NORD Breakthrough Summit

October 16-17, 2023
Washington, DC

NORD 40 YEARS
RARE. STRONG. TOGETHER.

Reimagining What is Possible, Together

Agenda subject to change.

SUNDAY, OCTOBER 15, 2023

1:00PM – 7:00PM	**REGISTRATION** *Save time Monday morning by picking up your badge early and joining us for a welcome reception.*
5:30PM – 7:00PM	**SUMMIT WELCOME RECEPTION AND POSTER HALL RECEPTION**

MONDAY, OCTOBER 16, 2023

* All times are ET

7:30AM	**CONFERENCE REGISTRATION AND CONTINENTAL BREAKFAST** *Visit the Poster Hall and Exhibit Hall*
8:15AM	**NORD'S WELCOME & SUMMIT PREVIEW** Peter Saltonstall - President and Chief Executive Officer, NORD
8:25AM – 8:55AM	**PATIENT/CAREGIVER KEYNOTE ADDRESS** *Patients and caregivers share stories that illustrate the life-changing impact of the Orphan Drug Act.*
9:00AM – 9:20AM	**FDA COMMISSIONER KEYNOTE ADDRESS** *(invited)*
9:25AM – 10:15AM	**CEO PERSPECTIVES** *Industry leaders share their thoughts on the current environment and outlook for the future in rare diseases and orphan products.* **Speakers:** Eric Dube, PhD - President and Chief Executive Officer, Travere Steve Uden, MD - Co-Founder and Chief Executive Officer, Rallybio
10:15AM – 10:45AM	**NETWORKING BREAK** *Visit the Poster Hall and Exhibit Hall*

MONDAY, OCTOBER 16, 2023 *(continued)*

* All times are ET

10:50AM – 11:40AM

THE IMPACT OF THE INFLATION REDUCTION ACT ON THE RARE COMMUNITY

Experts on pricing, access and public policy discuss how the IRA and other current policy initiatives may affect orphan product development and patient access to care.

Moderator: Heidi Ross, MPH - Vice President of Policy and Regulatory Affairs, NORD

Speakers:

Anna Kaltenboeck - Principal, ATI Advisory

Kristi Martin - Chief of Staff, Center for Medicare, Center for Medicare

Conor Sheehey - Senior Health Policy Advisor, Senate Committee on Finance

Nick Shipley - Chief Advocacy Officer, Biotechnology Innovation Organization (BIO)

11:45AM – 12:00PM

ANNOUNCE/BRIEFLY DISCUSS KEY FINDINGS FROM DIVERSITY SURVEY

12:05PM – 1:30PM

NETWORKING LUNCH OR ROUNDTABLE DISCUSSION GROUPS

Discussion group topics include:

- The Role of Student Advocacy Groups in Driving Change
- Selecting Clinical Trial Endpoints
- Partnering for Progress
- Rare Cancer Breakthroughs
- The Impact of Patient Registries in Accelerating Rare Disease Treatments and Cures
- The Mental Health Needs of Rare Disease Patients and Families
- The ABCs of PFDDs (Patient Focused Drug Development)
- Make Your Voice Heard in State and Federal Policy

1:35PM – 2:25PM

MOVING DEI FROM GOOD INTENTIONS TO EFFECTIVE ACTION

Inequities in clinical research and healthcare have been well-documented. How do we make the leap to truly effective action?

Speakers:

Eileen Novins-Masciale - Chief Program Officer, The Marfan Foundation

Charlotte Owens, MD - Vice President, Head of Center for Health Equity and Patient Affairs, Takeda

Joy Russell - Vice President, External Affairs, Genentech

2:30PM – 3:20PM

THE NEW FRONTIER OF GENE EDITING: CAN WE POTENTIALLY CURE GENETIC DISEASES?

Panel of experts to address state-of-the-science as well as financial/access challenges.

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

Speakers:

David Liu, PhD - Professor, Broad Institute Harvard University

3:20PM – 3:30PM

NETWORKING BREAK

Visit the Poster Hall and Exhibit Hall

MONDAY, OCTOBER 16, 2023 *(continued)*

* All times are ET

3:35PM – 4:25PM

HARNESSING THE POWER OF AI: FROM DATA TO DIAGNOSIS AND IMPROVED OUTCOMES

Artificial intelligence and other innovative use of data show promise for greatly reducing the diagnostic odyssey and supporting more personalized and effective care.

Speakers:

Kimberly Moran, PhD - Head of US Rare Diseases, UCB

4:30PM – 5:00PM

LIGHTNING ROUND POSTER PRESENTATIONS

Authors of the top 5 selected poster abstracts will share their key findings.

5:00PM – 6:00PM

NETWORKING RECEPTION AND POSTER HALL RECEPTION

TUESDAY, OCTOBER 17, 2023

* All times are ET

ROOM A: RESEARCH & DRUG DEVELOPMENT

Track Co-Chairs: PAG and Industry Representatives

8:30AM

OPENING REMARKS FROM TRACK CHAIRS

8:40AM – 9:30AM

WHAT MAKES A RARE DISEASE RESEARCH-READY?

How can patient advocacy groups lay the groundwork to encourage research on their diseases?

