9:00AM to 9:30AM  PATIENT/CAREGIVER OPENING ADDRESS

Innovation Transforms Lives

A panel of patients and caregivers will describe how their lives have been changed by medical capabilities that represent today’s climate of innovation.

Speakers and the NORD Member Organizations they Represent:
Jennifer Beck; American Porphyria Foundation
Amy Medina; Cure SMA
Mark Sleeper; Cystic Fibrosis Foundation

9:30AM to 9:55AM  KEYNOTE ADDRESS

Stephen M. Hahn, MD; Commissioner of Food and Drugs, US Food and Drug Administration

10:00AM to 11:00AM  PLENARY

What We’ve Learned from COVID-19 and How We Can Apply It

Panelists will share their perspectives on what we’ve learned from the COVID-19 experience and how we can apply this new knowledge to build a stronger healthcare system for the future.

Moderator: Shafali Spurling Jeste, MD; Associate Professor in Psychiatry, Neurology and Pediatrics, UCLA David Geffen School of Medicine Los Angeles, CA; NORD Board Member

Speakers:
Adam Gluck; Head, U.S. and Sanofi Genzyme Corporate Affairs, Sanofi Genzyme
Michelle McMurry-Heath, MD, PhD; President & Chief Executive Officer, Biotechnology Innovation Organization (BIO)
Kathleen Sullivan, MD, PhD; Professor of Pediatrics, Wallace Chair of Pediatrics Division of Allergy Immunology, Children’s Hospital of Philadelphia (CHOP); Medical Advisor, Immune Deficiency Foundation
Monica Webb Hooper, PhD; Deputy Director National Institute on Minority Health and Health Disparities, National Institutes of Health (NIH)

11:00AM to 11:10AM  NETWORKING BREAK

11:10AM to 12:00PM  POLICY PLENARY

Perspectives From The Hill: The Year to Date and What’s Ahead

NORD’s Vice President for Policy & Regulatory Affairs will moderate a panel of Capitol Hill staff members discussing policy developments during 2020 and looking ahead to possible post-election scenarios.

Moderator: Rachel Sher, JD; VP Policy and Regulatory Affairs, National Organization for Rare Disorders (NORD)

Speakers:
Nick Bath; Health Policy Director, US Senate HELP Committee
Ryan Long; Deputy Staff Director House Committee on Energy & Commerce
Stuart Portman; Health Policy Advisor US Senate Committee on Finance
Wendell Primus; Senior Policy Advisor on Budget and Health to Congresswoman Nancy Pelosi (D-CA)

12:00PM to 12:30PM  LUNCH BREAK
### TRACK A
**New Approaches to Advancing Diagnosis**

**12:30PM to 1:10PM**

**Update from Global Commission to End Diagnostic Odyssey**

**Moderator:** Pamela K. Gavin; Chief Strategy Officer, National Organization for Rare Disorders (NORD)

**Speakers:**
- Julian Ispa; President & Founder, Foundation 29
- Mike Porath; Founder & CEO, The Mighty
- Debra Regier, MD, PhD; Medical Geneticist, Rare Disease Institute, Children's National

**1:15PM to 1:55PM**

**Whole Genome Sequencing and Other Tools to Promote Early Diagnosis**

**Moderator:** Danielle Boyce; Associate Director of Research Programs, National Organization for Rare Disorders (NORD)

**Speakers:**
- David Keane; Senior Genomic Consultant, GeneDx
- Lydia Shenuoda, PharmD; Sr. Scientific Director Global Medical Affairs, Novartis Gene Therapies

**2:00PM to 2:40PM**

**Advancing Access to Diagnostic Testing**

**Moderator:** Marshall Summar, MD; Director, Rare Disease Institute, Children's National, NORD Board Member

**Speakers:**
- Christine Munro, MS MPH, CGC; Licensed Certified Genetic Counselor, Laboratory Services, Children's Hospital of Pittsburgh of UPMC
- Danyelle Sun; Wisconsin Rare Action Network Volunteer State Ambassador, National Organization for Rare Disorders (NORD)
- Karmen Iruzpeck, MS; CGC; Director, Clinical Trial Services, Director, Ocular & Rare Disease Genetics Services, InformeDNA

### TRACK B
**An Era of Innovation in Research**

**12:30PM to 1:10PM**

**Designing a Regulatory-Ready Natural History Study**

**Moderator:** Vanessa Boulanger; Director of Research Programs, National Organization for Rare Disorders (NORD)

**Speakers:**
- Martin Ho, PhD; Associate Director, Office of Biostatistics & Epidemiology, Center for Biologics Evaluation & Research, FDA
- Kristi Mitchell, MPH; Practice Director, Center for Healthcare Transformation, Avaral Health
- Marc Yale; Executive Director, International Pemphigus and Pemphigoid Foundation (IPPF)

