February 7, 2023 National Policy & Advocacy Taskforce Meeting Transcript

Allison Herrity (Policy Analyst, NORD):

Hi, everyone and thank you again for joining us. My name is Allison Herrity and I'm a Policy Analyst here at NORD. I cover our telehealth, newborn screening, and medical nutrition policy work, as well as some of our step therapy and prescription drug out-of-pocket work as well.

In addition to that, I've been kind of heading up the organization of our new Policy and Advocacy Taskforce for NORD's policy team, so you may recognize me from your email inbox. So, again, thank you, everyone, for joining this meeting today! I'm really excited to get things started. Next slide please.

So just a little overview of the agenda and a few quick housekeeping things. This meeting is being recorded which it should have told you when you logged on, or when I began recording it. So if, again, if you're someone who doesn't like to have your camera on recorded meetings or anything along those lines, this is a heads up, we do also plan on distributing the recorded meeting to meeting registrants in the next few days.

So just a brief overview of our agenda today, we're gonna begin meeting with the meeting with a quick introduction to NORD and NORD's policy team.

Then we're gonna give you some information about the National and Regional Policy and Advocacy Taskforces, as well as introducing you to some of our wonderful volunteers who you'll be working with.

And then to delve a little bit into some of the policy stuff, we'll give an overview of NORD's 2023 advocacy priorities, as well as share some highlights from NORD's latest edition of our signature State Report Card project, which just went live today. We're really excited about it.

Then we'll get into how you as advocates and Taskforce members can use your voice to catalye meaningful policy change for the rare disease community. And of course we want to give you all some time to ask some questions at the end.

So just a quick little overview of NORD for anyone who might not be familiar or might need a refresher. NORD is the leading, independent, patient advocacy organization representing individuals and families impacted by rare diseases in the United States. NORD was founded about 40 years ago by patients, families, and patient organizations and advocates, who, like yourselves, sought to address the unique policy problems and challenges faced by the rare disease community.

It's Nord's mission to improve the health, and well, being of people with rare diseases by driving advances in care, research, and policy.

Across Nord we share a vision for the rare disease community in which there's national awareness and recognition of the challenges the rare community faces, a country where people with rare diseases can secure access to care that extend and improve their lives, a culture of innovation that supports the research necessary to develop diagnostic tools and treatments for rare disorders, and a regulatory environment that encourages timely approval of safe and effective therapies for rare disease patients

We align our work with our values listed here on slides, and those are compassion, integrity, innovation, inclusion, and collaboration.

I'm very excited to get to introduce you to the wonderful group of people that I get to work with every day, which is NORD's policy team.

I'm going to let everyone on our team who is able to join this call introduce ourselves. And so if we just want to going in order, I can hand it over to Heidi real quick.

Heidi Ross (Vice President of Policy & Regulatory Affairs, NORD):

Hi, everybody! We're so excited to have you here. I'm Heidi Ross. I'm the Vice President of Policy and Regulatory Affairs at NORD, and I am really honored to have the opportunity to be a part of this task force and to work with all of you guys to further the policy goals of the rare disease community.

I have a brother who had a rare cancer, and he is fortunately in remission, but he, as a result of his treatment, has developed a rare immune deficiency disorder, and so the near the cause of rare is really near and dear to my heart, and I'm excited to get a chance to work with all of you guys to support our community.

I will also briefly introduce Karin Hoelzer. She's not able to be on the call today, but she is the person on our team who works most directly with the NIA and FDA around rare disease research and drug development so she will be a resource available to you guys as well. And with that I'll turn it over to Corinne Alberts.

Corinne Alberts (Associate Director of Policy, NORD):

Hi everyone, I am Corinne Alberts, I am Associate Director of Policy with the NORD team, and, like Heidi, and like a lot of members of our team, my family has been really directly impacted by rare disease and the the issues that we work on are near and dear to me. I'm just so excited to be launching this Taskforce and to have the opportunity to be able to work with all of you on policy issue areas.

I mostly handle issues related to insurance coverage and affordability. So things, related to Medicaid and Medicare. Anything to do with insurance accessibility and access. So you may also be seeing me in your inbox in the months to come. I will now turn it over to Alyss Patel.