Moderator: Aliza Fink, D SC - Director of Research Programs, NORD

Speakers:

Heidi Bjorson-Pennell - Program Manager, Rare As One, Chan Zuckerberg Initiative

Shawn Egan, PhD - Chief Scientific Officer, FamilieSCN2A Foundation

Seemi Khan, MD - Chief Medical Officer and Senior Vice President, Reata Pharmaceuticals

9:35AM – 10:25AM

STRENGTHS AND CHALLENGES OF NON-TRADITIONAL CLINICAL STUDIES

Panelists will discuss how digital health, in-home trials and other non-traditional approaches are changing clinical trials.

Speakers:

Brigid Garelik, MD, MPH - Chief Medical Officer, Childrens Tumor Foundation

Nell Meosky Luo - CEO and Founder, Folia Health

ROOM B: REGULATORY PERSPECTIVES IN RARE DISEASE MEDICAL PRODUCT DEVELOPMENT

Track Co-Chairs: FDA Representatives

8:30AM

OPENING REMARKS FROM TRACK CHAIRS

8:40AM – 9:30AM

BEST PRACTICES IN STAKEHOLDER COMMUNICATION TO FOSTER RARE DISEASE DRUG DEVELOPMENT

Panelists will discuss FDA Center Initiatives, such as the ARC program and Operation Warp Speed.

Speakers:

Scott Hambaugh - Vice President, Global Regulatory Strategy, CSL Behring

9:35AM – 10:25AM

FDA PERSPECTIVES ON ORPHAN PRODUCT DEVELOPMENT

Panelists from CBER, CDER, CDRH and OOPD will share wide-ranging insights on lessons learned over the years and best practices.

10:30AM – 11:00AM

NETWORKING BREAK

Visit the Poster Hall and Exhibit Hall

TUESDAY, OCTOBER 17, 2023

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ROOM A: RESEARCH & DRUG DEVELOPMENT

10:30AM – 11:00AM
NETWORKING BREAK
Visit the Poster Hall and Exhibit Hall

11:05AM – 11:55AM
THE POTENTIAL IMPACT OF DRUG REPURPOSING
Many experts see this as an untapped source of treatments for rare diseases that currently have none.

Speakers:

Bruce Bloom, DDS, JD - Chief Science Officer, Kabuki Syndrome Foundation

Jennifer Butler - Head of Commercial, Solaxa

Nicola Longo, MD, PhD - Professor and Chief Medical Genetics/Pediatrics, University of Utah, Spencer Fox Eccles School of Medicine

Tracey Sikora - Co-Founder, EveryCure

ROOM B: REGULATORY PERSPECTIVES IN RARE DISEASE MEDICAL PRODUCT DEVELOPMENT

11:05AM – 11:55AM
ADDRESSING THE SPECIAL CHALLENGES OF MEDICAL PRODUCT DEVELOPMENT FOR RARE PEDIATRIC DISEASES
Panelists will discuss the unique aspects of pediatric medical product development, recent product approvals and the path forward.

Speakers:

Kathie Bishop, PhD - CSO and Head of Rare Disease, Acadia

Collin Hovinga, PharmD, MS, FCCP - Vice President of the Rare and Orphan Disease Programs, Critical Path Institiute (C-PATH)

12:00PM – 1:25PM

GENERAL LUNCH NETWORKING OR ROUNDTABLE FOCUS GROUPS

Discussion group topics include:

- DEI: From Theory to Practice
- Telling your Story to Advance Awareness
- Partnering for Progress
- How Patient Data is Helping Drive Research Innovation: RDCA-DAP
- Reimagining Pediatric Drug Development
- Selecting Clinical Trial Endpoints
- Examining the Role of AI in Rare R&D
- Newborn Screening Considerations

TUESDAY, OCTOBER 17, 2023 *(continued)*

* All times are ET

ROOM A: DIAGNOSIS & PATIENT CARE

Track Co-Chairs: NORD RD CoE and Industry Representatives

1:30PM
OPENING REMARKS FROM TRACK CHAIRS

1:40PM – 2:30PM
THE TOPIC NO ONE TALKS ABOUT: MENTAL HEALTH AND RARE DISEASES

Too little attention has been paid to the fact that living with a rare disease often involves mental health challenges. This panel will explore what needs to be done to better meet patient/caregiver needs.

Moderator: Al Freedman, PhD - Psychologist, Freedman Counseling Associates

Speakers:

Sheldon Garrison, PhD - Scientist, Rogers Behavioral Health

2:30PM – 2:45PM
BREAK

2:50PM – 3:40PM
THE POWER OF THE COLLECTIVE: A NATIONAL NETWORK OF RARE DISEASE CENTERS

When the nation's leading rare disease research and treatment centers work together, health inequities are reduced, and the benefit will be felt in diagnosis, research and patient care.

ROOM B: ORPHAN PRODUCT DEVELOPMENT

Track Co-Chairs: Rare Cancer Coalition and Industry Representatives

1:30PM
OPENING REMARKS FROM TRACK CHAIRS

1:40PM – 2:30PM
THE SHORT- AND LONG-TERM OUTLOOK FOR ORPHAN PRODUCT INVESTMENT

A panel of investors will explore the outlook for orphan product investment over the near future and the next several years.

2:30PM – 2:45PM
BREAK

2:50PM – 3:40PM
CAN WE OVERCOME THE CHALLENGES FACING CELL AND GENE THERAPIES?

Initially perceived as transformational treatments, cell and gene therapies are now viewed by some through a less enthusiastic lens because of issues related to cost, access and duration of benefit. Is the original scenario still possible?

Speakers:

Diana Castro, MD - Founder and Director, Neurology Rare Disease Center

3:50PM – 4:30PM **CLOSING SESSION**