NORD RARE DISEASES & ORPHAN PRODUCTS

Breakthrough Summit®

OCT. 19-21, 2025

Agenda subject to change.

SUNDAY, OCTOBER 19, 2025

2:00PM – 8:00PM	**REGISTRATION** *Save time Monday morning by picking up your badge early and joining us for a welcome reception.*
6:00PM – 8:00PM	**SUMMIT WELCOME RECEPTION**
8:00PM	**RARE CANCER COALITION COCKTAIL RECEPTION**

MONDAY, OCTOBER 20, 2025

* All times are ET

7:30AM	**CONFERENCE REGISTRATION AND CONTINENTAL BREAKFAST** *Visit the Poster Hall and Exhibit Hall*
8:00AM	**NORD'S WELCOME & SUMMIT PREVIEW** Pamela K. Gavin - Chief Executive Officer, NORD
8:10AM	**YOUNG VOICES KEYNOTE PANEL** *Advances in rare disease diagnosis and treatment have particular relevance for young people living with rare diseases. Young advocates will share their stories, challenges and hopes for the future.* **Moderator:** Mike Porath, Chief Executive Officer and Founder, The Mighty; Executive Director, Dup15q Alliance
8:40AM	**U.S. FOOD AND DRUG ADMINISTRATION (FDA) ADDRESS *(TBD)***
9:00AM	**NATIONAL INSTITUTES OF HEALTH (NIH) ADDRESS *(TBD)***
9:25AM	**CEO PERSPECTIVES** *CEOs working in the rare space will share their thoughts on the current landscape for advancing science, addressing cost concerns and ensuring that patients and patient needs are the fundamental driving factor.* **Moderator:** Patrick Collins, Vice President of Community & Corporate Affairs, NORD **Speakers:** Pamela Gavin, Chief Executive Officer, NORD Miguel Fernandez Alcalde, President, EMD Serono Catherine Owen Adams, BSc., Chief Executive Officer, Acadia

MONDAY, OCTOBER 20, 2025 *(continued)*

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10:30AM **NETWORKING BREAK**

11:00AM **A FIRESIDE CHAT WITH FDA LEADERSHIP *(TBD)***

FDA senior officials will sit down with NORD's CEO for a conversation about their hopes and priorities and, in particular, how they view the future of rare disease product development and review.

12:00PM **LIGHTNING ROUNDS POSTER PRESENTATIONS**

Authors of the top selected poster abstracts will share their key findings.

12:30PM **LUNCH**

ROOM 1

2:00PM
COMMUNITY VOICES

2:10PM
A NEW ERA FOR RARE DISEASE CLINICAL TRIALS

This session will explore how technology and innovative trial designs address issues that historically have made clinical trials for rare diseases particularly challenging.

Speakers:

Claire Beggs, Executive Director, Global Regulatory Affairs and Drug Safety, Jazz Pharmaceuticals

Kaushik Ghosal, PhD, Amyloidosis Research Consortium ASPIRE Collaboration

Nerissa Kreher, MD, MBA, Chief Medical Officer, Alltrna

3:10PM
A MORAL IMPERATIVE: ADDRESSING THE CHALLENGES OF PEDIATRIC R&D

Even though about two-thirds of Americans with rare diseases are children, pediatric R&D lags far behind adult studies. What are the special challenges of pediatric research and how do we address them?

Moderator: Fumihiko Urano, MD, PhD, Samuel E. Schechter Professor in Medicine, Washington University School of Medicine

Speakers:

Stephanie Fradette, MD, Head of Neuromuscular Development Unit, Biogen

Dylan Ritter, PhD, Director of Scientific and Clinical Initiatives, Dup15Q Alliance

ROOM 2

2:00PM
COMMUNITY VOICES

2:10PM
CHARTING A PATH FORWARD FOR NEWBORN SCREENING

What are the current challenges related to newborn screening and how do we address them? Hear from leading voices in the field as they discuss recent developments in the American newborn screening landscape and actionable strategies for moving forward.

Moderator: Allison Herrity, MPH, Senior Policy Analyst, NORD

Speakers:

Danaé Bartke, Executive Director, HCU Network America

Amy Gaviglio, MS, CGC, Founder and Chief Executive Officer, Connetics Consulting LLC

3:10PM
RARE DISEASE COMMUNITIES OF CARE: INTEGRATING MENTAL HEALTH SUPPORT INTO TREATMENT

Rare disease patients and their families face unique mental health challenges that are often overlooked. This session will explore how mental health support can be integrated into the holistic care model for rare disease patients.

