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**

MONDAY, OCTOBER 21, 2024

* All times are ET

7:30AM **CONFERENCE REGISTRATION AND CONTINENTAL BREAKFAST**

Visit the Poster Hall and Exhibit Hall

8:30AM **NORD'S WELCOME & SUMMIT PREVIEW**

Pamela K. Gavin - Chief Executive Officer, NORD

8:45AM **OPENING PATIENT/CAREGIVER KEYNOTE**

Maria Kefalas, PhD, MA - Founder, The Calliope Joy Foundation and Cure MLD
Tanita Allen - Rare Community Member
Maria Miller - Rare Community Member

9:15AM **NIH VISION FOR RARE**

Joni L. Rutter, PhD – Director, National Center for Advancing Translational Sciences (NCATS), NIH

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:
P.J. Brooks, PhD - Deputy Director, Division of Rare Diseases Research Innovation, NCATS, NIH
Patrizia Cavazzoni, MD - Director, Center for Drug Evaluation & Research (CDER), U.S. Food & Drug Administration
Mat Davis - VP, Data Science, Jazz Pharmaceuticals
Maria Santaella, PhD(c), MSN, RN-BC, CPHON - Vice President of Research Strategy, National Bleeding Disorders Foundation

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.

Moderator: Joseph Sapiente - Senior Vice President, Science and Technology, Medical Device Innovation Consortium (MDIC)

Speakers:

Rich Brennan, MA - Vice President, Federal Affairs, ALS Association

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:

R. Duane Clark - General Manager, U.S. Rare Diseases, Sanofi

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

Lee Fleisher, MD, LLM – Principal and Founder, Rubrum Advising; Former CMS Chief Medical Officer and Director of the Center for Clinical Standards and Quality

Gemma Mayman - Global Rare Disease Commercial Lead, Pfizer

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

4:30PM

LIGHTNING ROUND POSTER PRESENTATIONS

Authors of the top 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

8:15AM

FIRESIDE CHAT: FROM DRUG APPROVAL TO PATIENT ACCESS

Speakers:

Robert M. Califf, MD, Commissioner of Food and Drugs, Food and Drug Administration (FDA)

Meena Seshamani, MD, PhD, Deputy Administrator and Director of the Center for Medicare at the Centers for Medicare & Medicaid Services (CMS)

9:00AM

THE ROAR OF RARE: PATIENT/CAREGIVER KEYNOTE

Speaker:

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

TUESDAY, OCTOBER 22, 2024 *(continued)*

* All times are ET

9:10AM

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.

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

Speakers:

Jim Palma - CEO, TargetCancer Foundation

Joni L. Rutter, PhD – Director, National Center for Advancing Translational Sciences (NCATS), NIH

Kristin Schneeman - Senior Director, FasterCures, Milken Institute Health

Jenifer Waldrop - Executive Director, Rare Disease Diversity Coalition (RDDC)

ENVIRONMENT FOR INNOVATION

10:10AM

POLICY PRIORITIES IN RARE FOR 2025 AND BEYOND

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

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

Speakers:

Jordan Brossi – Senior Policy Advisor, Office of Congresswoman Anna G. Eshoo (CA-16)

Brian Connell - Vice President for Federal Affairs, Leukemia & Lymphoma Society

Jennifer Dexter – Vice President, Policy, National Heath Council

Barrett Tenbarge – General Counsel for Ranking Member Cassidy, Senate Health, Education, Labor, and Pensions (HELP) Committee

11:00AM

NETWORKING BREAK

11:20AM

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

10:10AM

ENHANCING THE POWER OF PATIENT DATA

Patient registries, 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 - Chief Scientific Officer, CSNK2A1 Foundation

Speakers:

Jill Kiernan - Executive Director, TBRS Community

Rosa Sherafat-Kazemzadeh, MD - CBER Office of Therapeutic Products, Office of Clinical Evaluation

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

Yasmine Wasfi, MD, PhD - Executive Vice President, Head of Clinical Development and Clinical Operations, Savara

11:00AM

NETWORKING BREAK

11:20AM

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:

Kendall Davis, MPH - Director, Advocacy & Engagement Strategy, Center for Rare Diseases, ICON

Lola Fashoyin-Aje, MD, MPH - Director of the Office of Clinical Evaluation (Cell and Gene Therapies), Super Office of Therapeutics Products(OTP), CBER, FDA

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

TUESDAY, OCTOBER 22, 2024 *(continued)*

* All times are ET

1:30PM **BREAKOUT SESSIONS**

DIAGNOSIS AND PATIENT CARE

1:30PM
OPENING REMARKS FROM PATIENT/CAREGIVER
Mayra Martinez - Rare Community Member

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, MPH - 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 and Director, Early Check Program, RTI

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?

Moderator: Gregory Levine, JD - Ropes & Gray LLP

Speakers:

Qi Liu, PhD, MStat, FCP - Associate Director for Innovation and Partnership, OCP, OTS, CDER, FDA

Nara Sobreira, MD, PhD - Associate Professor of McKusick-Nathans Department of Genetic Medicine, Associate Professor of Pediatrics, Johns Hopkins University

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

PATHWAYS TO PATIENT ACCESS

1:30PM
OPENING REMARKS FROM PATIENT/CAREGIVER
Guadalupe Hayes-Mota, MBA, MS, MPA - CEO, Healr Solutions, Steering Committee Member, MA RDAC

1:40PM
NAVIGATING THE COVERAGE HURDLES TO RARE DISEASE ACCESS
This session will bring diverse perspectives to the table for an honest conversation about innovation, access and a sustainable, affordable healthcare system.

Moderator: Michelle Rice - Michelle Rice and Associates

Speakers:

Mike Baldzicki, CRCM – Chief Revenue Officer, Lyceum

Kellyn Madden, MS - Patient Engagement Manager, Friedreich's Ataxia Research Aliance (FARA)

Sal J. Morana RPh, PhD, EVP - Pharmacy Benefits Lead, Alliant Employee Benefits

Lauren Walrath - VP, Public Affairs- North America, Kyowa Kirin, Inc.

2:30PM
QUICK BREAK

2:45PM
DISPARITIES 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

Speakers:

Christal Delagrammatikas, PhD - Director of Research, Co-founder, Malan Syndrome Foundation

Fuki M. Hisama, MD, FACMG - Division of Medical Genetics, University of Washington School of Medicine

Holly Paparounis - Associate Director, Medicaid Market Access, GeneDx

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 - Executive Strategy Officer- Rare Disease, California Institute of Regenerative Medicine (CIRM)

Tom DiLenge - Senior Partner, Global Public Policy, Regulatory & Governmental Strategy, Flagship Pioneering

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

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

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

4:30PM **CLOSING REMARKS**