NORD & FDA Listening Session: COVID19 Impact on Rare Disease Communities

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All right.

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Well, let's get started.

00:08:59.000 --> 00:09:00.000
Hello I am Debbie Drell.

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Also known as NORD.

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I am not only director of membership but care give for my sister Alex who has been living with rare life threatening heart and lung disease called hypertension she had for 22 years now.

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The agenda you see on this screen, is a format for this 60-minute listening session what's listening session if you have never heard of this, listening session are 1 to 2 hour meeting via Zoom or phone, and before the pandemic when it was safe they used to take place in person.

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And these meetings are between FDA staff ask patients, and caregivers and their advocates.

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They are typically not open to public and they are non-advisory discussions.

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This today very special and it is rare because we opened to public.

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Listening session are meant to facilitate sharing of patient and advocate prospectives on how disease treatment affect their lives.

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We have collected questions in advance through the registration form.

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For this Zoom meeting and we will answer them throughout the meeting.

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As you can tell there is no Q&A function but we let you know at the end how you can submit questions to NORD and FDA.

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On next slide you will see our mission.

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Founded in 1983, NORD is an organization federation of patient-led nonprofits at this time organizations we smooth at the time none of profits he represent

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Who are living rare disease.

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You will see our mission on the screen.

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NORD an independence non profit is leading the fight to improve lives the rare disease patients and families.

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We do this by supporting patients and organization, accelerating research and providing education.

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And driving public policy.

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I want to thank FDA for continuing to seek the patient voice in all that they do.

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This listening session is testament to commitments of FDA to the idea rare disease patients deserve to be partners and to be heard throughout the work that FDA does in drug development and regulatory process.

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Two years ago, FDA created rare disease patient listening sessions in partnership with NORD.

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We have had honor collaborate with the affairs staff on these listening session.

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Which like I mentioned before offer patients and care givers tunes to speak directly with FDA staff about what it is like to live in their shoes with specific rare disease.

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Time and again, patients and their families tell us how much it has meant them that FDA is willing to listen.

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NORD's relationship to FDA is strong.

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And our work on more than 12 rare disease specific listening sessions leads up to where we are today.

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Hosting large scaling public facing meeting on the worm public health resist of our lifetime and how this pandemic directly and profoundly impact rare disease community in America.

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NORD took the time to survey community.

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And we asked them about how covid19 impacted their lives.

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In March, of this year, NORD began hearing questions and concerns from members of the rare disease community about the impact of covid19.

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To better understand your concerns, your experiences, to help address your questions, we designed this survey for rare disease community.

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Survey was conducted twice.

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Once in April.

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Very beginning.

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And then, again in June.

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Total of 1,600 participated in surveys providing us rich quantive and qualitative data then those find from second survey published in August.

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We did very web webinar to talk about the findings.

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This stats on this screen and infographic really just snapshot on the impact of covid19.

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I can also in there time talk about how NORD can help you today navigate challenges of living in pandemic.

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I mentioned this survey, because, this -- listening session all billion covid19 impact.

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And so, there might be questions you have that are not specific to what addressed.

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There might be information that you want or that could benefit you that's not related here.

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And NORD has information and support for you.

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But next let's talk about what to expect in this listening session.

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So survey showed us the depth and breadth pandemic impact on our community if you are patient or care give living in the pandemic, I mean if you are just LIVING in pandemic you already impacted.

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But if you have a rare disease, there is an extra level this list lengthing session it was no surprise to us, that he was received incredible response.

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638 people registered TO acontinued.

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116 people, had expressed an interest in sharing their personal experience at this meeting we received nearly 100 questions.

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SubmittedED TO FDA.

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Through that registration form that you used to get into this webinar.

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So 116 people expressed an interest.

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And Covid really the way it is impacted our community very comprehensive so who do we focus and, fat time constraints we identified, in those questions, three mayor topic areas around the coronavirus and those topic areas specifically go to key division within the FDA those areas, are drug shortage, personal protective equipment shortage, and access to clinical trials during the pandemic.

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To be honest, we received so many heart felt stories, and it was a painstaking process to identify three rare disease families stories of all of stories that were shared but we identified three stories one for each of these areas.

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And in addition, all of the questions that were submitted in registration regathered them all submitted in advance of this listening session, to the FDA for their consideration.

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What you will hear today, are three personal stories, of how covid19 had devastating life changing impact on their families.

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And, their stories really capture what we think is happening across the country.

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And these stories will have FDA response interwoven so you will hear thier story and then specific FDA staff who work in this area of one of these topics they will come in and answer questions relating to that topic and directly responding to the stories.

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So, that is the format and that's what you can expect.

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So now it is my pleasure to introduce Dr. Janet Maynard from the FDA she will provide the welcome from FDA.

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And I just provided welcome from NORD.

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Janet will provide the welcome from the FDA and discuss the crucial role FDA plays in ensuring new medication for rare diseases, their safe effective outful for patient.

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Doctor Maynard thank you so much for joining us.

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>> You can unmute and, get on video.

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>> Sure.

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Thank you.

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Can you see me?

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>> Yes there would you go.

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Thank you.

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>> Great.

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Thank you.

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>> Good afternoon.

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Thank you so much for being here today.

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We are experiencing an extraordinary time for our nation and the world covid19 public health emergency is it magnitude that requires all of us to join together to find solutions.

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We greatly appreciate you being here today can TRN bike you thing your voices and perspectives to listening session.

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Today we will discusses impact of covid19 pandemic on people living rare diseases and their families.

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And in addition we will discuss FDA's response to the covid19 pandemic.

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Covid19 pandemic has broad impact from accessing medical care to conducting and participating clinical trials.

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Patients rare disease may be especially vulnerable to it is impacts of Covid.

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It is essential that we support the development of safe and effective treatments, and vaccines for covid19, during the public health emergency.

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And continue to support FDA further priorities.

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These include supporting the development of safe and effective treatments for patients with rare disease.

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And my name janet and I am the director of the office of work product development.