Alyss Patel (State Policy Manager, Western Region, NORD):

Thank you, Corinne. Hi, everyone! My name's Alyss, and I serve as our State Policy Manager for the Western Region. I work with my counterpart, Annissa Reed, who unfortunately, I don't think is able to join us yet because she's doing testimony for a Rare Disease Advisory Council.

So that is one of the big focuses of our state team is working to get Rare Disease Advisory Councils across the country. Right now we have them in 24 States, and we also work on other issues that impact rare disease patients on the state level. I'm so glad to be with you all today, and thanks for joining us! And I'll turn it over to Hayley.

Hayley Mason (Policy Analyst, NORD):

Hi everybody! My name's Hayley Mason, and I'm a policy analyst with NORD. I work with Karin on our FDA and NIH-related issues. I'm really looking forward to starting this Policy and Advocacy Taskforce. And I believe that's everyone on NORD's Policy team who is currently here!

Allison Herrity (Policy Analyst, NORD):

Yes, alright, wonderful. Thank you guys for that introduction. One of the goals of the task force is to make NORD's policy team more accessible to the rare disease community, and especially to the members of our Policy and Advocacy Taskforce. So please, don't hesitate to reach out to us. We have an email that will be listed on a later slide, but it's pretty easy to remember, it's policy@rarediseases.org.

Alright, so now for the reason we're all here – NORD's Policy & Advocacy Taskforces.

So the Taskforce Project will consist of two different levels, so to say, there will be a National Taskforce which focuses on Federal policy and advocacy as well as four Regional Taskforces which focus on policy and advocacy at the state level.

Each of these regional task forces will be chaired by two volunteer co-chairs, who, I'm excited to introduce to all of you in a few slides here. We'll be having four national meetings each year, during which we'll delve into our Federal policy priorities and provide some updates as well as some advocacy skills training, and hear from some exciting guest speakers.

So here are some of the short and long-term goals of our National Taskforce. In the short term we hope to:

- Grow our movement and engage a greater number of advocates to push for meaningful policy change.
- Help you connect with your elected officials.
- Deliver frequent advocacy opportunities and ways to get involved.
- Facilitate media opportunities to highlight your advocacy efforts and bring attention to rare disease policy priorities.
- To answer your questions and help you succeed as an advocate.

In the long-term we are hoping to:

- Drive change and awareness to help make rare diseases a national priority.
- Help you build lasting, productive relationships with your elected officials.
- Prepare and train our community's next generation of advocates.
- Successfully leverage our collective influence and impact to pass legislation that benefits rare patients and families.

So you might be wonderful, how are we going to accomplish these goals? And I'm excited to get into it, but now I want to turn to our Regional Policy & Advocacy Taskforces.

So we split the United States into four Regional Taskforces with the goal of fostering collaboration and sharing strategy between states in the same region, as states in the same region often face similar policy challenges. Each region will have five meetings per year which will be led through a collaboration between NORD's Policy Team and volunteer co-chairs in each region who are rare disease advocates themselves and live in each region.

So now to look at what regions we all live in and who the co-chairs are.

Alright, so Region A. You're in Region A if you live in Washington, Oregon, California, Idaho, Montana, Wyoming, Nevada, Utah, Colorado, Arizona, New Mexico, Alaska, or Hawaii.

Our co-chair for Region A is Nick Kirchoff. Nick's daughter has a rare disorder, and he's worked with us on advocacy efforts in his home state of Colorado over the past few years. He's played an instrumental role in getting a Rare Disease Advisory Council passed in Colorado and has a wealth of advocacy knowledge to share. Unfortunately, Nick wasn't able to join us today, but he asked me to share that he's looking forward to working with all of you to advance policies that help the rare disease community.

So now we'll move on to Region B. Those of you who live in North Dakota, South Dakota, Nebraska, Kansas, Oklahoma, Texas, Minnesota, Iowa, Missouri, Arkansas, Louisiana, Wisconsin, or Illinois. Our Region B co-chair is Andrew Larcher, and I'm going to let Andrew introduce himself and share why the rare disease community and rare disease policy are important to him.