Moderator: Jill Pollander, RN, MSN, Vice President of Patient Services, NORD

Speakers:

Vanessa Acero, LPCC-Licensed Mental Health Therapist

Ray Merenstein, Parent, Director of NAMI CO, Colorado Rare Disease Advisory Council

Andres Trevino, Global Head of Patient Advocacy, Chiesi

MONDAY, OCTOBER 20, 2025 *(continued)*

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4:05PM	**NETWORKING BREAK**
4:30PM	**SPECIAL PLENARY: CURATIVE THERAPIES -- OPPORTUNITIES AND CHALLENGES** *This panel of investors and entrepreneurs will share their perspectives on the outlook for rare disease investment in 2026 and beyond.* **Moderator:** David Scheer, President, Scheer & Company, Inc. **Speakers:** Martin Mackay, PhD, Co-Founder, RallyBio Vinod Mitta – Director, Seamless Care Models Group, CMS Innovation Center, US HHS. Sean P. Nolan, CEO, Taysha Gene Therapies Maha Radhakrishnan, MD, Executive Partner, Sofinnova Investments Philip Ross, Vice Chairman, Jefferies LLC. Stephen Squinto, PhD, Chief Investment Officer, J.P. Morgan Life Science Private Capital David Tischler, CEO, Stealth Mode
5:30PM	**EXHIBIT HALL AND 'SIP IN SCIENCE' POSTER HALL RECEPTION**

TUESDAY, OCTOBER 21, 2025

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7:30AM	**CONTINENTAL BREAKFAST AND REGISTRATION**
8:15AM	**COMMUNITY VOICE KEYNOTE**
8:50AM	**MAKING YOUR VOICE HEARD: RARE DISEASE ADVOCACY AND THE 119TH CONGRESS** *Representatives of the Rare Disease Congressional Caucus will share their thoughts on current and upcoming policy priorities, and how patient advocates can have an impact.*
9:50AM	**CASE STUDIES IN COLLABORATION** *When patient organizations and companies work together in ways that are ethical, practical and creative, patients and families benefit. This session will look at case studies of collaboration and the best practices they illustrate.* **Speakers:** R. Duane Clark, General Manager, U.S. Rare Diseases, Sanofi Sarah Gheuens, MD, PhD, Chief Medical Officer, Head of R&D, Agios
10:45AM	**NETWORKING BREAK**
11:15AM	**HOW FDA REVIEW TEAMS INCORPORATE PATIENT DATA** *Panel composed entirely of FDA reviewers sharing their thoughts and firsthand experiences.*
12:15PM	**NETWORKING LUNCH**
1:30PM	**COMMUNITY VOICES: PATIENT/CAREGIVER STORY**

TUESDAY, OCTOBER 21, 2025 *(continued)*

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1:40PM

WHAT'S NEXT FOR GENE AND CELL THERAPIES?

Co-hosted by NORD and the American Society of Gene and Cell Therapies (ASGCT), this panel will discuss the current state-of-the-art, advances on the horizon and policy and economic challenges.

Moderator: Edward Neilan, MD, PhD, Chief Medical and Scientific Officer, NORD

Speakers:

Terence Flotte, MD, President, ASGCT

2:40PM

THE ROLE OF AI IN DRUG DEVELOPMENT

How can we harness the power of AI to expedite rare disease drug development, while still ensuring safety and efficacy? This panel of experts will explore the many ways AI is being utilized to accelerate and transform drug discovery.

Moderator: Tracey Sikora, Vice President of Research and Clinical Programs, NORD

Speakers:

Bruce Bloom, DDS, Chief Collaboration Officer, Healx, Chief Science Officer, Kabuki Syndrome Foundation

Jian Wang, PhD, VP, Digital Innovation, IQVIA R&D

3:40PM

CLOSING SESSION: NEXT STEPS FOR OPTIMAL IMPACT

NORD's senior leadership and community thought leaders will share perspectives on current priorities, next steps, and how to have optimal impact together in science, public policy, and patient care.

Moderator: Pamela Gavin, CEO, NORD

Speakers:

John Greally, MD, Children's Hospital at Montefiore Einstein

Amy Rick, JD, Director of Strategic Coalition, FDA's Rare Disease Innovation Hub

Cheryl Schwartz, Senior Vice President, U.S. Rare Disease Business Unit Lead and U.S. Commercial Operations, Takeda

Theresa Strong, PhD, Director of Research Programs, Foundation for Prader-Willi Research

4:35PM

CLOSING REMARKS

Pamela K. Gavin - Chief Executive Officer, NORD

NORD®
National Organization for Rare Disorders