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At FDA.

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It is an honor to be here today.

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We recognize that it is very difficult time for people with rare diseases, and their families, and greatly appreciate this opportunity to hear from you.

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Today we will hear directly from the rare disease community.

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And FDA will responds to questions submitted to NORD.

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To help responds to these questions we are by number of experts.

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Division of rare disease and medical ge net inches and direct kara.

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And, and then Dr. Ross from

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From the center for devices and radiology health.

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And please note we work closely with patient affairs staff, and also consulted with experts in our center for evaluation evaluation and drug shortages staff as he was were preparing for this webinar.

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This webinar just one example of how three medical product centers and offers commissioner work together to support rare disease product development.

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Before we turn these experts why pin SNUT.

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we will touch on three topic area yeah.

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First we will it aFDA role in supporting medical product development.

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Second we will recognize the prowl found.

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Impact of covid19.

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And third we will discuss the importance of working together during this challenging time.

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And background aFDA mission is to promote and protect the public health the helping stay effective treatment reach market and timely way.

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And monitoring treatment for safe continued safety as they are in use.

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FDA regulatory agency, that organized by-product areas.

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So scope FDA regina authority, is very proud.

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And FDA responsibility are closely related to those of several other government agencies.

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And general, aFDA regulates food including infants form.

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And, drug ds, and including prescriptions.

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And non prescription or over the counter drugs.

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Buying buying including vaccine and blood products, medical devise, and from things like tongue depressor to complex technology.

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Electronic product like give off radiation.

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And cot ME including colored atives.

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And, including pet foods and drugs and device and tobacco product including cigarettes and motorcycle SLEZ tobacco.

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FDA has many responsibilities.

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One FDA responsibility that central to our discussion today is to ensure drugs and vaccines and other products and medical devices were intended for human use are safe and effective.

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And in addition FDA advances public health by helping to speed medical product innovations.

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During this current public hell emergency, we continue to maintain foe to cause on our goal of supporting the development of safe and effective therapies for patients and families.

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This includes important efforts to support development and availability accurate and reliable covid19 and supporting development of treatment and vaccines for covid19.

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In addition to ASSISTing clinical trials for covid19 treatments and vaccines, we are ASSISTing drug companies that are conducting clinical trials during the covid19.

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Pandemic.

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Specifically with were helping TO ensure that any operational challenges ruling from covid19 are addressed so that clinical trials it can continue.

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Another very important areas I statement thousand ensure continued access to necessary medical products, including drugs, products and medical devices.

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Rare disease community including people are rare disease and their families have been pro founded impacted by covid19 he are cents by another of the people are rare diseases, have been impacted to some degree by covid19.

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And at cost to their immediate and long-term health and wellbeing.

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Covid19 has been associated with interruption in care.

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Cancellation of medical appoint disruption in clinical trials as well as job loss.

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Related to in.

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And also loss of health insurance.

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People with conditions, are rest rock of more severe illness from covid19.

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We appreciate this opportunity to hear directly from patients and care givers regarding impact of covid19.

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As we continue to responds covid19, our efforts rested to sponsors development of drugs and products to treat rare diseases, remains priority for the FDA.

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Covid19 pandemic may impact clinical trials of product.

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Potential shal challenges including travel limitation.

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We have been working to mitigate these potential impacts.

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Specifically we are working coastcly medical product companies who are conducting and planning clinical trials in addition we he have issued guidance to provide greater clarity to medical product companies.

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And about how to proceed with these trials.

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And deal with necessary deviation and trial conduct.

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Created by the pandemic.

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Developing treatment for rare disease can present unique challenge such small number of people affected.

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And different manifestations of given disease.

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We are committed to working together to overcome these challenges.

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While they are there challenge also opportunities.

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And rare disease product very much.

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With recent advances, there are new opportunities for development have therapies for rare disease.

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While this pandemic presents challenge, we are working together on strategy and solutions, to address these challenges.

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Further we are looking to learn from and apply more broadly, soon.

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Approaches we have taken during the pandemic which may prevents rare disease community.

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And we look forward to continuing advancing of treatment for rare disease.

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Thank you so much.

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>> Thank you.

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Very much.

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For that welcome.

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And helping our audience get to know FDA particularly in this current climate of of the pandemic.

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It really sets foundation and tone for rest of this listening session.

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So now we are going to get started with first rare disease family story on how impact of pandemic affected their lives.

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I would like to introduce Christie.

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Can you please unmute your line and come on to video.

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>> Yes.

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Thank you.

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I would like turn it over to you to tell us what happened to your family during the pandemic.

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>> Thank you very much.

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I may national Chris too and I have three children rare ge net being conditions called primary type one.

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And my 17 year old daughter molly started getting kidney stones when she was three years old and she didn't get diagnosis until she was six she had passed many kid know stones and needed surgery to remove larger stones throughout her livner currently kidney failure I my nine year old son ma new went end stage renal failure when he was five month old.

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After two years of dialysis six days a week and dialysis seven days a he can we, he received a liver and kidney transplants about when he was two ask half years old.

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He had several challenges and difficult recovery.

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But he seven years post transplant.

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He still faces transplants related health issues.

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And has on several recent hospital stays to treat those complication.

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He on do SDENS of medications to prevent rejection.

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And to deal with health issues related to his disease and the transplant.

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He has g tube assist in some medication and help get his threelier of fluid a day goal.

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We were facing several challenges before this pandemic hit.

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But once Covid restrictions hit nationwide it really made more things difficult and significantly altered our lives.

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Molly had been declining in health but watt participate clinical trial across country which required us to travel by plane every couple of weeks.

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Medication being studied had data that was very promising for this condition.

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She was unable to continue with the trial when flights were cancelled and prevented us from travelling to the study site.

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Her health be continued decline to where she had no other option but to begin dialysis six days a week and to be placed on transplants waiting limit.

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Making sure that our children are being cared for very challenging.