Andrew Larcher (Rare Disease Advocate & Region B Co-Chair):

Hello, everyone! I'm Andrew Larcher. I live in South Dakota. I was attending medical school a couple of years ago in the state of West Virginia at WVU and I started having some health issues to the extent that I was unable to continue medical school, and I ended up being diagnosed with my rare disease, stiff person syndrome, which many of you probably know about due to Celine Dion being diagnosed with it. I'm excited to take this opportunity to work with NORD on rare disease policy. Thank you!

Allison Herrity (Policy Analyst, NORD):

Thank you so much, Andrew, for that introduction. We look forward to working with you, to advance rare disease policy in Region B.

So moving on now to Region C. If you live in Michigan, Indiana, Ohio, Kentucky, West Virginia, Tennessee, North Carolina, South Carolina, Mississippi, Alabama, Georgia, or Florida, you live in Region C like I do!

We have two wonderful volunteer co-chairs in Region C, Pam Judge and Aaron Blocker. Unfortunately, Pam has a family conflict today, and Aaron is needed as a last-minute appointment for his wife, who is 36 weeks pregnant with twins, so I think we can forgive him for that one.

Both Pam and Aaron are looking forward to connecting with our Region C folks in a few weeks at our Regional meetings, but for now I can provide an introduction for each of them.

So Pam's connection to the rare disease community began, as many of ours do, when her son Connor was diagnosed with Neuromyelitis Optica Spectrum Disorder. Wanting to do something to help her son and other people with NMO, Pam and her daughter Chelsea started the Connor B Judge Foundation, a nonprofit focused on raising awareness and research funding for her son's condition that has since joined forces with the Sumaira Foundation. Pam lives in Ohio like me.

Aaron is a rare disease patient, based in Mississippi, who lives with a rare metabolic bone disease, called hypophosphatasia. He has done extensive advocacy work, raising awareness for his condition by sharing the story of his diagnostic journey, including being featured in the Washington Post's Monthly Medical Mysteries column.

Pam and Aaron are both excited to work with the folks in Region C and cannot wait to meet all of you on the Region C meeting in a few weeks.

So last, but certainly not least, we have region D. If you live in a state that we haven't let yet listed, including Virginia, the District of Columbia, Maryland, Delaware, New Jersey, Pennsylvania, New York, Connecticut, Rhode Island, Massachusetts, Vermont, New Hampshire, or Maine, you live in Region D.

To head up Region D, we have our two lovely co-chairs Julie Raskin and Tai Pasquini. And I will give each of them the opportunity to introduce themselves to you. So, Julie, would you like to start?

Julie Raskin (Rare Disease Advocate & Region D Co-Chair):

Sure! Hi, everyone! It's a pleasure to be here today and to be part of the Regional Policy and Advocacy Taskforce. Prior to being a part of this Taskforce, I was the Rare Action Network Volunteer Ambassador for many years in New Jersey and worked on many advocacy projects. We passed our RDAC and the copay accumulator bill so that was really terrific.

And in my day job I am the executive director of Congenital Hyperinsulinism International. My son Benjamin was born with the condition 26 years ago, and we've joined with people all over the world, to create a worldwide movement to improve the lives of people born with the disease. And now I'll pass it over to Tai.

Tai Pasquini (Rare Disease Advocate & Region D Co-Chair):

Thanks, Julie. I'm Tai Pasquini. I also, during my day job, work with Julie as the Research and Policy Director of Congenital Hyperinsulinism International. I live in Massachusetts, and a member of the Massachusetts Rare Disease Advisory Council, and I am a researcher and advocate for rare diseases.

One of my first early-day claim to fames is that I helped create the first State House toolkit back in the very first round of advocacy days. So I'm excited to continue working as a member of the RDAC and this Taskforce on many projects. Thanks so much.

Allison Herrity (Policy Analyst, NORD):

Great, thank you, Julie and Tai! So as I was mentioning earlier, our regional Taskforces will have five meetings per year, which will take place during the week of February 20th, the week of April 3rd, the week of June 26th, the week of October 9th, and the week of December 11th.

