

ENTERING A NEW ERA

Now Virtual: October 8-9, 2020



#NORDSUMMIT | nordsummit.org

Bringing Transformational Therapies to Patients with Rare Diseases During Challenging Times

THURSDAY, OCTOBER 8, 2020

8:50AM

NORD'S WELCOME & SUMMIT PREVIEW

Peter L. Saltonstall, President and Chief Executive Officer, NORD

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:

Jennifer Beck - American Porphyria Foundation

Amy Medina - Board of Directors, Cure SMA, Caregiver

Mark Sleeper - Cystic Fibrosis Foundation

9:30AM to 10:10AM

KEYNOTE ADDRESS

10:10AM 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, 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

Closed captioning of sessions available with support from:



12:30PM to 2:35PM BREAKOUT SESSIONS

TRACK A	TRACK B	TRACK C
New Approaches to Advancing Diagnosis	An Era of Innovation in Research	A New Era for Patient Organizations
<p>12:30PM to 1:10PM Update from Global Commission to End Diagnostic Odyssey</p> <p>Moderator: Pamela Gavin - Chief Strategy Officer, National Organization for Rare Disorders (NORD)</p> <p>Speakers: Julian Isla - Foundation 29 Mike Porath - Founder & CEO, The Mighty Debra Reiger, MD - Children's National and the Rare Disease Institute</p> <p>1:15PM to 1:55PM Whole Genome Sequencing and Other Tools to Promote Early Diagnosis</p> <p>Moderator: Danielle Boyce - Associate Director of Research Programs, National Organization for Rare Disorders (NORD)</p> <p>Speakers: David Keane - Senior Genomic Consultant, GeneDx Susan Manganaro, MD - Executive Medical Director, Avexis</p> <p>2:00PM to 2:40PM Advancing Access to Diagnostic Testing</p> <p>Moderator: Marshall Summar, MD - Director, Rare Disease Institute, Children's National, NORD Board Member</p> <p>Speakers: Christine Munro - CHOP (Children's Hospital of Pittsburgh) Danyelle Sun - Wisconsin Rare Action Network Volunteer State Ambassador, National Organization for Rare Disorders (NORD) Karmen Trzupke, MS, CGC - Director, Clinical Trial Services, Director, Ocular & Rare Disease Genetics Services, InformedDNA</p>	<p>12:30PM to 1:10PM Designing a Regulatory-Ready Natural History Study</p> <p>Moderator: Vanessa Boulanger - Director of Research Programs, National Organization for Rare Disorders (NORD)</p> <p>Speakers: Martin Ho - Associate Director, Quantitative Patient Inputs & RWE, US Food & Drug Administration Kristi Mitchell, MPH - Practice Director, Center for Healthcare Transformation, Avalere Health Marc Yale - Executive Director, International Pemphigus and Pemphigoid Foundation (IPPF)</p> <p>1:15PM to 1:55PM Latest News on Gene Therapy and Genome Editing Research</p> <p>Moderator: Phillip Tai, PhD - Assistant Professor, Director, VIDE Program, Horae Gene Therapy Center, Department of Microbiology and Physiological Systems, University of Massachusetts Medical School</p> <p>Speakers: Paul Solari, MD - Global Medical Lead, Hemophilia, BioMarin Pharmaceutical Inc. Laura Trutoiu, PhD - Director of Research, Association for Creatine Deficiencies Kate Zhang, PhD - VP of Biological Development, Editas</p> <p>2:00PM to 2:40PM Streamlining Orphan Drug Development</p> <p>Moderator: Kay Holcombe - C-Path, NORD Board Member</p> <p>Speakers: Julie Breneiser - Executive Director, Gorlin 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</p>	<p>12:30PM to 1:10PM Moving into a Virtual World: Conferences, Fundraisers & Grassroots Advocacy</p> <p>Moderator: Debbie Drell - Director of Membership, National Organization for Rare Disorders (NORD)</p> <p>Speakers: Alice Houk, MPS - Senior Director of Health Professional Programs, Aplastic Anemia & MDS International Foundation Heidi Ross - Director of Policy, National Organization for Rare Disorders (NORD) Vanessa Vogel-Farley - Executive Director, Dup15q Alliance</p> <p>1:15PM to 1:55PM Designing and Implementing a Strategic Plan in the Wake of a Pandemic</p> <p>Moderator: Lisa Sarfaty - Director of Strategic Planning & Project Development, National Organization for Rare Disorders (NORD)</p> <p>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</p> <p>2:00PM to 2:40PM How Patient Organizations Can Encourage Research on their Disease</p> <p>Moderator: Allison Seebald - Senior Research Programs Manager, National Organization for Rare Disorders (NORD)</p> <p>Speakers: Deanna Portero - Management Analyst, Office of Rare Diseases Research (ORDR), National Center for Advancing Translational Sciences (NCATS) Sandra Sermone - President and Executive Director ADNP Kids Research Foundation Theresa Strong, PhD - Director of Research Programs, Foundation for Prader-Wili Research</p>