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Matthew needs to have his medication in fluids given throughout the day on strict schedule so that medication does not counteract with each other.

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His immune system significantly compromised and we have to be careful with who is around him.

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He currently suffering from side affects from his moment recents procedure, and he not responding well to that treatment.

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We are not sure if he will need to be readmitted to hospital.

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Or if we will be able to continue his treatment at home.

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In person appointment were changed TO video for matthew.

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Wanted to limit his exposure to hospital settings so labs were CLUMENTerred.

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Too.

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And looked good.

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But what could not be seen on the video appoint was his blood pressure was dangerously high.

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When we finally got him to inperson appointment they realized I had blood pressure issues and he was kept in a hospital for over a week, until it could be brought under control.

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We could not be discharged from the hospital.

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Until medication prescriptions could be filled.

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However the pharmacy the hospital used, had to change their hours or close due to Covid some of the medication considered special order and not kept at other pharmacies.

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We could not find the medication within 150 miles of us.

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Whether I asked the hospital, that prescribed the medication why they did not have it available a?

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I was told there restrictions due to covid19.

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We had stay in hospital extra days until we were able to fined medication at out of network facility.

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Which we had to payout of pocket for.

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When our son had to come back to the hospital for another treatment and stay, we had to bring that hard to find medications to the hospital.

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Because they still did not have it in stock.

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Other medications we needed were limited to how much we can get filled each time.

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Covid19 has completely changed our lives.

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My teenage daughter had hope with her out of state clinical because infections risk from air travel she had leave the trial.

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And is now in kidney failure on dialysis and waiting transplants for liver and kidney.

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My son, with my son having frequent admissions in hospital, and my daughter at die will a list appoints my husband had to leave his job to be able to help me care for our children.

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Which has our financial situation more strained.

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When my son has to go to the hospital they have drug shoresages and supply challenge we never know in we are bring our own medication with already difficult to find, and some times more expensive because, it may be only found at out of network facilities IN.

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When he goes to the hospital they won't discharge him until he has those medications.

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So we have to spend more time in hospital went increase his exposure risk.

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He very compromised.

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I have always tried to be prepared with medication and supplies to be care for my children with rare disease.

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Since covid19, it has been difficult to find quality supplies that are not overpriced.

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I never thought that I would be the mom to stop Kyle's medication this situation feels dire and I worry about having enough since I can't trust the hospitals or pharmacies to have them.

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As rare disease mom and care give I am used to unknown and unPREDICTable.

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However, 202 threw us for a loop.

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I don't think any of us were prepared for this.

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Thank you for letting had me share my family's story.

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>> Thank you so much for sharing your family stories for these beautiful photos.

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I can only imagine what it is like to have work so hard for your children's care.

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And lively hood during such challenging time.

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And don't seem like there is an end in sight your story an important experience to share because we don't know how long this pandemic will last.

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And unfortunately, we have heard similar stories from rare disease community around the country.

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We are so grateful that you share your experience so honestly about the reality that rare kids face and adult face with drug shortage during the pandemic.

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So I would like to welcome doctors, is from the center for drug evaluation and research.

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We are so glad that we could bring them here to tell us their thoughts on this subject.

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As well as answer very specific questions we did collect 100 questions from community.

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And with the family story in mind, first question we have for FDA goes to Dr. Done know hue and it is this.

00:30:46.000 --> 00:30:51.000
What are recommended plans for facilitating access for medication during this global pandemic.

00:30:51.000 --> 00:30:54.000
>> Thank you for your story and thank you for NORD for having us today.

00:30:54.000 --> 00:30:57.000
I am glad we continue to talk about it.

00:30:57.000 --> 00:31:27.000
Given Covid KOIF I pan drugs are in shortage.

00:31:31.000 --> 00:31:41.000
We are seeing disruptions across the supply chains in FDA working proactive three with manufacturers and especially those for rare disease therapies evaluate supply chain include active pharmaceutical ingredients, and other components that maybe impacted, with the supply chain disruption from outbreak for example, we reached you the ought manufacturers for immune and, in order assess avail ability and make sure there continued supply of critical product for patients with primary disorders.

00:31:41.000 --> 00:31:43.000
And so we work closely with manufacturers like this to try ask prevents or least reduce impact of shortages.

00:31:43.000 --> 00:32:10.000
>> Thank you for that response.

00:32:10.000 --> 00:32:13.000
That's incredibly helpful to no FDA works assess issues around drug shortage any to that, synergy and this one will go to -- that we can medication we take in short supply or back ordered is there any way to prepare for that ahead of time so what can we do if medication we take is in short supply or back ordered?

00:32:13.000 --> 00:32:14.000
And, I think a lot of patients are thinking about this.

00:32:14.000 --> 00:32:17.000
>> Thank you so much.

00:32:17.000 --> 00:32:25.000
And thank you NORD for this opportunities.

00:32:25.000 --> 00:32:37.000
And also I want to think Christie for this story, her story and along with molly and matthew.

00:32:37.000 --> 00:32:48.000
And to able answer of course you know, our health care providers at the front line of all of this if medicationer shortage or tan it is it -- really important.

00:32:48.000 --> 00:32:55.000
It is not uncommon for example to providers to identify alterative to provide same medication for example the -- we hope find alternatives altogether.

00:32:55.000 --> 00:32:57.000
We also ask to please continue to check the drug shortage web page.

00:32:57.000 --> 00:33:08.000
And page for have have.

00:33:08.000 --> 00:33:24.000
This will help get latest information on currents shortage as well as anticipated due raise for such shortages and along with multiple other information.

00:33:24.000 --> 00:33:43.000
FDA keeps those web sites updated regularly most recents information on them and their continue to make sure those web seats are updated if you have any questions regarding drug shortage and general please contact the staff.

00:33:43.000 --> 00:33:44.000
I believe we have slide with those e-mail addresses that this center for drugs e-mail address is drug shortage one word at FDA hhs.gov for biologicalic project you with e-mail us at --.

00:33:44.000 --> 00:33:49.000
Thank you.

