BREAKTHROUGH SPEAKERS

Scott Gottlieb, M.D.
Commissioner
FDA

Mike Porath
Founder/CEO
The Mighty

REGISTER BY AUGUST 25th AND SAVE UP TO $400!

Advancing the dialogue on emerging issues, policies and strategies impacting the healthcare environment, rare diseases and orphan products

BROUGHT TO YOU BY:

SILVER SPONSORS:
WHY YOU SHOULD ATTEND

Invitation from NORD

As major changes to the nation’s healthcare system are debated, the NORD Summit will provide you with the unique opportunity to hear from the experts and join the conversation on issues of unprecedented importance. We are committed to delivering the latest updates and emerging trends. From ethical guidelines to next-generation treatments to advancing global collaboration, this year’s topics focus on timely issues from subject matter leaders. We encourage you to attend the 2017 Rare Diseases and Orphan Products Breakthrough Summit and look forward to your participation!

Peter L. Saltonstall
President and CEO
NORD

With Special Appreciation for the 2017 Program Advisory Board Members:

NORD would like to extend a thank you to the program advisory members from the FDA who advised on the FDA elements of the program. Their dedication, time and insights contributed to this most meaningful agenda, which continues to inspire new ideas and dialogue to advance education within the rare disease community.

Eleanor Dixon-Terry
Julienne Vaillancourt, R. Ph., M.P.H.
Althea Cuff
Gayatri R. Rao, M.D., J.D.
Jonathan Goldsmith, M.D., FACP
Larry Bauer

The Largest Multi-Stakeholder Gathering in the Rare Disease Community

Previous Participant Profile

Back by popular demand!
RARE-TO-RARE NETWORKING

Maximize your time at the event and build meaningful partnerships by utilizing our appointment-setting software to connect with attendees in advance to schedule on-site meetings.

Our staff and board members who attended were thrilled with the meeting as well as yesterday’s special session for NORD members. We all came home armed with so much new information, many wonderful contacts and a wealth of exciting ideas to put into place. Thank you for creating a wonderful three-day experience for all of us!

-PSC Partners Seeking a Cure
Day 1: Monday, October 16, 2017
7:00  Conference Registration and Continental Breakfast
8:00  NORD’S WELCOME & SUMMIT PREVIEW - Peter L. Saltonstall, President and Chief Executive Officer, NORD
Marshall L. Summar, M.D., Director, Children’s National Rare Disease Institute and Chief, Genetics and Metabolism, Children's National Medical Center; Chairman, Board of Directors, NORD
8:15  COMMUNITY KEYNOTE ADDRESS
9:00  FDA KEYNOTE ADDRESS
9:30  ETHICAL GUIDELINES FOR PATIENT ORGANIZATIONS & INDUSTRY TO COLLABORATE
10:15 Networking & Refreshment Break
11:00 ASSURING PATIENT ACCESS: FUTURE OUTLOOK FOR PATIENT ASSISTANCE PROGRAMS
12:15 LUNCH AND LEARN BREAKOUT ROUNDTABLES
1:30  CHOOSE ONE OF THREE BREAKOUT SESSIONS
2:45 Networking & Refreshment Break
3:30  THE CHALLENGE OF HEALTHCARE COSTS & TREATMENT PRICES
4:30  RIGHT TO TRY, CURRENT POLICY NEWS & NORD’S POLICY PRIORITIES
5:45 Networking Reception

Day 2: Tuesday, October 17, 2017
7:00  Continental Breakfast Opens
7:45  DAY TWO INSIGHTS - Peter L. Saltonstall, President and Chief Executive Officer, NORD
Marshall L. Summar, M.D., Director, Children’s National Rare Disease Institute and Chief, Genetics and Metabolism, Children’s National Medical Center; Chairman, Board of Directors, NORD
8:00  SUSTAINING ORPHAN DRUG DEVELOPMENT AND AVAILABILITY
9:00  CHOOSE ONE OF THREE BREAKOUT SESSIONS
10:00 Networking & Refreshment Break
10:30 CURRENT TOPICS FROM THE FDA
10:30  FDA/CDER Director Janet Woodcock (via video)
10:40  FDA Leadership Perspectives on Orphan Drug Review
11:40  FDA Perspectives on Gene Therapy
12:00 Patient Voice and Engagement with the FDA
Externally-led Patient-Focused Drug Development meetings and lessons learned
How Patients have been Involved
Patient Engagement in CDRH
12:30 Lunch and Learn Breakout Roundtables
2:00 INVESTOR PERSPECTIVE: THE OUTLOOK FOR INVESTMENT IN ORPHAN PRODUCTS
3:00 RARE DISEASE DRUG, BIOLOGIC AND DEVICE DEVELOPMENT: ACHIEVEMENTS AND OPPORTUNITIES
4:00 Closing Remarks
Featured Keynote Speakers

