:40.392
Maddie:
Thank you. I'll come down to the front row.

04:53:48.684
Eric:
Okay, so when I think about the long term impacts and side effects for Kristy. And we think about the safety of it. I think our concern

04:53:57.963
stems around just the knowledge base around these medicines and what they can do for patients. We're in a room right now where there are multiple people who have been on metreleptin therapy for a decade. Unfortunately, my wife went to the doctor who had prescribed it for multiple years last September, without any explanation she was just told oh well, I'm concerned about the long term impact. And he took her off. In the last nine months, she has been to the ER twice. And I have heard the stories of all the people on stage. And I see all of the indicators in Kristy and I know that she is heading down that road.

04:54:40.449
She is now in a position where she has to prove that she is sick all over again. Because one doctor didn't have the courage or the tenacity to reach out to other doctors to confirm that it is safe that there are other patients and so we were unable to continue her medical necessity. And that's what we would like help with today.

04:55:06.234
Maddie:
Thank you so much. Sharon.

04:55:13.129
Sharon:
Personally, I am willing to take a lot of risk to get treatment for my lipodystrophy, specifically for my lipodystrophy and not just the symptoms, and I'm willing to do whatever I need. But my life is not just my life. I have a family. I don't have kids, but I have an older mom. I have a sister who has lipodystrophy as well. And every time I have a complication from my treatment or from my other symptoms, they need to stop working and take care of me.

04:55:42.664
So the impact is on me, I'm on disability. I'm not working. So it's less on me, but my sister has to stop working. She needs to take me to meet my physicians. She needs to stop working so she doesn't get any money on those days. So it's not just me, it's my whole family that is involved with those risks. But for my health, I will take any risk.

04:56:13.468
Maddie:
Thank you, Sharon. We're going to go ahead and move to our last question? If you've been waiting for your moment to speak, this might be it. Also recognizing this isn't it because the docket remains open after this for things that may come to mind after or things that you didn't get a chance to say in full today?

04:56:38.562
Andra: Kate, really quick. Since we've mentioned the docket, how long is the docket open? Let's give folks.

04:56:47.470
Kate:
Two weeks

Andra:

2 weeks, 2 weeks.

04:56:51.358
Maddie:
Great. OK, Our last question today, what aspects of your lipodystrophy would you most like to see a new drug treat and short of a complete cure, what would you consider a success or meaningful improvement? For example, changes in specific symptoms, daily function or quality of life. We'll start with Gretchen.

04:57:15.750
Gretchen:
I, one of my biggest things for us is we don't really have a pain control. If you go and tell them how much pain you're in, you're assumed you're an addict trying to get your pain medications. Tylenol doesn't work for me. Ibuprofen doesn't work, there's just no. I understand why it's hard to get a pain medication, but I think with some conditions it's. Just give us grace and mercy and allow us to use what we have available to get rid of the pain.

04:57:52.098
Maddie:
Thank you.

04:57:53.958
Thank you. Serj

04:57:58.063
Serj:
I think going off of what you just said with the pain, I think this would come along with something like that, but something for mental health, something for that brain fog, something for that memory lapse, for the whirlwind. I can't think and my thoughts are all over the place. The I don't know. I mean, I've, I've been reading about possible demyelination being involved with certain forms of lipodystrophy and that needs to be more researched, first of all, it needs to be more common knowledge. But it also, I mean, maybe we need something for the neurological aspects of this disease. I think that really needs to be researched and, and something needs to be given for that. Something for the small fiber polyneuropathy, something for the MEC myalgic encephalomyelitis that I think a lot of us may have. You know, it's, it's there's. We need stuff,

Maddie:
Serj. I'm going to follow up. I'm going to follow up quick on that. Thank you for that answer. When we think about what would success look like, what would a meaningful improvement? What would the before and after be? Can you say more?

04:59:07.585
Serj:
Happiness. I think it would just be happiness like not, not transient happiness, not like, oh, I have a day here or there that I'm able to breathe through the pain and smile, but like I'm actually living a life like other able bodied folks you know that they take for granted.

04:59:31.445
Maddie:
Thank you so much.

04:59:37.815
Rebekah:
I'm just gonna say excuse my voice everybody. Rebekah with partial umm, I think for me it would just be that the treatment would treat the disease as a whole. Umm thankfully I have been successful on treatment, but we are realizing that it doesn't treat the disease as a whole. So we're having to add extra aspects, you know, a GLP-1 other medications, umm So I would just like it to treat the disease as a whole.

05:00:07.931
Andra:
So work with fewer medications?

05:00:11.224
Rebekah:
Yes.

Andra:
OK. Just wanna make sure I heard that right.