00:33:49.000 --> 00:33:51.000
>> That he very useful information let's skip to next slide.

00:33:51.000 --> 00:34:05.000
and show what doctor mentioned.

00:34:05.000 --> 00:34:05.000
These resources thank you so much for being here and for answering that question and let us how key follow up, talking to our doctor about the therapy reon, and what our needs are in the future?

00:34:05.000 --> 00:34:09.000
>> And today.

00:34:09.000 --> 00:34:12.000
>> We will keep this slide up for couple of seconds.

00:34:12.000 --> 00:34:16.000
If you want to take screen shot.

00:34:16.000 --> 00:34:20.000
Also note there recording will be made available.

00:34:20.000 --> 00:34:22.000
On line, and we will will be sharing that with you.

00:34:22.000 --> 00:34:30.000
As soon as it is ready.

00:34:30.000 --> 00:34:40.000
>> So you can always access this slide and the whole talk later.

00:34:40.000 --> 00:34:43.000
So next we will be hearing from another community voice, Laura bono will share her family experience with PPE shortages during pandemic.

00:34:43.000 --> 00:34:45.000
Can you please unmute your line and come on to camera?

00:34:45.000 --> 00:34:46.000
>> Thank you.

00:34:46.000 --> 00:34:49.000
Good afternoon.

00:34:49.000 --> 00:35:06.000
I live in a Detroit suburb in Michigan.

00:35:06.000 --> 00:35:09.000
And my two daughters 25 year old molly and 23 year old Emily live with CYSTIC fibrosis which rare disease, that produces thick sticky mucous causes chronic fatal lung infections and interferes are with digestion.

00:35:09.000 --> 00:35:19.000
It is affects multiple organ says it in the body.

00:35:19.000 --> 00:35:25.000
Since my daughters diagnosis, all of those years ago we have continuously used PPE equipment we DN think twice about it until the pandemic hits.

00:35:25.000 --> 00:35:29.000
When pandemic did hit the United States, I knew there was just a matter of time before it landed in Michigan.

00:35:29.000 --> 00:35:32.000
And I was concerned about discrimination against people with cf.

00:35:32.000 --> 00:35:36.000
And for everybody with underlying conditions.

00:35:36.000 --> 00:35:40.000
We have always needed PPE but now there would be much greater demand for it.

00:35:40.000 --> 00:35:45.000
But for my daughters it is always been life and death.

00:35:45.000 --> 00:35:50.000
As mom of two girls with cf I always had PPE in the home.

00:35:50.000 --> 00:35:56.000
But I wasn't stocked up on those supplies.

00:35:56.000 --> 00:36:01.000
Unfortunately, we didn't have any n-79D five mass acts we only had about two disposable masks.

00:36:01.000 --> 00:36:07.000
Lot of other cf mom were in same situation.

00:36:07.000 --> 00:36:10.000
And mom -- we thought we were TAESHL mom destocked up on n-95 masks.

00:36:10.000 --> 00:36:13.000
Cf doctors were telling us there were none available.

00:36:13.000 --> 00:36:17.000
And we couldn't get any during the pandemic.

00:36:17.000 --> 00:36:25.000
We were running out of variety of PPE, including masks.

00:36:25.000 --> 00:36:28.000
This is critical for my daughter because she lives with her boyfriend who is following the Covid rules but still visits his family.

00:36:28.000 --> 00:36:38.000
He takes appropriate precautions masking and hand washing.

00:36:38.000 --> 00:36:41.000
But he has to return to work and protocol are will be taken but he needs the daily new mask to keep my daughter safe.

00:36:41.000 --> 00:36:49.000
My 25 year old daughter was getting anxious and stressed.

00:36:49.000 --> 00:36:53.000
She lives like I said in her own apartment ten minutes from us, and I could not find any PPE on line for her.

00:36:53.000 --> 00:36:57.000
There was jump none in the stores and we were not going into the stores anyway.

00:36:57.000 --> 00:36:59.000
People in my cf SHAT groups couldn't find any either.

00:36:59.000 --> 00:36:59.000
I was mad.

00:36:59.000 --> 00:37:01.000
Frustrated.

00:37:01.000 --> 00:37:08.000
And in a bit of panic.

00:37:08.000 --> 00:37:17.000
The girl cf doctors told us that during the pandemic, one parents should always be the parents connected to the outside world.

00:37:17.000 --> 00:37:21.000
So if we didn't have to go into the store, if we did have to go into the store it should be the same person and that person should not go near our daughter.

00:37:21.000 --> 00:37:26.000
So my husband Joe was forced to go into cost co by necessity.

00:37:26.000 --> 00:37:28.000
I have foundation that helps people with cx.

00:37:28.000 --> 00:37:39.000
Living with cf.

00:37:39.000 --> 00:37:48.000
And I called my county commissioner and said Dave, how can all of these metro Detroit businesses have so much PPE and I can't find masks or anything?

00:37:48.000 --> 00:37:49.000
And he said that the county bought everything, that their suppliers had in stock as soon as covid19 hit Michigan I had warehouse full of supplies.

00:37:49.000 --> 00:37:51.000
What did I need.

00:37:51.000 --> 00:37:56.000
I told him that I needed a lot.

00:37:56.000 --> 00:37:58.000
He brought over a truck load of variety of supplies and masks.

00:37:58.000 --> 00:37:59.000
I post all to my cf people.

00:37:59.000 --> 00:38:01.000
That I had it.

00:38:01.000 --> 00:38:03.000
And they could come pick it up.

00:38:03.000 --> 00:38:06.000
Or would drive to them.

00:38:06.000 --> 00:38:12.000
I drove some two hours to meet one family's needs.

00:38:12.000 --> 00:38:15.000
And so many people were coming to pick it up from my house and I had a to make actual all gone in a week.

00:38:15.000 --> 00:38:18.000
People from hours away were requesting it.

00:38:18.000 --> 00:38:32.000
I documented everything with photos and video.