SCOTT GOTTLIEB, M.D., Commissioner, FDA
Dr. Scott Gottlieb was sworn in as the 23rd Commissioner of Food and Drugs on May 10, 2017. Dr. Gottlieb is a physician, medical policy expert, and public health advocate who previously served as the FDA’s Deputy Commissioner for Medical and Scientific Affairs and before that, as a senior advisor to the FDA Commissioner. He also worked on implementation of the Medicare drug benefit as a Senior Adviser to the Administrator of the Centers for Medicare and Medicaid Services, where he supported policy work on quality improvement and the agency’s coverage process, particularly as it related to new medical technologies.

MIKE PORATH, Founder/CEO, The Mighty
Mike Porath got his start in journalism at ABC News, where he was the network’s first overseas digital reporter and was awarded the Society of Professional Journalists’ top honor for his reporting in Kosovo. He has held a variety of writing, editing, producing and executive roles at media companies including ABC News, NBC News, The New York Times and AOL. Mike is also on the board of directors and fundraising chair of The Dup15q Alliance, a non-profit organization that supports people with Dup15q syndrome like his daughter.

Featured FDA Speakers, Panelists & Moderators

RICHARD MOSCICKI, M.D.
(former) Deputy Center Director for Science Operations, CDER, FDA and Chief Medical Officer and Executive Vice President for Science and Regulatory, PhRMA

ROBERT TEMPLE, M.D.
Deputy Center Director for Clinical Science, Center for Drug Evaluation and Research (CDER), FDA

PETER MARKS, M.D., Ph.D.
Director, Center for Biologics Evaluation and Research (CBER), FDA

JEFFREY E. SHUREN, M.D., J.D.
Director, Center for Devices and Radiological Health, CDRH, FDA

JANET WOODCOCK*, M.D.
Director, Center for Drug Evaluation and Research (CDER), FDA

Other Featured Speakers, Panelists & Moderators

CARRIE WOLINETZ, Ph.D.
Associate Director for Science Policy, NIH

ARTHUR CAPLAN, M.D., Ph.D.
Drs. William F. and Virginia Connolly Mitty Chair Director, Division of Medical Ethics, NYU Langone Medical Center

ANNE WILLIS, M.A.
Senior Director, Policy and Advocacy, Cystic Fibrosis Foundation

STEVE USDIN
Washington Editor, BioCentury

PETRA KAUFMANN, M.D.
Director of Clinical Innovation, National Center for Advancing Translational Sciences, NIH

ANNE MCDONALD PRITCHETT, PH.D
Senior Vice President, Policy & Research, PhRMA

*via special recorded video message
Conference Registration and Continental Breakfast

NORD’s Welcome & Summit Preview
Peter L. Saltonstall, President and Chief Executive Officer, NORD
Marshall L. Summar, M.D., Director, Children’s National Rare Disease Institute and Chief, Genetics and Metabolism, Children’s National Medical Center; Chairman, Board of Directors, NORD

Community Keynote Address
Mike Porath, Founder and CEO, The Mighty.com

FDA Keynote Address
Scott Gottlieb, M.D., Commissioner, FDA

Ethical Guidelines for Patient Organizations & Industry to Collaborate
How can patient organizations and pharmaceutical companies work together toward shared goals without compromising ethical standards?
Moderator: Arthur Caplan, M.D., Ph.D., Drs. William F. and Virginia Connolly Matty Chair Director, Division of Medical Ethics, NYU Langone Medical Center
Fireside Chat: Henry R. Moehringer, President and CEO, Alpha-1 Foundation

RARE CANCER ORGANIZATION NETWORKING
Jim Palma, Executive Director, TargetCancer Foundation
John Hopper, President, Fibromyalgia Support Foundation and Founding Co-chair, G1 Cancers Alliance

THE PROMISE OF IMMUNOTHERAPY FOR CANCER PATIENTS
Rob Metz, Senior Vice President, Business Operations & External Affairs, Horizon Pharma
Sachin K. Shah, M.D., Medical Affairs Director, Horizon Pharma

ATTRACTING YOUNG RESEARCHERS TO RARE DISEASES
Anne Pariser, M.D., Deputy Director, Office of Rare Diseases Research, NCATS, NIH