**1:15PM to 1:55PM**

**Latest News on Gene Therapy and Genome Editing Research**

**Moderator:** Phillip Tai, PhD; Assistant Professor, Director, VIDE Program, Horae Gene Therapy Center, Department of Microbiology and Physiological Systems, University of Massachusetts Medical School

**Speakers:**
- Paul Solari, MD; Global Medical Lead, Hemophilia, BioMarin Pharmaceutical Inc.
- Laura Truitoiu, PhD; Director of Research, Association for Creative Deficiencies
- Kate Zhang, PhD; VP of Biological Development, Editas

**2:00PM to 2:40PM**

**Streamlining Orphan Drug Development**

**Moderator:** Kay Holcombe, MS; Critical Path Institute, NORD Board Member

**Speakers:**
- Julie Breneiser; Executive Director, Goltin Syndrome Alliance
- Camilla Lauritzen, MSc, MCC, MMBA; Chief Patient Officer, Leo Pharma
- Terence Sanger; MD, PhD; Vice President for Research and Chief Scientific Officer; Professor of Electrical Engineering and Computer Science, UCI School of Engineering; Vice Chair of Research for Pediatrics, UCI School of Medicine, CHOC Children's Hospital of Orange County
- Shoshana Shendelman, PhD; Founder & Chief Executive Officer, Applied Therapeutics

### TRACK C
**A New Era for Patient Organizations**

**12:30PM to 1:10PM**

**Moving into a Virtual World: Conferences, Fundraisers & Grassroots Advocacy**

**Moderator:** Debbie Drell; Director of Membership, National Organization for Rare Disorders (NORD)

**Speakers:**
- Kristen Angell; Associate Director of Advocacy, National Organization for Rare Disorders (NORD)
- Alice Houk, MPS; Senior Director of Health Professional Programs, Apraxia Anemia & MDS International Foundation
- Vanessa Vogel-Farley; Executive Director, Dup15q Alliance

**1:15PM to 1:55PM**

**Designing and Implementing a Strategic Plan in the Wake of a Pandemic**

**Moderator:** Lisa Sarfaty; Director of Strategic Planning & Project Development, National Organization for Rare Disorders (NORD)

**Speakers:**
- Melissa Bryce Gamble; Executive Director & Co-Founder, The Global Foundation for Peroxisomal Disorders
- Miriam O'Day; President & CEO, Alpha-1 Foundation
- Lisa-Anne Thompson Taylor; Chief Executive Officer, Board Veritas

**2:00PM to 2:40PM**

**How Patient Organizations Can Encourage Research on their Disease**

**Moderator:** Allison Seebald; Senior Research Programs Manager, National Organization for Rare Disorders (NORD)

**Speakers:**
- Deanna Porter; Management Analyst, Office of Rare Diseases Research (ORDR), National Center for Advancing Translational Sciences (NCATS)
- Sandra Sermone; Management Analyst, Office of Rare Diseases Research (ORDR), National Center for Advancing Translational Sciences (NCATS)
- Theresa Strong, PhD; Director of Research Programs, Foundation for Prader-Willi Research

**2:45PM to 3:00PM**

**NETWORKING BREAK**
3:00PM to 3:45PM PLENARY

International Perspectives on Innovation, Affordability and a Sustainable Healthcare System

A panel of global patient advocates, industry representatives, and others will discuss challenges and opportunities related to creating sustainable healthcare for all.

Moderator: Durhane Wong-Rieger, PhD; President & CEO, Canadian Organization for Rare Disorders

Speakers:
- Fritz Bittenbender; Senior Vice President, Access and External Affairs, Genentech
- Bert Bruce; VP Global Commercial Development Rare Disease, Pfizer
- Yann le Cam; Chief Executive Officer, EURORDIS
- Charles Holmes, MD; Director, Center for Innovation in Global Health, Professor of Medicine, Georgetown University School of Medicine

THURSDAY, OCTOBER 8, 2020

8:50AM DAY 1 SUMMARY & DAY 2 PREVIEW

Peter L. Saltonstall; President and Chief Executive Officer, National Organization for Rare Disorders (NORD)

9:00AM to 9:45AM PLENARY

Telehealth and Rare Diseases

Panelists will discuss the current status of telehealth and its particular relevance to those living with rare diseases, with emphasis on related policy questions and issues.