00:38:32.000 --> 00:38:38.000
And I had e-mails and calls from people in other states who had cf that were requesting that ship them PPE, but sadly I couldn't do it for fear of going into if the post office because ever covid19.

00:38:38.000 --> 00:38:48.000
There are many ADVANCE to beinged a advocate having strong connection to the cf community.

00:38:48.000 --> 00:39:05.000
One all parents who are isolate dodd not have the training time and energy, resources or o knowledge on how to fight for their children's PPE needs?

00:39:05.000 --> 00:39:10.000
And even with my privilege and position to advocate for my girls I still cannot get much-needed and masks in my girls do need to pro TKT themselves and not rely on others to possibly protect them by wearing mask and wearing correctly.

00:39:10.000 --> 00:39:15.000
Each girl has one n-95 they are saving it in case things get worse than they are now.

00:39:15.000 --> 00:39:17.000
I am hon in other words to share my family's story and my concerns for the larger cf community.

00:39:17.000 --> 00:39:18.000
Thank you for hearing me.

00:39:18.000 --> 00:39:21.000
>> Thank you.

00:39:21.000 --> 00:39:22.000
>> Laura thank you so much for sharing your family's experience.

00:39:22.000 --> 00:39:30.000
That's unbelievable.

00:39:30.000 --> 00:39:38.000
It is just incredibly to think about how far you had to go and how much need there was for all of these families.

00:39:38.000 --> 00:39:47.000
And even with your connection and your leadership with the foundation even then it was still really difficult to get this life preserving PPE.

00:39:47.000 --> 00:39:57.000
So with your story in mind I would like to welcome the doctor cdrh.

00:39:57.000 --> 00:40:09.000
We are so glad that she could join us today she also was part of the group that reviewed 100 west and looked specifically at PPE questions.

00:40:09.000 --> 00:40:11.000
I like to turn it over to her for response from FDA about PPE shortage and even just some basic for those are unfamiliar about complex aspect of using PPE.

00:40:11.000 --> 00:40:14.000
Dr. Ross thank you so much for joining us.

00:40:14.000 --> 00:40:14.000
>> Thank you.

00:40:14.000 --> 00:40:20.000
Good afternoon.

00:40:20.000 --> 00:40:27.000
First I would like to thank Laura for sharing perspective with us.

00:40:27.000 --> 00:40:42.000
We certainly recognize concern expressed by patient living with chronic pulmonary conditions.

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Because there are variety of ways patients can protect them and each patient unique, we would recommend that they discuss their concerns with their health care provider and work together to determine reasonable option to protect themselves.

00:40:45.000 --> 00:40:49.000
Including, appropriate use of the PPE.

00:40:49.000 --> 00:40:58.000
For them and or their family members.

00:40:58.000 --> 00:41:13.000
Some options, that can be worn to help slow the spread including facility coverings.

00:41:13.000 --> 00:41:23.000
First CDC items for consideration as engage in discussions with their health care provider about face coverings, include, fits and comfort.

00:41:23.000 --> 00:41:30.000
Fit is important because if face covering does not fit well, it does not provide maximum protection to the wearer.

00:41:30.000 --> 00:41:42.000
Comfort is important because you may have to wear the it for long period of time.

00:41:42.000 --> 00:41:50.000
You want to be able to breathe easily, and you don't want to have to touch the it or to adjust it.

00:41:50.000 --> 00:41:57.000
FDA continues on to aa number ever efforts, to help assure that health care providers have PPE available.

00:41:57.000 --> 00:41:59.000
Emergency use authorization authority, allows FDA to help strengthen the nation's protection.

00:41:59.000 --> 00:42:01.000
Against public health threats.

00:42:01.000 --> 00:42:07.000
By facilitating available.

00:42:07.000 --> 00:42:10.000
And use of medical counter measures during emergencies.

00:42:10.000 --> 00:42:14.000
To address concerns about lack ever availability.

00:42:14.000 --> 00:42:17.000
P PPE for healthcare personal.

00:42:17.000 --> 00:42:33.000
U a from many types of PPE.

00:42:33.000 --> 00:42:42.000
And including n-95 and others, surge and face she wills in addition, FDA has issued guidance documents, providing regulatory flexibility, and encourage availability after downs.

00:42:42.000 --> 00:42:58.000
And medical gloves, and, in surgical mask during covid19 public health emergency.

00:42:58.000 --> 00:43:14.000
FDA has also issued, u a for face mask non surgical for use by the general public and has discretion policy to help facilitate additional availability, of these products.

00:43:14.000 --> 00:43:20.000
If you are having challenge, accessing face coverings, you may want to discuss what resources for PPE that may be available, with your health care, provider.

00:43:20.000 --> 00:43:21.000
We also have a web site that that is lot of different resources, available.

00:43:21.000 --> 00:43:26.000
For the public.

00:43:26.000 --> 00:43:32.000
And I know they have the resource page a little bather on in the presentation.

00:43:32.000 --> 00:43:35.000
Again, very much appreciate the opportunity to listen to stories and to participate in this events.

00:43:35.000 --> 00:43:38.000
>> Thank you so much.

00:43:38.000 --> 00:43:47.000
For sharing important information about accessing PPE.

00:43:47.000 --> 00:43:52.000
And the emergency use authorization you mentioned lot of information including link and we will show on next slide some of resources that you mentioned.

00:43:52.000 --> 00:43:58.000
In ever he a VOVRLing environment around accessing personal protective equipment.

00:43:58.000 --> 00:43:59.000
So these resources here we are going hold on screen for a minute or so.

00:43:59.000 --> 00:44:01.000

And take a look.

00:44:01.000 --> 00:44:02.000
And take a screen shot.

00:44:02.000 --> 00:44:03.000
Take your phone out.

00:44:03.000 --> 00:44:08.000
Take a picture.

00:44:08.000 --> 00:44:11.000
And know that this recording also be made available and you can just hit pause.

00:44:11.000 --> 00:44:15.000
So yes these links a little bit long.