WHAT’S AHEAD FOR PERSONALIZED MEDICINE?
Phillip John (P.J.) Brooks, Ph.D., Program Director, Division of Clinical Innovation, NCATS, NIH

ASSURING PATIENT ACCESS THROUGH PAPs
Janica Stroock, Director, Corporate Responsibility, Pfizer
Catherine Blandfield, M.A., B.S., RN, Vice President of Patient Services, NORD

CDER ENGAGEMENT WITH PATIENT ADVOCACY GROUPS
Scott Wienecke, M.D., Team Lead, Professional Affairs and Stakeholder Engagement, CDER, FDA

INCENTIVES FOR DEVELOPING ORPHAN PRODUCTS
Gayatri Rao, M.D., J.D., Director, Office of Orphan Products Development, FDA

NATURAL HISTORY STUDIES
Gumet Liu, M.D., Ph.D., Office of Orphan Products Development, FDA
Vanessa Boulander, Director of Research Programs, NORD
Allison Seebald, Research Programs Associate, NORD

DEVICES FOR RARE PEDIATRIC DISEASES
Vasum Peiris, M.D., M.P.H., Director Rare Diseases Program, Office of New Drugs, FDA

SAFETY ISSUES IN RARE DISEASE DRUG DEVELOPMENT
Kathryn O’Connell, M.D., Ph.D., Medical Officer, Rare Diseases Program, Office of New Drugs, CDER, FDA

RARE PEDIATRIC DISEASE PRIORITY REVIEW VOUCHERS & DESIGNATIONS
Larry Bauer, Regulatory Scientist, Rare Diseases Program, Office of New Drugs, CDER, FDA

Carlos Espo, M.P.H., Medical Officer, Office of Orphan Product Development, CDER, FDA

NAVIGATING CBER
Diane Maloney, J.D., Associate Director for Policy, CBER, FDA
Julienne Vallaurcourt, R.P.H., M.P.H., CAPT, US PHS CC, Policy Advisor and Rare Disease Liaison, Office of the Center Director, Associate Director for Policy, CBER, FDA

GENE THERAPY POLICIES AT CBER
Ilan Inory, M.D., Deputy Director Division of Clinical Evaluation and Pharmacovigilance/Toxicology, CBER, FDA

CREATIVE PROTOCOL DESIGNS TO ACCELERATE REGULATORY APPROVALS
David Luke, Pharm.D., Senior Director, Clinical and Scientific Affairs, Target Health, Inc.
Warren M. Pearson, Director, Business Development, Target Health, Inc.

ASSESSING THE VALUE OF ORPHAN DRUGS
Derek Jackson, Senior VP, Public Affairs, CSL Behring Biopharmaceuticals
Paul Melmeyer, Director of Federal Policy, NORD

A CONVERSATION WITH JONATHAN GOLDSMITH, COER RARE DISEASE OFFICE
Jonathan C. Goldsmith, M.D., F.A.C.P, Associate Director, Rare Diseases Program, Office of New Drugs, CDER, FDA

BOARD MANAGEMENT FOR PATIENT ORGANIZATIONS
Meegan Carey, Executive Director, PSC Partners
Panama K. Gavin, Chief Operating Officer, NORD

MANAGING VOLUNTEERS IN PATIENT ORGANIZATIONS
Kristen Angel, Associate Director of Membership, NORD
Becky Strong, Outreach Manager, International Pemphigus and Pemphigoid Foundation

SOCIAL MEDIA STRATEGIES TO MAKE YOUR MESSAGE HEARD
Shaza Ahmad, Director, Patient and Physician Strategies, NBC

ROLE OF PATIENT ORGANIZATIONS IN ADVOCATING FOR NEW DRUG APPROVALS
Terry Jo Bichell, Foundation for Angelman Syndrome Therapeutics; A-BOM & Angelman Syndrome Foundation
Debbie Drell, Director of Membership, NORD

EDUCATING MEDICAL PROFESSIONALS ABOUT RARE DISEASES
Amy R.L. Calhoun, M.D., Clinical Assistant Professor, Division of Medical Genetics, Stead Family Department of Pediatrics, University of Iowa Carver College of Medicine and Medical Consultant, Iowa
Newborn Screening Program

Ronald J. DeBellis, Pharm.D., FCP, Chief Scientific Officer, NORD, Professor of Clinical Sciences, Keck Graduate Institute School of Pharmacy