05:00:16.627
Maddie:
Mallory.

05:00:22.627
Mallory:
Since I spent most of my high school time in the hospital with pancreatitis, I just have the fear of getting pancreatitis every single day. And I think a meaningful improvement, which is not having that fear that something's gonna go wrong, that I'm gonna be in a lot of pain and I'm not gonna be able to do anything about it. And I just want to live my life and not worry about any flare ups.

05:00:54.910
Maddie:
Thank you. We'll come to Cori in the 2nd row.

Cori:
I think if we could stop doing the Band-Aid thing that would be great. Because every new problem gets a Band-Aid put on it. So if we could find something, I mean I would go for a complete cure. I mean if y'all can do that, I'm not gonna object. But, the Band-Aid thing needs to stop. Because that's just not successful. It's hemorrhaging at times. It's, sometimes it's a cut and sometimes it's a hemorrhage and the Band-Aid thing needs to stop.

Andra:
Can you clarify what do you mean Band-Aid by traditional therapies right now?

05:01:50.553
Cori:
Traditional therapies to try this medication, to try this test, try this surgery. It's just a constant. And I know doctors are trying their best, I know that without a shadow of a doubt. But sometimes I feel like they're just throwing a Band-Aid on and trying to make you not hemorrhage.

05:02:00.430
And sometimes I wish we could just figure out, OK, how do we stop the Band-Aid action and come up with maybe a, a full cure or a drug that works 95% of the way or whatever. I would give anything just to have an hour, one hour.

05:02:20.245
Andra:
One hour of what normalcy?

05:02:22.821
Andra:
OK, so look like

Cori:
I'm sorry?

Andra:
What does normalcy look like?

05:02:28.197
Cori:
I would love to be able to sit on the couch after a full day and with my husband and watch a movie and not fall asleep. And then having him explain to me what happened in the movie was over.

Andra:
So one hour of that, would be a success?

Cori:
Just one hour of that, one hour of quality time with whatever I wanna do, whether it's with my husband, a friend or just me time because, you know, everybody just needs some me time. So whatever. An hour would mean the world to me.

05:02:59.935
Maddie:
Thank you. Sharon.

05:03:02.722
Sharon:
Yeah, just Cori, I have that the same answer for me, short of a cure, just getting some time like one hour, 2 hours a week even, to do my hobbies, which I stopped to do chores around the house and not getting my sister come and help me clean the house or clean the yard. That would mean the world to me. Right now, I stopped doing stuff just because of the fatigue. I cannot get myself out of the house because. So even two hours a week, that would be an amazing change for me.

05:03:38.513
Maddie:
Thank you. Go to the back in the orange. There yeah.

05:03:49.450
Shannon:
Hi, I'm Shannon again. Partial. I guess the biggest thing is cardiac, right? You kind of need your heart to live. So something to definitely treat the cardiac. I don't feel it on a daily basis but it's always in my head that what if you know what if something bad were to happen and I just became a new grandma, so four months old Lincoln, you know, I want to see him grow up. So that's scary, but I don't

05:04:27.347

for me personally, in my day-to-day I don't remember what it's like to not feel tired and to not be in pain. So having freedom for that selfishly. Yeah. Would be unbelievable, would be, the best.

05:04:50.373
Andra:
What would a meaningful reduction of that look like for you?

05:04:59.303
Shannon:
Can you repeat that? Brain fog.

05:05:01.173
Andra:
What would a meaningful reduction of pain and or fatigue look like for you?

05:05:08.899
Shannon:
Honestly, I don't know. Because I feel it every day I. I honestly don't have an answer for that, I guess anything. Anything would be an improvement.

05:05:25.999
Andra:
OK. Thanks. Yeah. Thanks for letting me put you on the spot, Shannon.

05:05:30.192
Maddie:
Yeah, Ananda.

05:05:35.275
Ananda:
What I would love to see is my memory return. I would love to see me graduating from college because I haven't been able to focus enough and have the energy or the confidence in myself enough to accomplish that. So if there was some kind of treatment to help me with my memory, that would be great. I am going to test it. Just a little side note, I am going to test it and I am going to try and go back to school.

05:06:08.403
Yeah.

Maddie:

Thank you,

Ananda:
memory.

05:06:11.624
Andra:
Totally. And so we have about 4 minutes before we get a closing remarks.

05:06:18.900
Kenzo:
Hi Kenzo, CGL. I think for me, the thing that I would like to see, I've mentioned it a lot when I spoke today, is the hunger. Because I spend so much money grocery shopping. If I'm not grocery shopping, I'm stopping to get something to eat on the way home because I'm just so hungry that I'm not gonna make it to the house before I need something to eat. So something that's really going to like drive that hunger down cause then maybe I could have a little more money in my pockets.