00:44:15.000 --> 00:44:16.000
Don't spend too much time but, we will keep this slide up.

00:44:16.000 --> 00:44:21.000
For you replay recording.

00:44:21.000 --> 00:44:26.000
So now, we will hear from our final community story.

00:44:26.000 --> 00:44:28.000
About her son access to clinical trials during the pandemic.

00:44:28.000 --> 00:44:30.000
Thank you so much for Johning us.

00:44:30.000 --> 00:44:30.000
Can you tell us what happened.

00:44:30.000 --> 00:44:31.000
>> Yes.

00:44:31.000 --> 00:44:36.000
in hi.

Good afternoon and thank you to everyone for your listening to how the pandemic has impacted our participation and experience in a clinical trial.
My name is Renie Moss, and my 15-year-old son, Philip, and 12-year-old daughter, Helen, have neurofibromatosis type 1, an incurable genetic disorder that
causes tumors to grow along nerves in the body as well as carries an increased risk of leukemia and other types of cancer. Philip is acutely affected by

a large inoperable plexiform tumor in his neck. When Philip was ten, his tumor had grown to surround his carotid arteries and was threatening his airway.
The growth of the tumor continued relentlessly. We watched and waited for a promising clinical trial to come along that might save his life.
That opportunity presented itself in the form of a clinical drug trial at the NIH Pediatric Oncology branch. He enrolled in September 2015 and began
to take an oral MEK inhibitor targeted drug therapy. There are serious side effects that can be associated with this drug that are carefully monitored
by his clinical trial medical team in Bethesda and for the past five years, we have traveled from our home in Birmingham, Alabama to Bethesda at a minimum,
twice per year. At each study visit, Philip completes safety monitoring exams that include EKG, echocardiogram to monitor his heart ejection fraction due
to the known elevated CPK levels that can sometimes cause muscle deterioration and a thorough eye exam due to a rare but serious side effect that can cause
a retinal blister. He also has blood work, pulmonary function tests, sometimes sleep studies and dexascans, but most important is the contrast MRI.
Over the course of the last five years, Philip's tumor has shrunk just over 60%, resulting in significant clinical improvements and improved quality of life.
We are the epitome of a grateful family for what this clinical trial has meant to our son. In late April 2020, the drug received the coveted FDA approval and
children across the country now have access to the same life changing drug therapy our son continues to take. For the first time, there is an FDA approved
treatment for this type of tumor associated with neurofibromatosis type 1.
When COVID-19 arrived to the United States, we monitored the situation closely, knowing that Philip had a study visit scheduled for the last week of April
that would require us to fly to Bethesda.. At each of these twice a year visits, following all the required tests, Philip has been approved to continue to
receive six more months of the oral drug to continue treatment. Because of COVID-19, Philip was not able to complete the in-person tests. Instead, we had
a telemedicine visit with his clinical trial team, and completed pain and quality of life surveys by email to return to the study team. We were not able to
complete the MRI that is now delayed until our tentative next trip to the NIH upcoming in October. We were told to contact the study team if we suspected
any new concern and to seek local medical care immediately if necessary. This will be the longest Philip has gone between MRIs. I shared that Philip is now
15 and it is important to understand that with neurofibromatosis, puberty is yet another new time of fear for us as plexiform tumors carry a risk of turning
malignant. This is why the MRI is a critical part of Philip's ongoing medical care. Thus, I would say the greatest impact COVID-19 has had on us is the gap
in life saving medical surveillance by the same medical team that has imaged Philip's tumor for over five years now. Thankfully, Philip continues to receive
drug so we are grateful that treatment can continue, but we are on high alert for changes in his vision, heart, muscle strength, any sign of new pain, and other
issues that can signal either a severe side effect from the drug therapy or our worst fear, leukemia or a tumor malignancy that carries with it a very low
survival rate.
I will also share that our daughter, Helen, has developed several small tumors on her scalp and she was due to enroll in the NF Natural History Study in April

but is now scheduled to coincide with her brother's October evaluation. Should COVID-19 cause a cancellation in our October trip, we will work to complete an
MRI locally in Birmingham, just in case there are unknown acute concerns.
This clinical trial has given us our son's life, back, not only life but a significantly improved quality of life. What this means to us is impossible to put
into words. But COVID-19 has brought back fears that, for a while, we were able to push aside due to the excellent medical evaluation we receive from our
clinical trial team at the NCI Pediatric Oncology Branch. Should COVID-19 cause a cancellation in our upcoming trip, we will work with our local medical team
to, at the very least, complete an MRI to rule out malignancy and to hopefully show a continued positive response to the drug therapy. We are saving our
COVID-19 stimulus check and cutting any expenses we can for any additional medical costs to ensure our son's continued improved health, as we can never turn
our back on this cruel genetic disorder and the ways it affects our son.
Thank you for hearing our story.

00:49:43.000 --> 00:49:54.000
>> Thank you so much for sharing your experience.

00:49:54.000 --> 00:50:14.000
I am sure that lot of people watching were not nod feeling very similarly, especially at the end, it is really wonderful to hear how clinic such game changing for.

00:50:14.000 --> 00:50:17.000
And, literally saving his life and his quality of life that's really great news we could not be happier for your family, but you really drover message is lot unknown around Covid how long it will continue make world and what will mean for your son'sable to being assess his trial if pandemic interrupts, this trial any way.

00:50:17.000 --> 00:50:18.000
And future does seem uncertain.

00:50:18.000 --> 00:50:26.000
Unset amming.

00:50:26.000 --> 00:50:29.000
And while there so much hope for research and trials for rare disease you are family story really demonstrates that we have questions about the future.

00:50:29.000 --> 00:50:33.000
The rare disease community wants to know.

00:50:33.000 --> 00:50:37.000
And so we will really grateful that you shared was heard by FDA.

00:50:37.000 --> 00:50:39.000
And it reflects our greatest hopes trials are working.