GENOME SEQUENCING & RARE DISEASE DIAGNOSIS
Robert Nussbaum, M.D., Chief Medical officer, Invitae
Jodi M. Vento, M.G.C., L.C.G.C., Manager, Center for Rare Disease Therapy & Brain Care Institute, Genetic Counseling Supervisor, Laboratory Studies, Children’s Hospital of Pittsburgh of UPMC

COMMERCIALIZING GENE THERAPIES
Nancy Pilcher, Director, Business Development, Lias Group

NEWBORN SCREENING AND RARE DISEASES
Stephen Cederbaum, M.D., Director, Medical Genetics Clinic, Jane and Terry Semel Institute for Neuroscience and Human Behavior, Intellectual and Developmental Disabilities Research Center, UCLA

RARE DISEASES 2018
Lisa M. Phillips, Director of Marketing and Community Relations, NORD

STATE RIGHT-TO-TRY LAWS
Tim Boyd, M.P.H., Director of State Policy, NORD

GUERRILA MARKETING TO MASS MARKETING: HOW TO DEVELOP A SUCCESSFUL MARKETING STRATEGY
Silke Dunyoh, Director of Education Programs, NORD

LIVING WITH A RARE DISEASE: TRANSITION OF CARE FROM PEDIATRICS TO ADULT
Swagana Kukanai, Health Policy/Advocacy Intern, NORD

RARE DISEASES FDA/EMA CLUSTER
Tracy Cather, M.P.H., C.C.R.P, C.I.P, Health Science Administrator, Rare Diseases Program, Office of New Drugs, CDER, FDA

FDA PATIENT REPRESENTATIVE PROGRAM
Andrea C. Fura-Helmis, M.P.H., Office of Health & Constituent Affairs, Office of External Affairs, FDA

DIVISION OF PEDIATRIC AND MATERNAL HEALTH
John Alexander, M.D., M.P.H., Deputy Director, Division of Pediatric and Maternal Health, CDER, FDA

RECENT ACTIVITY SURROUNDING SECTION 1115 AND SECTION 1332 WAIVERS
Mélanie Swic, Policy Coordinator, NORD

Networking & Refreshment Break

Networkig Appointment Times Available

Assuring Patient Access: Future Outlook for Patient Assistance Programs
As Patient Assistance Programs come under fire, what can be done to assure patient access to lifesaving treatments for rare diseases?
Moderator: Jayson Soltink, J.D., Partner, Health Policy Strategies, Inc.
Panelists: Erin Willis, Vice President of parent Anne Willis, M.A Senior Director, Policy and Advocacy, Cystic Fibrosis Foundation
Bill Schultz, Partner, Zuckerman Spaeder, LLP
Rob Metz, Senior Vice President, Business Operations & External Affairs, Horizon Pharma

Lunch & Learn Breakout Roundtables
Special booths open and poster presenters available at their posters; Reserve your seat when registering.
1:30 CHOOSE ONE OF THREE BREAKOUT SESSIONS (A – C)

**BREAKOUT A**

**THE PHYSICIAN PERSPECTIVE**
Debra Regier, M.D., Ph.D., Director of Education, Children’s National Rare Disease Institute
Amy R.U.L. Calhoun, M.D., Clinical Assistant Professor, Division of Medical Genetics, Stead Family Department of Pediatrics, University of Iowa Carver College of Medicine and Medical Consultant, Iowa Newborn Screening Program

**ADVANCING DIAGNOSIS**
Michael Patrick Gray, M.P.H., Senior Director of Medical Services, Pulmonary Hypertension Association
Derek Blackway, Senior Manager, Communications & Advocacy, Guthy-Jackson Charitable Foundation

**ADVANCES IN GENETIC TESTING**
Robert Nussbaum, M.D., Chief Medical Officer, Invitae

**Track Moderator:** Philip John (P.J.) Brooks, Ph.D., Program Director, Division of Clinical Innovation, National Center for Advancing Translational Sciences, NIH

**THE PROMISE OF GENE THERAPY**
David Lebwohl, M.D., Senior VP and Global Head for Cell and Gene Therapy, Novartis
Maria Kefalas, Co-Founder, Calliope Joy Foundation

**SHARE FOR RARE—A COLLABORATIVE PLATFORM MODEL**
Petra Kaufmann, M.D., Director of Clinical Innovation, National Center for Advancing Translational Sciences, NIH

**DEVELOPMENT OF NOVEL ENDPOINTS USING MOBILE TECHNOLOGIES**
Theresa Strong, Ph.D., Director of Research Programs, Prader-Willi Research
Les Jordan, VP, Chief Product Evangelist, Target Health, Inc.