05:06:52.834
Maddie:
Yeah. We'll come right here.

Christine:
Hi, Christine again. I would like to see something to help with the visceral fat, get it off our organs, help us live longer. I lost my mom and my grandma both to organ failure and that would be very meaningful to me because that's where all of mine is. It's on my organs.

05:07:19.465
Maddie:
Thank you

Andra:
We heard a lot today about body image. And I'm curious if anyone has thoughts about what success would look like for you with body image.

05:07:32.668
Maddie:
Yeah, Jennifer.

05:07:36.951

Jennifer:
I would say like a reduction in like fat around like some people's necks, like my neck, because this sounds silly, but like I can't wear certain pieces of jewelry. I can't put on a necklace cause they're not long enough or they choke me. And like, so if you get a gift from somebody that's like jewelry, you're like, thank you. But like it's hard to actually wear to put it on to show them. So then it makes you feel like crap. If nobody had a better word.

05:08:05.802
Maddie:
That doesn't sound silly at all.

05:08:09.311
Andra:
Yeah.

05:08:11.741
Ashley:
If I could have an butt that would be wonderful. Thank you. Just sitting on bones is not very comfortable. My husband jokes that I just have a slit in my back. So that would be wonderful for me, for everybody I'm sure just being comfortable sitting and not waking up with pain from just your bones just in your bones. It's awful. Thank you.

05:08:41.923
Maddie:
Thank you. Briana.

Andra:
Baby, don't got back, just say that.

05:08:52.322
Briana:
As far as body image. Us women actually being able to look like women. I think that would be important, especially for our mental health.

05:09:08.662
Maddie:
Thank you. We can take one more. Yep.

05:09:13.207
Rebekah:

I think for me, just being comfortable in my own skin, like I get like I can't show my body to my husband and luckily enough he's here with me to support me, but I will not show my body unless it's in the dark cause I'm not comfortable the way that I look. So just being able to be comfortable in my own skin.

05:09:35.640
Maddie:
Thank you. We are at time. Thank you everyone for all of your thoughtful responses and engagement and all of the sitting and the emotions today. I think I speak for everyone. We could not be more grateful for all that you've shared. It's beyond.

05:09:54.598
Maddie:
We're now going to move on to the meeting closing. We did it. I'd like to welcome back Kate Stratton, Executive Director of Lipodystrophy United for closing comments.

05:10:13.097
Andra:
While Kate walks up, just remind you that we have the open docket. So if you really feel you, if you really if you kind of feel, use the docket and also Give an Hour is still here with us. It was a long day. So Julie and Ashley are here. Please feel free to take advantage of that and take care of yourselves. Kate.

05:10:36.903
Kate:
Hi everyone. Hi. We made a couple slides but I don't think we're able to pull them up. I could plug it in.

05:10:51.417
Maddie:
Yeah.

05:10:58.993
Kate:
No worries. If not, they would.

Maddie:
Thanks A/V. Amazing.

05:11:15.693

Andra:
Thanks Danny and team.

05:11:18.886
Kate:
Moderators, if there's anything I missed, please jump in at the end. We were writing this up while it was happening in real time. The first half of today's EL-PFDD meeting painted a powerful picture of lipodystrophy as it affects nearly every part of a person's body, daily life, relationships, identity, and future. Across patient and caregiver stories, we heard repeatedly about unrelenting hunger,

05:11:47.082
and food that cannot satisfy that food cannot satisfy profound fatigue that structures the entire day for some and many forms of persistent pain. Participants also describe progressive organ damage and life-threatening complications, including diabetes, pancreatitis, liver disease, cardiovascular disease and other serious metabolic consequences. But the burden extends well beyond physical symptoms. We heard about years of diagnostic delay,

05:12:16.521
and being disbelieved by healthcare providers, visible and invisible stigma, fertility and reproductive losses, body image, damaged relationships and social isolation. Depression and anxiety. Fear about the future, loss of careers and financial strain. Caregiver burden, brain fog and difficulty concentrating, and treatment regimens that can themselves become an enormous daily burden.

05:12:43.946
We also heard about the uncertainty of living with the progression of lipodystrophy. Participants describe feeling as though they are constantly waiting for the next complication, wondering what additional pain, fatigue, hunger, organ damage, or unknown symptom may come and whether I quote the “sunniest days are behind us”. The polling reinforced what we heard in these stories. 87% of participants reported that lipodystrophy significantly or extremely interferes with their daily lives, while not a single participant selected not at all.