00:50:39.000 --> 00:50:46.000
But also some of our fears for the future.

00:50:46.000 --> 00:50:51.000
So I would like to bring back doctor Carol and doctor done know hue for some feedback on his family's story.

00:50:51.000 --> 00:50:55.000
And, about clinical trials during the pandemic.

00:50:55.000 --> 00:51:03.000
So, as you can imagine, whether comes to clinical trials.

00:51:03.000 --> 00:51:11.000
We received so many questions and the top question from rare disease community for this listening session was this.

00:51:11.000 --> 00:51:18.000
How can ultra rare disease still be researched and possible treatment discovered and studied inspite of covid19?

00:51:18.000 --> 00:51:20.000
So I am going throw that to the doctor what is FDA's response to this?

00:51:20.000 --> 00:51:35.000
>> Thank you.

00:51:35.000 --> 00:51:38.000
Again I thank you for telling us situation with Phillip and helen hearing in your voice like the importance of clinical trials and how likibilities greated and into every aspect of your life and to reminds why we do what we do across the board.

00:51:38.000 --> 00:51:46.000
So thank you so much for sharing that story with us.

00:51:46.000 --> 00:51:54.000
Obviously recognized that covid19 is impacting the conduct of it clinical trials across the board.

00:51:54.000 --> 00:52:05.000
Medical product and that impact may be felt acutely by patient with their disease for which there is often no available therapies.

00:52:05.000 --> 00:52:10.000
And FDA working to advance treatment of rare disease and help ensure continuity of care with people rare disease which altogether remain apply other tie even through this pandemic.

00:52:10.000 --> 00:52:12.000

Working both with sponsors and patients.

00:52:12.000 --> 00:52:15.000
We remain focussed on this.

00:52:15.000 --> 00:52:29.000
For serious where he Tree --.

00:52:29.000 --> 00:52:35.000
To help sponsor conducting it ongoing clinical trials during covid19 public health emergency we published guidance called conduct ever clinical trials during covid19.

00:52:35.000 --> 00:52:43.000
It was published in March since then it has been updated multiple times.

00:52:43.000 --> 00:52:48.000
Has POE tension shah ways met gate impact I have covid19 clinical trial what safety.

00:52:48.000 --> 00:53:01.000
And data qualify and to make sure trial useful.

00:53:01.000 --> 00:53:05.000
And, guidance can also be found on is, often updated a mentioned with new information based on questions we receive from our stake holders I am going to discuss a little bit more details about this guidance in coming up question.

00:53:05.000 --> 00:53:08.000
But thank you again so much for this supporting.

00:53:08.000 --> 00:53:12.000
>> Thank you for that response.

00:53:12.000 --> 00:53:13.000
Our next question is for doctor done know hue.

00:53:13.000 --> 00:53:25.000
This is good follow up.

00:53:25.000 --> 00:53:26.000
what are some ever new practices acome DAY being put into place to allow patients with rare disorders that participate in clinical trials during this pandemic?

00:53:26.000 --> 00:53:43.000
>> Sure.

00:53:43.000 --> 00:53:47.000

So, you know we encourage, appropriate approaches and tools that could enhance at that KRIL takes, or enhance facilitate participation in inI cancal trials so for example whether appropriate decan help reduce, burden on trial pair it is pans.

00:53:47.000 --> 00:54:00.000
Travel burdens also expanding the geographic reach recruit men.

00:54:00.000 --> 00:54:10.000
I know for lot of my trials we have patients some times who relocate to different country in order to participate in clinical trial it is massive burden so we decentralized have potential to address that.

00:54:10.000 --> 00:54:12.000
Then use digital tools can allow remote data capture in some Kirks we are hoping that adapt likes that could make a difference for patients like helen and Phillip.

00:54:12.000 --> 00:54:13.000
And all of the ones on the call.

00:54:13.000 --> 00:54:18.000
>> Thank you for that.

00:54:18.000 --> 00:54:24.000
And at this time no one in United States allowed to go anywhere.

00:54:24.000 --> 00:54:50.000
So, it is seems like having these decentralized trials is really silver line.

00:54:50.000 --> 00:54:53.000
And there are some real opportunities on to make trials more accessible so I guess the follow up to that would be, this question, how flexible will they be with remote capture in clinical trials and will they value data at much in person versus remote capture and that question goes to SGLTS thank you again.

00:54:53.000 --> 00:54:57.000
Covid19 can impact on going trials.

00:54:57.000 --> 00:54:58.000
Because challenges such site closure travel limitation.

00:54:58.000 --> 00:55:01.000
Ateens and interruption to --

00:55:01.000 --> 00:55:09.000
lot of this was illustrated in story we just heard.

00:55:09.000 --> 00:55:12.000
And FDA already working with sponsors hospital and investigators to understands and address challenges that may disrum trials.

00:55:12.000 --> 00:55:42.000
With patient safety of course being paramount.

00:56:03.000 --> 00:56:11.000
And as I mentioned before guidance that so this is of course when public hell measure control covid19 limit travel laws as we are all experiencing right now we encourage sponsors considering, incorp remote performance outcome assessments.

00:56:11.000 --> 00:56:16.000
Review based clinician reported and health trial to discuss, with relevant review division at the agency.

00:56:16.000 --> 00:56:22.000
Whether they are appropriate for type of data they plan to collect.

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We think really an important step to understands which areas lends themselves to this type of it data capture.

00:56:29.000 --> 00:56:39.000
We understands whether it is safe for example feasibler to participants in assessments at the subject location itself.

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And, we also in guidance we have we provided mail books to get questions and we use all of those questions to really advance how we develop this guidance and modify.

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The mailbox for that is clinical trials conduct.

00:56:46.000 --> 00:56:56.000
-- covid19.

00:56:56.000 --> 00:56:58.000
And FDA hhs.gov I encourage community look at this guidance plumb tip will ways that we hope they can make clinical trial more efficient and more feasible.

00:56:58.000 --> 00:57:06.000
Thank you again for the question.

