Agenda subject to change.

SUNDAY, OCTOBER 20, 2024

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 21, 2024

* 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** Pamela K. Gavin - Chief Executive Officer, NORD
8:30AM	**OPENING PATIENT/CAREGIVER KEYNOTE**
9:00AM	**FDA COMMISSIONER KEYNOTE ADDRESS (INVITED)**
9:40AM	**CEO PERSPECTIVES PANEL** **Moderator:** Patrick Collins - Vice President of Community and Corporate Affairs, NORD **Speakers:** Giacomo Chiesi – Head of Global Rare Disease and Member of the Board, Chiesi Pharmaceuticals Brian Goff – Chief Executive Officer, Agios Pharmaceuticals Kate Haviland – President and CEO, Blueprint Medicines Rachid Izzar – Executive Vice President, Global Product Strategy & Commercialization, Biogen Julie Kim – President, US Business Unit and US Country Head, Takeda
10:45AM	**NETWORKING BREAK**
11:20AM	**EMBRACING INNOVATION TO ENHANCE CLINICAL TRIAL SUCCESS** *Advances in science and technology have brought us to the brink of a new era in rare disease research. A panel of experts discusses how best to apply and build upon these new capabilities in the way clinical trials are designed and conducted.* **Moderator:** Dr. Rachele Hendricks-Sturrup, DHSc, MSc, MA - Research Director, Real-World Evidence (RWE), Duke-Margolis Institute for Health Policy **Speakers:** Patrizia Cavazzoni, MD - Director, Center for Drug Evaluation & Research (CDER), U.S. Food & Drug Administration Mat Davis - VP, Data Science, Jazz Pharmaceuticals Dominique C. Pichard, MD, MS - Director, Division of Rare Diseases Research Innovation, NIH
12:15PM	**LUNCH**

MONDAY, OCTOBER 21, 2024 *(continued)*

* All times are ET

1:30PM

CAN WE MAKE GENE THERAPY MORE ACCESSIBLE?

The promise of gene and cell therapies has brought great hope to the rare community, but access remains a huge challenge. How can we work together to make manufacturing more efficient and ensure equitable access to these life-saving treatments?

Moderator: P.J.Brooks, PhD -Deputy Director, Division of Rare Diseases Research Innovation, NCATS, NIH

Speakers:

David Barrett, JD - CEO, American Society for Gene and Cell Therapy (ASGCT)

Nicole Gaudelli - Entrepreneur in Residence, Google Ventures

Peter Marks, MD, PhD - Director, Center for Biologics Evaluation & Research (CBER), US Food and Drug Administration (FDA)

2:25PM

THE GROWING ROLE OF MEDICAL DEVICES

For both diagnosis and treatment, medical devices are a topic of increasing importance in the rare community. This panel will explore the outlook for creative new applications of device technologies to improve patient lives.

Speakers:

Michelle Tarver, MD, PhD - Interim Director, Center for Devices and Radiological Health (CDRH), US Food and Drug Administration

Matt Wetzel - Partner, Goodwin Procter LLP

3:20PM

NETWORKING BREAK

3:35PM

BALANCING COST, INNOVATION AND ACCESS

This session will bring diverse perspectives to the table for an honest conversation about innovation, access and a sustainable, affordable healthcare system.

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

Speakers:

Mary Dwight - Senior Vice President of Policy and Advocacy, Cystic Fibrosis Foundation

Jason Spangler MD, MPH, FACPM - CEO, Center for Innovation & Value Research

4:30PM

LIGHTNING ROUND POSTER PRESENTATIONS

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

5:00PM

NETWORKING RECEPTION AND POSTER HALL RECEPTION

TUESDAY, OCTOBER 22, 2024

* All times are ET

7:30AM

CONFERENCE REGISTRATION AND CONTINENTAL BREAKFAST

Visit the Poster Hall and Exhibit Hall

8:30AM

DAY 2 OPENING REMARKS AND PATIENT/CAREGIVER KEYNOTE

Speakers:

Mousumi Bose, PhD - Rare Community Member and Associate Professor, Department of Nutrition and Food Studies, College for Community Health, Montclair State University

8:45AM

NIH DIRECTOR REMARKS (INVITED)

TUESDAY, OCTOBER 22, 2024 *(continued)*

* All times are ET

9:00AM

BRINGING RESEARCH TO PATIENTS TO BE MORE INCLUSIVE

Bringing clinical research to where potential participants live, work and receive their medical care is critically important as we seek to address inequities in trial participation. This panel will talk about current initiatives to meet this challenge.