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

Yann le Cam - Chief Executive Officer, EURORDIS

Charles Holmes - Director, Center for Innovation in Global Health, Professor of Medicine, Georgetown University School of Medicine

Suneet Varma - Global President of Rare Disease, Pfizer

FRIDAY, OCTOBER 9, 2020

8:50AM DAY 1 SUMMARY & DAY 2 PREVIEW

Peter L. Saltonstall, President and Chief Executive Officer, 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, NH

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, Rare Tumor Initiative, CCR, NCI

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 Roscoe, 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 Saltonstall - President & CEO, National Organization for Rare Disorders (NORD)

Speakers:

Patrizia Cavazzoni, MD - Deputy Director for Operations- CDER, US Food & Drug Administration

Peter Marks, MD, PhD- Director, CBER, US Food and Drug Administration

12:15PM to 12:45PM LUNCH BREAK

12:45PM to 2:15PM BREAKOUT SESSIONS

TRACK D	TRACK E	TRACK F
Innovative Approaches to Rare Cancer Therapies	Investment & Business Development	Patient Empowerment
<p>This track will be co-hosted by NORD and the Rare Cancer Coalition</p> <p>12:45PM to 1:30PM Increasing Application of Patient-Reported Data in Rare Oncology</p> <p>Moderator: John Hopper - President, Fibrolamellar Cancer Foundation</p> <p>Speakers: Vishal Bhatnagar, 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</p> <p>1:30PM to 2:15PM Current Trends & Future Directions for Precision Oncology</p> <p>Moderator: Jim Palma - Executive Director, TargetCancer Foundation</p> <p>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 - Group Vice President, Oncology Targeted Therapeutics MD Ashley Ward, MD - Senior Director, Clinical Development, Foundation Medicine Inc.</p>	<p>12:45PM to 1:30PM Impact of COVID-19 on Investment</p> <p>Moderator: David Scheer</p> <p>Speakers: Jason Hafler - Managing Director, Sanofi Ventures Maha Katabi - General Partner, Sofinnova Investments Martin Mackay Francois Nader, MD, MBA - Chairman, Acceleron</p> <p>1:30PM to 2:15PM Advancing Patient Access to Care Through Drug Repurposing</p> <p>Moderator: David Fajgenbaum, MD, MBA, MSc, FCPP - Co-founder and Executive Director of the Castleman Disease Collaborative Network (CDCN), Assistant Professor of Medicine in Translational Medicine & Human Genetics at the University of Pennsylvania, and Associate Director, Patient Impact for the Penn Orphan Disease Center</p> <p>Speakers: Randy Milby - Chief Executive Officer, Hillstream BioPharma Heather Stone - Health Science Policy Analyst, US Food & Drug Administration</p>	<p>12:45PM to 1:30PM Diversity and Inclusion in the Rare Community</p> <p>Moderator: Eric Dube - CEO, Retrophin</p> <p>Speakers: Hannah Armstead - GFPD Diversity Committee Board Member, Sibling Caregiver and Advocate Kirk Campbell, MD - Associate Professor of Medicine; Vice Chair for Diversity and Inclusion, Director, Nephrology Fellowship Program, Icahn School of Medicine at Mount Sinai Linda Goler Blount - President & CEO, Black Women's Health Imperative Kevin Mott - Member, Board of Directors, NephCure Kidney International</p> <p>1:30PM to 2:15PM Unlocking the Power of Healthcare Data</p> <p>Moderator: Oodaye Shukla - Eversana</p> <p>Speakers: Jane Larkindale - Executive Director, Duchenne Regulatory Science Consortium, Executive Director, Rare Diseases Cures Accelerator- Data and Analytics Platform Kira Mann - Chief Executive Officer, Mito Action</p>

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

7:00PM to 9:00PM JOIN US FOR THE 2020 RARE IMPACT AWARDS PRESENTED VIRTUALLY. Register here: rarediseases.org/rare-impact-awards