**BREAKOUT B**

**NEXT-GENERATION TREATMENTS & ADVANCING CLINICAL TRIALS**

**Track Moderator:** Jennifer Knapp, Executive Director, Adrenal Insufficiency United, Oregon Rare Action Network State Ambassador, NORD

**THE POWER OF STATE-LEVEL LEGISLATION**
Erica Barnes, Founder/President, Chloe’s Fight Rare Disease Foundation, Minnesota Rare Action Network State Ambassador, NORD
Jana Monaco, Patient/Family Advisory Council Chair, Children’s National Medical Center, Children’s National Medical Center, Virginia Rare Action Network State Ambassador, NORD
Vanessa Puopolo, California Rare Action Network State Ambassador, NORD

**EFFECTIVE FAMILY PARTNERSHIPS WITH THE MEDICAL CARE TEAM**
MaryBeth Hollinger, R.N., Director of Education, Support and Advocacy, MitoAction
Colleen Clarke Muraresku, M.S., L.C.G.C., Children’s Hospital of Philadelphia

**BREAKOUT C**

**SUCCESSFUL STRATEGIES FOR PATIENT ORGANIZATIONS**

**Track Moderator:** Steve Usdin, Washington Editor, BioCentury

**THE CHALLENGE OF HEALTHCARE COSTS & TREATMENT PRICES**
A look at the many factors impacting healthcare costs and treatment prices today.

**Moderator:** Steve Usdin, Washington Editor, BioCentury

**Panelists:**
Anne McDonald Pritchett, Ph.D., Senior Vice President, Policy & Research, PhRMA
Bill Martin, Vice President, Pharma Strategy and Account Management, Express Scripts
James Geraghty, Entrepreneur in Residence, Third Rock Ventures

**RIGHT TO TRY, CURRENT POLICY NEWS & NORD’S POLICY PRIORITIES**

**Moderator:** Kate Rawson, Senior Editor, The RPM Report: Regulation, Policy and Market Access and “The Pink Sheet”, Prevision Policy LLC

**Panelists:**
Martha Rinker, J.D., VP Policy, NORD
Kurt R. Karst, Director, Co-Founder and Primary Author of FDA Law Blog, Hyman, Phelps & McNamara, PC
Paul Melmeyer, Director of Federal Policy, NORD
Tim Boyd, M.P.H., Director of State Policy, NORD

**NETWORKING APPOINTMENT TIMES AVAILABLE**

**NETWORKING RECEPTION**
DAY 2  TUESDAY, OCTOBER 17, 2017

7:00  Continental Breakfast Opens

7:45  DAY TWO INSIGHTS

Peter L. Saltonstall, President and Chief Executive Officer, NORD
Marshall L. Summar, M.D., Director, Children’s National Rare Disease Institute and Chief, Genetics and Metabolism, Children’s National Medical Center; Chairman, Board of Directors, NORD

8:00  SUSTAINING ORPHAN DRUG DEVELOPMENT AND AVAILABILITY

Moderator:
Wayne L. Pines, President, Health Care, APCO Worldwide

Panelists:
Peter L. Saltonstall, President and Chief Executive Officer, NORD
Murray Aitken, Senior Vice President and Executive Director, QuintilesIMS
Mike Lanthier, Operations Research Analyst, Office of the Commissioner, FDA
Stephen J. Aselage, CEO, Retrophin, Inc.

9:00  CHOOSE ONE OF THREE BREAKOUT SESSIONS

BREAKOUT SESSION I

THE POWER OF DATA-SHARING

Track Moderator: Lewis M. Fredane, M.D., Clinical Vice President, Bracket Global
CENTERS OF EXCELLENCE
Marshall L. Summar, M.D., Director, Children’s National Rare Disease Institute and Chief, Genetics and Metabolism, Children’s National Medical Center; Chairman, Board of Directors, NORD
PARTICULAR RELEVANCE OF DATA-SHARING TO RARE DISEASES
Carrie Wolinetz, Ph.D., Associate Director for Science Policy, NIH
Sheetal Telang, Senior Director, Therapeutic Strategy, Head of Global Site Identification, Therapeutic Science & Strategy Unit, QuintilesIMS
Salvo La Rosa, Vice President Research and Development, Children’s Tumor Foundation