Speakers:

Kristin Schneeman - Senior Director, FasterCures, Milken Institute Health

ENVIRONMENT FOR INNOVATION

9:50AM

THE EVOLVING POLICY LANDSCAPE: POLICY PRIORITIES IN RARE FOR 2025 AND BEYOND

What are the key policy issues that need collective community action at this moment in time?

10:40AM

NETWORKING BREAK

11:15AM

HOW INVESTORS SEE THE RARE SPACE NOW

Some market analysts have predicted a cooling of investor interest in rare diseases. This panel will explore the current and future outlook for investment in orphan product development.

Moderator: David Scheer - President, Scheer & Company, Inc.

Speakers:

Martin Mackay, PhD - Co-Founder and Executive Chairman, RallyBio

Sukumar Nagendran, MD - President of R&D, Taysha Gene Therapies

Maha Radhakrishnan, MD - Executive Partner, Sofinnova Investments

Tal Zaks, MD, PhD - Partner, OrbiMed

HARNESSING THE POWER OF PATIENTS

9:50AM

ENHANCING THE POWER OF PATIENT DATA

External control arms, Real-World Evidence, clinical outcomes assessments and more will be part of this conversation about patient data as a primary driver of success.

Moderator: Gabrielle Rushing, PhD - Science Program Director, CSNK2A1 Foundation

Speakers:

Kimberly Smith, MD, MS - Senior Medical Advisor, Real-World Evidence Analytics, Office of Medical Policy, CDER, FDA

10:40AM

NETWORKING BREAK

11:15AM

EXPLORING STRATEGIES FOR STREAMLINING TRIALS

A panel of experts will discuss strategies for making clinical trials shorter, more patient-friendly and more likely to succeed.

Moderator: Samuel Mackenzie, MD, PhD - University of Rochester

Speakers:

Catherine Pilgrim-Grayson, MD, MPH - Director of CDER Office of New Drugs, Division of Rare Disease and Medical Genetics, FDA

12:10PM **LUNCH**

TUESDAY, OCTOBER 22, 2024 *(continued)*

* All times are ET

1:30PM **BREAKOUT SESSIONS**

DIAGNOSIS AND PATIENT CARE

1:30PM
OPENING REMARKS FROM PATIENT/CAREGIVER

1:40PM
THE CURRENT STATE & FUTURE OUTLOOK FOR NEWBORN SCREENING

Newborn screening successes and challenges, inequities among states and the outlook for the future all will be discussed.

Moderator: Allison Herrity - Senior Policy Analyst, NORD

Speakers:

Mei Baker, MD, FACMG - Professor, Division of Genetics and Metabolism, University of Wisconsin School of Medicine and Public Health; Director of Newborn Screening Program, Wisconsin State Laboratory of Hygiene

Joel Cartner, Esq. - Director, Access Policy, Muscular Dystrophy Association

Holly Peay, PhD, MS - Senior Research Scientist, RTI International

2:30PM
QUICK BREAK

2:45PM
HOW CAN AI HELP THE RARE COMMUNITY NOW?

We hear a lot about the future of AI but what can it do to shorten the diagnostic odyssey, create pathways to treatments and improve patient care now?

Speakers:

Joseph Zabinski, PhD, MEM - VP and Head of Commercial Strategy and AI, OM1

PATHWAYS TO PATIENT ACCESS

1:30PM
OPENING REMARKS FROM PATIENT/CAREGIVER

1:40PM
BALANCING COST, INNOVATION AND ACCESS

This session will bring diverse perspectives to the table for an honest conversation about innovation, access and a sustainable, affordable healthcare system.

2:30PM
QUICK BREAK

2:45PM
DISPARITIES AMONG STATES IN ACCESS TO GENETIC TESTING

A panel of experts will discuss disparities in access to genetic testing and share thoughts on how to address this challenge.

Moderator: Josh Trent - Managing Principal, Leavitt Partners

3:40PM **CLOSING PLENARY**

BLUE SKY THINKING: WHAT STRATEGIES WILL MOVE THE NEEDLE MOST FOR THE RARE COMMUNITY?

Creative thinkers from across the community will share their thoughts on key strategies to advance innovation and improve the lives of patients.

Speakers:

Abla Creasey, PhD - Vice President of Therapeutics Development at California Institute of Regenerative Medicine (CIRM)

David Fajgenbaum, MD, MBA, Msc - Co-Founder & President, Every Cure

Tricha Shivas - Chief of Staff and Strategy, Foundation for Sarcoidosis Research

Hilary Marston, MD, MPH - Chief Medical Officer, FDA

4:30PM **CLOSING REMARKS**