00:57:06.000 --> 00:57:09.000
>> Thank you so much in guidance sounds like game changer thank you for that e-mail address that's on the screen.

00:57:09.000 --> 00:57:14.000
So, our next question goes to Dr. Done know hue.

00:57:14.000 --> 00:57:18.000
What is FDA plan on dealing with data interruption for ongoing clinical trials?

00:57:18.000 --> 00:57:19.000
Say, if assessments could not be check asked?

00:57:19.000 --> 00:57:29.000
>> Yes.

00:57:29.000 --> 00:57:35.000
So we recognize that challenge for rare disease, clinical trials and frankly clean cakele given impact of the pandemic on trial conduct.

00:57:35.000 --> 00:57:50.000
And he we are committed to working with communities who find ways to XHAK sure important trials can continue.

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And we have a lot of experience working with pharmaceutical companies on trials and rare disease that use you know small patient populations, and you know study recruit minute challenges are nothing new for us.

00:57:58.000 --> 00:58:06.000
And so, we will continue to work with sponsor that may have experienced additional difficulty in recruiting due to covid19.

00:58:06.000 --> 00:58:09.000
And so you know impact of measures institute to control, covid19, can affect clinical trials in lot of ways including design.

00:58:09.000 --> 00:58:24.000
What regions study can be conducted.

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And, in the guidance that we have been talkingB-we outlined general considerations, to assist sponsors in in ensuring safety trial participants, and maintaining compliance with the good clinical practice and also minimizing risk to trial and integrity.

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And so, bottom line is that safety of patients, participating in trials during this pandemic is really our top priority, and so modification that need to be made to address that we have to accommodated them.

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And we really encourage stake holders to talk with us timely fashion so we are asking sponsored of pharmaceutical companies, don't just change Mike sure.

00:58:51.000 --> 00:58:59.000
That, changes are going to keep marshes safe.

00:58:59.000 --> 00:59:03.000

And really you know preserve trial integrity so that patients, and doctors get answers we need from these trials.

00:59:03.000 --> 00:59:08.000
>> Thank you so much.

00:59:08.000 --> 00:59:12.000
So we have heard from our patients stories and we leader from FDA responding to these three areas.

00:59:12.000 --> 00:59:17.000
Of the drug shortage, personal protective equipment.

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Shortage, and clinical trials and impact we know that you have so many questions.

00:59:18.000 --> 00:59:24.000
For FDA.

00:59:24.000 --> 00:59:27.000
And he due time con stratedgy this only 60 we spend whole day talking about pandemic impact.

00:59:27.000 --> 00:59:31.000
And how FDA responding.

We didn't have time to answer the near-100 questions submitted by our audience in advance, but there is a place to go for more information.
Visit FDA.gov/PatientsAskFDA for a special portal to ask a question to FDA about diseases, health conditions, drugs, devices, vaccines/blood and biologics.
This form is for patients, caregivers, advocates, and healthcare providers. This form is not for industry stakeholders. This form is also not a place to
report adverse events (like harmful side effects) about your medical product or drug. You should visit the MedWatch reporting form, which is linked on this page.

Do you have a question for NORD? How can NORD Support you? NORD is here for you. We offer financial patient assistance for co-pays and travel to
medical appointments. We have developed a series of educational webinars bringing experts to help you navigate this pandemic, including what the future
looks like in a post-pandemic work for access to treatment and research. You can access the recordings online by visiting rarediseases.org/covid-19.
We offer ways to get connected locally, as well as advocacy opportunities to learn about your state and local issues for staying safe during the pandemic.

I'd like to end with some concluding remarks.
With my sister struggling with her rare disease, pulmonary hypertension, many of our family and friends unaware of all of this incredible work that
FDA is orchestrating across various researchers and stakeholders. Personally, it is a relief to hear directly from FDA that their staff is working day and night,

proactively on helping to facilitate these important trials. My family is but one of 30 million Americans living with rare diseases – and for many, this
may be your first time of hearing about what you do and how you are working "behind-the-scenes" to help bridge the challenges we've seen from the pandemic.
But I can't say "behind-the-scenes" because everything FDA is doing is broadcasted online and their team are so proactively meeting with patient communities
through these Listening Sessions. For example, dozens of FDA staff regulators are watching this Listening Session live and many more will access the recording later.

These family stories are unforgettable and really capture what is at stake here: Some rare patients may find themselves in a life and death struggle if COVID19
stops them from getting access to our rare disease therapies and personal protective equipment. Some rare disease patients are alive only because a clinical
trial allowed access to life-saving investigational therapy. The future is unsure, but from what we've heard throughout this session from our FDA speakers is
that the focus and commitment of FDA is stronger than ever: facilitating development and approval of products for serious conditions where there is tremendous
unmet medical need.

I'd like to take a moment to thank all of our speakers, from the brave accounts by our community speakers: Kristi Ouimet, Laura Bonnell, and Renie Moss.
The COVID-19 pandemic has presented significant challenges for the rare disease community and your stories really demonstrate the hard work to overcome them and
our real concerns about the future. Also, NORD would like to offer a special thank you to all of you who shared your stories, asked very poignant questions and
those who listened in with us on this important topic during this unprecedented time.

On behalf of NORD, I also want to thank our FDA speakers, Drs. Donohue, Dr. ElZarrad, and Dr. Ross, for their time and thoughtful responses to our questions.
A thank you to all of the FDA staff who took time to thoroughly review the dozens of questions submitted by the rare disease community, and to ascertain the
most prevalent and significant topic areas on which to focus.

Thank you to Dr. Maynard, the Centers for Drug and Evaluation Research, the Centers for Drug and Biological Research, the Patient Affairs Staff and everyone at
FDA who continue to create opportunities listen to – and support – the rare disease community in our struggles during this pandemic.

NORD stands ready to remain a constructive partner to the FDA now and long after the pandemic. We encourage anyone who needs support or wants to fight back to
join us online at www.RareDiseases.org. Thank you and good day.