The National Taskforce will have four meetings which will take place today, obviously, as well as on May 23rd, September 12th, and December 5th.

While the Taskforce may only have nine total meetings each year, we hope to keep folks engaged with advocacy opportunities and an easy, accessible line of communication throughout the year.

Alright. So I hope you guys are ready to get into some policy priorities because I'm ready to get into some policy priorities.

So here is a little overview of NORD's 2023 Advocacy Agenda. This won't be an exhaustive overview of all of the policy issues that NORD engages on, but these are the ones where I expect us to find the most advocacy opportunities.

First, we have telehealth. So rare disease patients, as I'm sure you all well know, are often forced to travel long distances, and often across state lines, to find a health care provider qualified to provide care for their rare disorder. As we've seen during the COVID-19 pandemic, when telehealth restrictions were

relaxed quite a bit to enable greater telehealth access, telehealth visits can really cut out unnecessary and burdensome travel for rare disease patients and, you know, folks who might not have a rare disorder as well.

They also can allow providers to observe patients in the home setting which can be really helpful for certain conditions as well as provide access to physicians that people with rare disorders sometimes were not even able to see due to the distance.

We've been supporting state level legislation to join the Interstate Medical Licensure Compact, which is a compact that streamlines medical licensure for out-of-state physicians, and thereby increases access to those physicians and we look forward to engaging at the federal level as well as telehealth legislation is introduced in the 118th Congress.

Step Therapy has long been a policy priority for NORD. People with rare diseases need access to the treatments that their doctors prescribe without being forced to unnecessary, or sometimes even dangerously trial other medications when they are, especially when they are, for example, stable on a treatment already. So we support policies at the State and Federal level that would create a clear and accessible exemption process for situations in which being exempted from step therapy is appropriate.

Genetic testing is a prominent issue for the rare disease community, and as such is a priority for NORD's policy team. We look forward to supporting policies that expand access to genetic testing, potentially cutting down on the diagnostic odyssey that patients are often forced to go through, as well as policies to help expand the genetic workforce and thereby expand access to genetic services.

Medicaid programs provide critical access to health care for many rare disease patients, and especially children. We support expanding Medicaid, eligibility in states that have not yet done so, as well as support the Accelerating Kids Access to Care Act, legislation that facilitates coordination across state lines by clarifying the process that which state Medicaid programs can cover services regardless of where a child lives and where their care is received. This is by no means an exhaustive list of all the ways that we engage on Medicaid, but I could have several slides on how much Medicaid work we do.

So we'll move on to newborn screening. Newborn screening is one of the most successful public health programs this country has ever seen. It's particularly important for the rare disease community, as all of the conditions on the recommended uniform screening panel, which is the panel of conditions that the Federal Government suggests states screen for in their state newborn screen programs, and all of the conditions on that recommended uniform screening panel are rare diseases. We support robust and well-funded newborn screening programs at the state level, and as many of you know, we have also worked for many years on Federal newborn screening legislation as well.

So finally, NORD's Rare Disease Advisory Council initiative. Annissa and Alyss work on this particular project quite a lot. Project RDAC has facilitated the enactment of RDACs in 24 States, and we look forward to working to pass even more this year and years in the future. These advisory bodies provide the Rare Disease community with a platform to communicate our needs to their state government and give the rare disease community a seat at the table in their state government.

So moving on to NORD's State Report Card Project. The latest edition of the State Report Card just went live on our website today, so this is a really exciting day for NORD's policy team. To both have the State Report Card go live and have the kickoff meeting for the Policy & Advocacy Taskforce. And it's really

great that they lined up just like that, because we get to share some of the highlights from them with all of you.

So our State report card project for anyone who's not familiar with it is a project in which NORD's policy team evaluates states on their policies that relate to things that impact the rare disease community.

So we cover policies, including Medicaid financial eligibility, medical nutrition, newborn screening, protecting patients in a State Medicaid programs, in which we evaluate states on certain waivers that they apply for and maybe granted to their state Medicaid program, protecting patients in State regulated insurance, Rare disease Advisory Councils, step therapy, telehealth, and then prescription drug out of pocket costs.