BREAKOUT SESSION II

DEVELOPING A GLOBAL STRATEGY FOR RARE DISEASES & ORPHAN DRUGS

Track Moderator: Durhane Wong-Rieger, CEO, Canadian Organization for Rare Disorders and Chair, Rare Diseases International
GLOBAL RESEARCH AND DEVELOPMENT AND ACCESS STRATEGIES
Julie Rashkin, Congenital Hyperinsulinism International
Steve Roberds, Ph.D., Chief Scientific Officer, Tuberous Sclerosis Alliance
THE CURRENT SITUATION IN INDIA
Ramaiah Muthyala, M.D., President & CEO, Indian Organization for Rare Diseases
ADDRESSING RARE DISEASES AS A GLOBAL PUBLIC HEALTH CHALLENGE
Bert Bruce, Vice President Global Marketing, Pfizer Rare Disease

BREAKOUT SESSION III

SUCCESSFUL STRATEGIES FOR PATIENT ORGANIZATIONS

Track Moderator: Debbie Drell, Director of Membership, NORD
FUNDRAISING STRATEGIES FOR RARE DISEASE ORGANIZATIONS
Valerie Navy-Daniels, Chief Development Officer, Foundation Fighting Blindness
Danielle Pinders, Officer and Event Coordinator, Foundation for Angelman Syndrome
Kelly Sitkin, Chief Advancement Officer, American Brain Tumor Association
PCORI FUNDING OPPORTUNITIES AND RESOURCES FOR RARE DISEASE ORGANIZATIONS
Gyasi Moscou-Jackson, Ph.D., M.H.S., R.N., Program Officer, Science, Healthcare Delivery and Disparities Research, PCORI

10:00  Networking & Refreshment Break

10:30  CURRENT TOPICS FROM THE FDA

Moderator: Robert Temple, M.D., Deputy Center Director for Clinical Science, CDER, FDA

FDA/CDER Director Janet Woodcock (via video)
Janet Woodcock, M.D., Director, Center for Drug Evaluation and Research, FDA

10:40  FDA Leadership Perspectives on Orphan Drug Review

Billy Dunn, M.D., Director, Division of Neurology Products (DNP), Office of New Drugs, CDER, FDA
Wilson Bryan, M.D., Director, Office of Tissues and Advanced Therapies, CBER, FDA
Amy Barone, M.D., M.S.C.I., Oncology Center of Excellence, FDA

Julie Beitz, M.D., Director Office of Drug Evaluation III, Office of New Drugs, CDER, FDA

11:40  FDA Perspectives on Gene Therapy

Ilan Irony, M.D., Deputy Director Division of Clinical Evaluation and Pharmacology/Toxicology, CBER, FDA

12:00  Patient Voice & Engagement with the FDA

Externally-led Patient-Focused Drug Development Meetings and Lessons Learned
Pujita Vaidya, M.P.H., Office of Strategic Programs, FDA

How Patients Have Been Involved
Andrea Furia-Helms, M.P.H., Health Programs Coordinator, Office of Health and Constituent Affairs, FDA

Patient Engagement in CDRH
Anindita “Annie” Saha, Director, External Expertise and Partnerships, CDRH, FDA
INVESTOR PERSPECTIVE: THE OUTLOOK FOR INVESTMENT IN ORPHAN PRODUCTS

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

Tony Gibney, Managing Partner, Leerink Partners

Kriss Jenner, Founding Partner, Rock Springs Capital

Jonathan Leff, Partners Private Transactions Team, Deerfield Management

Maha Katabi, Partner, Sectoral Asset Management

David Bonita, M.D., Private Equity Partner, Orbimed Advisors, LLC

Bernard Davitian, Vice President and Managing Director, Sanofi-Genzyme Bioventures

RARE DISEASE DRUG, BIOLOGIC AND DEVICE DEVELOPMENT: ACHIEVEMENTS AND OPPORTUNITIES

Moderator: Wayne L. Pines, President, Health Care, APCO Worldwide

Richard Moscicki, M.D., (former) Deputy Center Director for Science Operations, CDER, FDA and Chief Medical Officer and Executive Vice President for Science and Regulatory, PhRMA

Peter Marks, M.D., Ph.D., Director, Center for Biologics Evaluation and Research, FDA

Jeffrey Shuren, M.D., J.D., Director, Center for Devices and Radiological Health, FDA

CLOSING REMARKS

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

MUSIC, MEDICINE AND MOVING FORWARD

A Cocktail Reception Hosted by Children’s National Rare Disease Institute to celebrate the NORD Summit