Moderator: Heidi Ross; Director of Policy, National Organization of Rare Disorders (NORD)

Opening Remarks: Congresswoman Robin L. Kelly; Representative D-IL 2nd District, State of Illinois

Speakers:
- Susan Berry, MD; Professor, Division of Genetics and Metabolism Departments of Pediatrics and Genetics, Cell Biology & Development, University of Minnesota
- Dennis V. Chornenky, MBA, MPH, MS; White House Presidential Innovation Fellow, Executive Office of the President, Office of Science and Technology Policy
- William Marsh, MD; State Representative, New Hampshire
- Brooke Thomas; Alabama Rare Action Network Volunteer State Ambassador, National Organization for Rare Disorders

9:45AM to 10:30AM NIH PLENARY

Changing the Clinical Development Paradigm

This panel of NIH senior staff will explore new frontiers in research and how to advance the development of treatments by moving beyond one-disease-at-a-time.

Moderator: Christopher Austin, MD; Director National Center for Advancing Translational Sciences, National Institutes of Health (NIH)

Speakers:
- Walter Koroshetz, MD, PhD; Director, National Institute of Neurological Disorders and Stroke (NINDS)
- Karlyne Reilly, PhD; Director, National Cancer Institute Rare Tumor Initiative, Center for Cancer Research
- Charles Venditti, MD, PhD; Head Organic Acid Research Section, Senior Investigator, National Human Genome Research, National Institutes of Health (NIH)

10:30AM to 10:45AM NETWORKING BREAK

10:45AM to 11:30AM FDA PLENARY

A New Era for Regulatory Science

A panel of FDA speakers will discuss how the landscape has changed this year and how this has become “A New Era for Regulatory Science.”

Moderator: Khair ElZarrad, PhD; Deputy Director, Office Medical Policy- CDER, US Food and Drug Administration

Speakers:
- Hylton Joffe, MD; Acting Director, Office of Rare Diseases, Pediatrics, Urologic and Reproductive Medicine, US Food & Drug Administration
- Larissa Lapteva, MD; Associate Director, DCEPT/OTAT Center for Biologics Evaluation and Research, US Food & Drug Administration
- Janet Maynard, MD, MHS; Director, Office of Orphan Products Development, US Food & Drug Administration
- Donna Rescoe, PhD; US Food & Drug Administration

11:30AM to 12:15PM PLENARY

An Update from FDA Centers

NORD’s CEO will moderate an information conversation with CBER Director Peter Marks MD, PhD, and CDER Deputy Director Patrizia Cavazzoni MD

Moderator: Peter L. Saltonstall; President & CEO, National Organization for Rare Disorders (NORD)

Speakers:
- Patrizia Cavazzoni, MD; Deputy Director for Operations- CDER, US Food & Drug Administration
- Donna Rescoe, PhD; US Food & Drug Administration
- Peter Marks, MD, PhD; Director, CBER, US Food and Drug Administration
This track will be co-hosted by NORD and the Rare Cancer Coalition

12:45PM to 1:30PM Increasing Application of Patient-Reported Data in Rare Oncology

**Moderator:** John Hopper; President, Fibrolamellar Cancer Foundation

**Speakers:**
- Vishal Bhutnagar, MD; Associate Director for Patient Outcomes, Oncology Center of Excellence, US Food and Drug Administration
- Corrie Painter, PhD; Associate Director of Operations and Scientific Outreach for the Broad Cancer Program, Associate Director of Count Me In, Broad Institute

1:30PM to 2:15PM Current Trends & Future Directions for Precision Oncology

**Moderator:** Jim Palma; Executive Director, TargetCancer Foundation

**Speakers:**
- James Geller, MD, FAAP; Professor of Pediatrics, Medical Director, Kidney & Liver Tumor Programs, Director, Advanced Cancer Therapeutics Network, Director, Pilot Translational & Clinical Studies Program, CCTST, Cincinnati Children's Hospital Medical Center, Division of Oncology
- Peter Langmuir, MD; Group Vice President, Oncology Targeted Therapeutics, Incyte
- Ashley Ward, MD; Senior Director, Clinical Development, Foundation Medicine Inc.

2:15PM to 3:00PM DISCUSSION AND NETWORKING

**Continuation of Plenary or Breakout Sessions:**
- Strategic Planning in the Wake of a Pandemic
- Update from Global Commission to End Diagnostiic Odyssey
- Orphan Product Investment in the Wake of COVID-19
- Designing Regulatory-Ready Natural History Study

**Innovative Approaches to Rare Cancer Therapies**

**New Topics:**
- Innovative Approaches to Rare Cancer Therapies
- Moving Into a Virtual World
- Telehealth and Rare Diseases

7:00PM to 8:30PM THE 2020 RARE IMPACT AWARDS STREAMING LIVE!

Registration is complimentary, but you must register to receive access.

Register here: rarediseases.org/rare-impact-awards

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