05:13:18.139
When asked which one symptom they would eliminate with a magic wand, 62% chose fatigue or pain. The words participants used to describe life with lipodystrophy included exhausting, lonely, debilitating, frustrating, overwhelming, tiring, and hellish. The first half of this meeting made one message unmistakably clear. Living with lipodystrophy is a whole life, full body burden. Meaningful treatment advances must be measured not only by lab values according to people in this room, but by whether people have less pain or fatigue, for example, experience relief from

relentless hunger, preserve their health and independence, and have greater ability to work, build relationships, participate in their communities, and live the lives they want to live. In Part two, we heard about treatment burden.

05:14:10.316
We heard that treatments often provide only partial relief to symptoms that matter to people. They may improve certain symptoms or lab values, but many daily burdens still continue for a lot of people in the Zoom space and in this room. Treatment effectiveness may not last. People describe treatments becoming less and less effective over time and repeatedly having to search for another option. Access to effective treatment can be uncertain. Losing access to a treatment that is working can mean returning to severe symptoms and simply trying to survive.

05:14:48.149
Treatment itself can be burdensome and traumatic. Daily injections, particularly for children, we've heard, can cause pain, fear and distress for both the patient and caregiver. Also, refrigeration planning your schedule around medication is a burden for some. Managing lipodystrophy often requires many medications. Many different types like oral injection. Diet and exercise require significant daily effort, but maybe won't adequately control the disease or its symptoms.

05:15:23.340
Patients experience blame and dismissal. Delayed diagnosis delays treatment. Years spent sick without appropriate recognition of treatment can have lasting physical, emotional and developmental consequences for people. Treatment creates a substantial financial and time burden. People expressed significant time spent at appointments, managing their medications, monitoring and daily disease related work. This is ongoing fear. There is ongoing fear about what will happen next. People describe anticipating treatment failure, disease progression, or reaching the point where no treatment options might remain for them.

05:16:11.385
Clinical trials are not accessible to everyone. Some people who want additional treatment options cannot participate because they do not meet eligibility criteria or are considered not sick enough. Ultimately, people describe wanting more than survival or baseline functioning. They want the freedom to live longer, participate in family and community life, and spend less of each day managing lipodystrophy. Less pain, fatigue, hunger, fear that something will go wrong.

05:16:41.881
More time living, less time surviving. Anything else from the moderators?

05:16:48.584

Andra:
I have some closing thoughts, Kate, that I'd like to share, if that's okay Kate.

05:16:52.591
Andra:
Yep Yeah,

Andra:
Great. Thank you. Thank you. I've been leading this community for about 15 years and I wasn't diagnosed till I was 37 and I wouldn't be able to tell my story without number one the diagnosis, but without expanded access for a treatment for the last 11 years, I've been healthy enough to do that and I want to acknowledge though that because I'm healthy enough to be able to do this doesn't mean I've forgotten. And that I don't still experience the tremendous burden in coming, taking this time both emotionally to think about the hardest parts of our disease and also the physical energy it requires to be here. So thank you, thank you, thank you to the patients and family members who spent the day on Zoom with us, who dug into the hardest parts of our disease to talk about it today and to be here with us.

05:18:05.131
We promise some spark and joy for those of you who are staying with us in the coming days. But us screaming to like the universe without anyone listening is also exhausting. So I have to send a tremendous amount of gratitude to the clinicians and researchers who've listened to us with open hearts and ears and vulnerability and recognizing what we still have to continue to learn about this disease. We would not have gotten this far without all of you.

05:18:40.142
And thank you so much to the FDA for being part of our journey in seeking better treatments and approvals and really truly understanding that data in rare disease is tricky. But the patient experience and what we feel and what improves our lives, matters. And so we, we thank you for that. And to the industry in the room, thank you for fighting harder, for listening to us, for working with us, for thinking about the next stage.

05:19:14.048
We are eternally grateful to not being told that this is in our head, that this is our fault, that we should go to therapy. I should probably go to therapy, but also, I will do both. The volunteers are just, this almost a year long process to get this and make this happen and we could not do it without those who care about us, who love us, who are healthier than us, to do it, to help us, to be there. To Maddie for leading us today in this, we trusted you with our hearts, our stories, our minds, and you led us on a beautiful journey for the last several hours and I'm so grateful for that and to the A/V team who also made it happen for everyone on Zoom. This is an expensive event

physically, emotionally and financially. We could not have done it without Regeneron and Regeneron's support. We also had some support from Entos and we had some support from our own community who fought really hard to help with donations and and sharing their stories. So much gratitude.

05:20:33.915
I would give anything for this community, most any trial except for don't make me drink, only ensure it's not gonna happen. It's definitely not gonna happen, but I will fight long and hard to continue for events like this, so thank you so much. Get the docket. Don't forget the docket is still open.