NORD 2018 DAY 2018

Lisa M. Philips, Director of Marketing and Community Relations, NORD

STATE RIGHT-TO-TRY LAWS

Tim Boyd, M.P.H., Director of State Policy, NORD

GUERRILLA MARKETING TO MASS MARKETING: HOW TO DEVELOP A SUCCESSFUL MARKETING STRATEGY

Sika Dunyoh, Director of Education Programs, NORD

LIVING WITH A RARE DISEASE: TRANSITION OF CARE FROM PEDIATRICS TO ADULT

Swagga Kakari, Health Policy/Advocacy Intern, NORD

RARE DISEASE FDA/EMA CLUSTER

Tracy Cutler, M.P.H., C.C.R.P., C.I.P., Health Science Administrator, Rare Diseases Program, Office of New Drugs, CBER, FDA

FDA PATIENT REPRESENTATIVE PROGRAM

Andrea C. Furia-Helms, M.P.H., Office of Health & Constituent Affairs, Office of External Affairs, FDA

DIVISION OF PEDIATRIC AND MATERNAL HEALTH

John Alexander, M.D., M.P.H., Deputy Director, Division of Pediatric and Maternal Health, CBER, FDA

RECENT ACTIVITY SURROUNDING SECTION 1115 AND SECTION 1332 WAIVERS

Melanie Swick, Policy Coordinator, NORD

EDUCATING MEDICAL PROFESSIONALS ABOUT RARE DISEASES

Amy Ratliff, U.I. Calhoun, M.D., Clinical Assistant Professor, Division of Medical Genetics, Stein Family Department of Pediatrics, University of Iowa Carver College of Medicine and Medical Consultant, Iowa Newborn Screening Program

Ronald J. DeBello, Pharm.D., FCP, Chief Scientific Officer, NORD, Professor of Clinical Sciences, Keck Graduate Institute School of Pharmacy

GENOME SEQUENCING & RARE DISEASE DIAGNOSIS

Jodi M. Vento, M.C.G., J.C.G., Manager, Center for Rare Disease Therapy & Brain Institute, Genetic Counseling; Laboratory Sciences, Children’s Hospital of Pittsburgh of UPMC

COMMERCIALIZING GENE THERAPIES

Matt Letow, Business Development Manager, IC3

NEWBORN SCREENING AND RARE DISEASES

Stephen Cederbaum, M.D., Director, Medical Genetics Clinic, Jane and Terry Semel Institute for Neuroscience and Human Behavior, Intellectual and Developmental Disabilities Research Center, UCLA
Life-Transforming Treatments
An opportunity throughout each of the networking breaks and luncheons to view original research, innovations and advancements as numerous posters are displayed, illustrating key themes:

- INNOVATIVE RESEARCH
- MEDICAL EDUCATION ADVANCEMENT
- PATIENT COMMUNITY BUILDING
- OTHER LIFE-TRANSFORMING TREATMENTS & ADVANCEMENTS

Bringing the Community Closer
1:1 Appointments Elevate Networking Opportunities!
Maximize your time at the event and build meaningful partnerships by utilizing our appointment-setting software to connect with attendees in advance to schedule on-site meetings.

- Customized profiles for you and your organization
- Access attendee lists to find potential interesting clients, contacts or partners
- Software automatically recommends potential matches using keywords
- Easy scheduling for face-to-face meetings at the event; reserved space handled by NORD
- Ability to confirm or deny meeting requests
- Secure, confidential messaging
- Customize your personal agenda for the conference
- Automated personal schedule that can be exported to Outlook, iCal, or printed as a PDF
- Easy access using mobile or desktop
Who attends?
The Summit provides a collaborative environment for researchers from academia, drug and device companies, patient organizations and advocates, policy experts and government organizations responsible for rare disease research and orphan product oversight.

BREAKTHROUGH INDUSTRIES & FOCUS AREAS INCLUDE:
• Patient Services
• Advocacy
• Reimbursement
• Health Policy / Affairs
• Clinical Development
• Regulatory Affairs
• R&D
• Corporate Communications

Sponsorship of NORD’s Rare Diseases and Orphan Products Breakthrough Summit exposes your organization to hundreds of stakeholders, decision makers and influencers committed to the identification, treatment, and the cure of rare diseases. This is through accomplished programs of education, advocacy, research, and patient services.

