

NORD Rare Diseases & Orphan Products Breakthrough Summit
October 20-21, 2025

From Voices to Breakthroughs

Monday, October 20, 2025

7:30am Conference Registration and Continental Breakfast

8:00am NORD's Welcome & Summit Preview

Pamela Gavin, CEO, NORD

8:10-8:40am 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.

8:40-9:00am FDA ADDRESS

9:00-9:20am NIH ADDRESS

9:25-10:25am 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, VP of Community & Corporate Affairs, NORD

- Pamela Gavin – CEO, NORD
- Catherine Owen Adams, BSc. – CEO, Acadia
- Paula Ragan, PhD – CEO and President, X4 Pharmaceuticals
- Miguel Fernandez Alcalde – President, EMD Serono

10:30-11:00 Networking Break

11:00-11:55am A Fireside Chat with FDA Leadership *(invited)*

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.

12:00-12:30pm Lightning Rounds Poster Presentations

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

12:30-1:55pm Lunch

2:00-2:55pm/3:00-3:55pm Breakout Sessions

ROOM 1	ROOM 2
<p>2:00- 2:10pm Community Voices</p> <p>2:10- 3:05pm: A New Era for Rare Disease Clinical Trials</p> <p><i>This session will explore how technology and innovative trial designs address issues that historically have made clinical trials for rare diseases particularly challenging.</i></p> <p>3:10 – 4:05pm: A Moral Imperative: Addressing the Challenges of Pediatric R &D</p> <p><i>Even though about two-thirds of the 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?</i></p>	<p>2:00pm- 2:10pm Community Voices</p> <p>2:10-3:05pm: Earlier Diagnosis to Promote Better Outcomes</p> <p><i>It's well established that, for many rare diseases, earlier diagnosis can greatly improve long-term outcomes. What are the current challenges related to newborn screening, genetic testing and genomic sequencing, and how do we address them?</i></p> <p>3:10 – 4:05pm: Rare Disease Communities of Care: Integrating Mental Health Support into Treatment</p> <p><i>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.</i></p>

4:05-4:30pm Networking Break

4:30-5:30pm 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.

5:30pm Exhibit Hall and “Sip and Science” Poster Hall Reception

Tuesday, October 21, 2025

7:30am Continental Breakfast

8:15-8:45am Day 2 Community Voice Keynote

8:50-9:45am 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- 10:45am Case Studies in Collaboration

When patient organizations and companies work together in ways that are ethical, practical and creative, patients and families will benefit. This session will look at case studies of collaboration and best practices they illustrate.

10:45-11:15am Networking Break

11:15am- 12:10pm- How FDA Review Teams Incorporate Patient Data

Panel composed entirely of FDA reviewers sharing their thoughts and first-hand experiences.

12:15 to 1:25pm Networking Lunch

1:30-1:40pm: Community Voices: Patient/Caregiver Story

1:40 to 2:35pm: What's Next for Gene and Cell Therapies?

This panel will discuss the current state-of-the-art, advances on the horizon and policy and economic challenges.

2:40 to 3:35pm 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.

3:40-4:35 pm 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.

4:35pm Closing Remarks

Pamela Gavin, CEO, NORD