So I'm excited to share some of the highlights from this year's edition of the State Report card on our next slide. So in 2022, and as a disclaimer, we compile all of the data in the State Report card that it is recent as of November 1stof the previous year, So anything that you'll see on the State report card website is going to be data from up to November 1st 2022. So sometimes if you see something was passed late in the year, or something that passed early this year, we might not have updated it yet, because, you know, we have to have the time to compile the report card after we get all of the data.

But onto 2022 highlights. we had four States signed step therapy reform legislation into law.

Four States enacted legislation to create a Rare Disease Advisory Council, which, as I mentioned, brings our total number of States up to 24 and we're hoping, to expand that number.

All 50 States and the District of Columbia Screen for at least 31 out of the 37 conditions that are currently on the recommended uniform screening panel.

We had four new States join the Interstate medical Licensure Compact, which is a step towards expanding telehealth, access to providers across State lines, and, as I mentioned in our 2023 advocacy priorities, that's another one that we will definitely be working on this year. There are bills in several States right now to expand the number of States who are members of the interstate medical licensure compact.

12 States expanded medicaid eligibility for low-income residents, and we had several States see their grade improve for the prescription drug out of pocket costs.

So one of the things that we're looking for to go along with the State report card is that we like to feature patient stories related to the nine issues that we advocate, or that we evaluate in the State report card. So if you have a story personally, or if you know of someone who has a story related to one of our issues that we have highlighted in the State Report card, and they have an experience with that, they can share their story with NORD, and we may feature it on the state report card site as well as on social media and other avenues.

So that's a little bit of an overview of the State Report card. I encourage people to check it out. We have the ability for you to read a page that gives a little bit more information than we're able to give on this call about each of the issues that we work on, as well as the ability to click on your state and it'll bring you to kind of your own State report card where you'll be able to see the grade that your State has for all of those nine policy areas.

So that's the State report card, and we've gone over the policy priorities that we've talked about, but how can you get involved, as a member of the Taskforce?

Well, we have several ways. So one of the out ways that many of you are familiar with and are NORD's action alerts. Some of you might already receive them via email. These are alerts that we set up, that allow you to easily contact your legislators about a given policy issue, typically with the option for you to include your own text, which lets you share your story and detail why that specific policy option, or why that specific policy is important to you.

Another opportunity that we find, primarily on the State level, is providing testimony for a bill in a state legislature. So folks can provide oral testimony by sharing their story verbally, usually before a committee or chamber of the legislature, or you can submit written testimony before a hearing begins, and in some states, they let you submit it after as well.

NORD's policy team will work with our volunteer co-chairs to communicate opportunities available to you all to provide oral and written testimony and support of legislation in your state that benefits the rare disease community in the regional meetings that we have discussed are going to be in a few weeks, and we'll have five throughout the year for each region.

We're going to review current bills that are in the state legislatures in each region, and you might be able to identify a bill that you really connect to it. And you know, if you identify a bill in your state that we're not yet tracking the regional meetings are a great opportunity to bring those to our attention, but you can always reach out to NORD's policy team at policy@rarediseases.org.

Another way to help push policy forward is to share your story. I honestly should have put this one at the top. So your rare to see story, whether you're a patient, a caregiver friend, or a family member, a provider, a neighbor, a teacher, you name it. Your story is the most powerful advocacy tool that you have in your toolkit. Without rare disease stories, legislators have no way of knowing of the challenges that the rare disease community face and there's no pressure on them to fix them.

So we provide opportunities to easily share your story, as I mentioned, with us here at Nord, so that we can use them in advocacy materials, or on social media as well as provide opportunities for you to speak directly with your legislators. We also hope to connect you potentially with media outlets to show your story to a wider audience. Not only do policymakers often see stories published by local media, but people in your community may read your story and want to get involved to help the rare disease community as well.

So, as I mentioned, we also want to hear from you. So, if you have an advocacy opportunity that you know of in your local community that you saw somewhere that you think might be something worth sharing with members of your region, or members of the National Taskforce more widely, or if you identify a bill in a your State that you think that we should be tracking, but you're not sure if we are yet, we want to hear from you. So share those ideas with us.