For more information contact:
Alexa Moore  VP of Development, National Organization for Rare Disorders 617-934-6397 | amoore@rarediseases.org
Derek Gavin  Director of Development, National Organization for Rare Disorders 617.249.7304 | dgavin@rarediseases.org | NORDsummit.org | #NORDSummit

Sponsors
SILVER
SANOFI GENZYME
Retrophin

ADVOCACY
HORIZON PHARMA

BRONZE
ALEXION
CSL Behring

Additional sponsors
BRIDGES
Vertex

Media Partners
AJMC
Medscape

©2017 The National Organization for Rare Disorders (NORD®). All rights reserved.
NORD® is a registered trademark of the National Organization for Rare Disorders. Rare Diseases & Orphan Products Breakthrough Summit is a trademark of NORD. NORD is a registered 501(c)(3) charity organization. Please note that NORD provides this information for the benefit of the rare disease community. NORD is not a medical provider or health care facility and thus can neither diagnose any disease or disorder nor endorse or recommend any specific medical treatments. Patients must rely on the personal and individualized medical advice of their qualified health care professionals before seeking any information related to their particular diagnosis, cure or treatment of a condition or disorder.
## PRICING

### NORD Patient Organization Members
- **Advantage Rate:** $349
- **Standard Rate:** $649
- **Register Onsite:** $749

### Non-Profits/Patients/Academics
- **Advantage Rate:** $399
- **Standard Rate:** $699
- **Register Onsite:** $799

### NORD Corporate Council Members
- **Advantage Rate:** $1,699
- **Standard Rate:** $2,099
- **Register Onsite:** $2,199

### Government
- **Advantage Rate:** $499
- **Standard Rate:** $499
- **Register Onsite:** $599

### Students, Residents and Fellows
- **Advantage Rate:** $399
- **Standard Rate:** $699
- **Register Onsite:** $799

### Physicians, Pharmacists and Nurses (2-day Summit)*
- **Advantage Rate:** $499
- **Standard Rate:** $699
- **Register Onsite:** $799

### Industry (Pharma, Service Providers, Co-Pay Foundations)
- **Advantage Rate:** $2,199
- **Standard Rate:** $2,499
- **Register Onsite:** $2,599

---

*This price is offered to individuals attending the CME program offered by NORD during the conference. Please visit rarediseases.org/CME for more information.

For additional pricing information, please contact NORDREG@MeetingExpectations.com.

**Registration fee includes continental breakfast, lunch, refreshments, wine and cheese reception and conference materials.**

Credit Card (Visa, MC, AMEX, Discover) or checks accepted. Please make checks (in U.S. funds drawn on a U.S. bank) payable to:

National Organization for Rare Disorders, Inc.
Department 5430
P.O. Box 4110, Woburn, MA 01888-4110

**PLEASE NOTE:** All advertised discounts are taken from the full, standard rate.

---

### VENUE
**Marriott Wardman Park**
2660 Woodley Rd NW,
Washington, DC 20008

**Phone Reservations:** 1-877-212-5752 (mention NORD)


### SUBSTITUTION AND CANCELLATION
Any cancellations received in writing on or before 14 days prior to the start date of the event will be refunded, less a $399 administrative charge. No refunds will be made after October 1, 2017. Your registration may be transferred to another member of your organization up to 24 hours in advance of the summit.

In case of a conference cancellation beyond our control*, you will receive a refund for your conference registration fee only. NORD reserves the right to alter this program without prior notice. **Please Note:** Speakers and agenda are subject to change. In the event of a speaker cancellation, every effort to find a suitable replacement will be made.

*Events beyond our control include: severe weather conditions, natural and man-made disasters and any other similar events.

### ACCOMMODATIONS
**To receive NORD’s discounted hotel rate:**
**Online:** https://aws.passkey.com/go/NORD17

**Phone reservations:** 1-877-212-5752 (mention NORD)

**Book Now!** Marriott Washington Wardman Park is accepting reservations on a space and rate availability basis. Rooms are limited so please book early. All travel arrangements are subject to availability. Please make sure you book your room by September 22, 2017.

---

### Scholarship Applications
NORD is pleased to provide patient organizations with scholarships to help with the cost of attending the Summit. Scholarships are awarded on a first-come, first-served, as-needed basis with priority given to NORD patient organization members and students.

**To apply, please go to www.nordsummit.org and download the application.**

### Poster Submissions
Academics, researchers, industry, government agencies, health care professionals, patient organizations and any other interested parties that have conducted rare disease or orphan product research studies or public health projects are invited to submit a poster abstract to the NORD Summit. The overall theme of the poster session is “Life-Transforming Treatments.” Suggested specific topic areas within that over-arching theme that the planning committee would like to address include: Innovative Research, Medical Education Advancement, Patient Community Building, and other topics. For more information please contact: Katherine Morgan, kmorgan@meetingexpectations.com
National Organization for Rare Disorders
55 Kenosia Avenue
Danbury, CT 06810