Finally, of course, we want you to be able to engage with other advocates. Really use the members of the task force to collaborate with and share strategy with, to help advance policies that help the rare disease community

Another thing we hope to do is to provide all of you with at least one action alert every month that is something that you can do to help rare disease policy at the federal level as a member of the National Taskforce.

So the first step that, as you know, we're really nailing it in, in developing a working relationship with your elected officials, is that you need to share your story. So this month's action alert that we're gonna focus on is an action that will help you introduce yourself to your elected officials in Congress – to your Senators and your Representative and tell them why you care about rare disease policy and why it's important to you.

So we've created an action alert that will go out in we'll be sending in a follow up email. So, for an example of something that you might share is your story. So this is not my story. I just wanna make a disclaimer, it's an example. But your story could say something like this:

"Shortly after birth I was diagnosed with a rare condition called Isovaleric Acidemia through newborn screening. This means my body can't break down, the amino acid leucine, so I get most of my nutrition through a specialized formula. Without access to a appropriate medical nutrition products I could face brain damage, or worse. I care about rare disease policy, because newborn screening saved my life and medical nutrition legislation has the potential to eliminate a significant financial burden for me and my family." so that's just an example of something that you could share if you wanted to share your story.

So if we want to go to the next slide. So, as I was at detailing before you're going to receive a follow up email with a Zoom recording of this meeting, an action alert to introduce yourself and share your story, with your elected officials, and a form to sign up for the first set of Regional Taskforce meetings. The states are listed in the form, so you won't have to at remember which region you lived in from this meeting, and you'll also be able to view what region you live in on our Taskforce web page, that I mentioned earlier as well.

And then if you have any unanswered questions or if you know of any bills that we should be tracking or have advocacy opportunities to share, email the NORD Policy Team, email me!

And again, thank you so much for joining this meeting. We know that by working together we can accomplish great things, and we're looking forward to working with all of you to improve the lives of rare disease patients and their families.

I'm sure that people have questions, and I've seen some of them coming in in the chat as I've been speaking, but I haven't been able to read them all as I was talking because I was looking at the slides a little bit. So if you have questions, you can put them in the chat.

Question from the chat:

Will NORD be in DC for their Rare Disease Day events?

Allison Herrity (Policy Analyst, NORD):

So if you're referring to Rare Disease Week on Capitol Hill, that is an initiative of the Everylife Foundation, but NORD is the official U.S. sponsor of Rare Disease Day at NIH and you can view our Rare Action Network events for Rare Disease Day, which I think some are virtual and some are in-person.

Question from the chat:

If I am on vacation and I miss a meeting, how can I get the information?

Allison Herrity (Policy Analyst, NORD):

So we plan on recording the meetings, so you can view the meeting afterwards via the recording or read/listen to the transcript, but I am also able to share the slides from the meeting.

Question from the chat:

Will you ever do meetings in the evenings?

Allison Herrity (Policy Analyst, NORD):

So meetings for the National Taskforce will likely be around this time. We try to choose a time that is okay for people in different time zones, but it gets difficult because, you know, once people on the West Coast get out of work, people it's like very late at night for people on the East Coast. But the Regional meetings are at times that are more curtailed to those time zones.

Question from the chat:

Does my disease need to be on the list on NORD's website in order to participate in the Taskforce?

Allison Herrity (Policy Analyst, NORD):

No, your condition does not need to be listed on NORD's website in order to participate in the Taskforce, or in any of NORD's programming. The list of rare diseases on NORD's website is not an exhaustive list of rare diseases, and new rare diseases are being discovered all the time. But no, your disease does not need to be listed on that list for the Taskforce or for any of NORD's programming.

Question from the chat:

When can we expect an invite to our regions task force meeting this month?

Allison Herrity (Policy Analyst, NORD):

So when you fill out the sign up sheet for your regional task force meeting, it'll be similar to this one. You'll get a follow up email thanking you for registering, so you'll be sure that you registered, and we'll send a reminder email as well as an email about 24 hours before the event, with the zoom link as well as the agenda for that meeting.

All right. So, as I mentioned earlier, well, I'm gonna stop the recording before we take a group photo.