NORD® Rare Summit

OCTOBER 16-17, 2017
MARRIOTT WARDMAN PARK
WASHINGTON, D.C.

RARE DISEASES & ORPHAN PRODUCTS BREAKTHROUGH SUMMIT®

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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

@NationalOrganizationforRareDisorders | @RareDiseases | National Organization for Rare Disorders

BROUGHT TO YOU BY:

NORD® National Organization for Rare Disorders

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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 commited 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

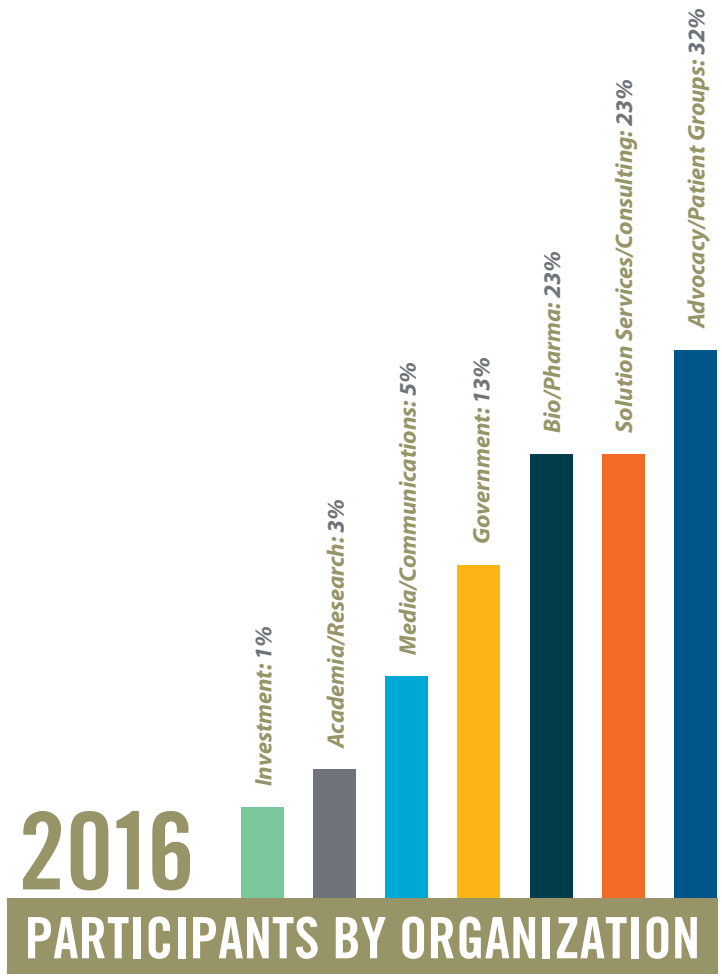
With Special Appreciation for the 2017 Program Advisory Board Members:

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



EXPANSIVE **POSTER SESSION**
HIGHLIGHTING THE BRIGHTEST MINDS IN THE INDUSTRY

OVER 60 SPEAKERS

GROUNDBREAKING **KEYNOTES**

INSIGHTFUL
PATIENT PARTICIPATION

PRE-EVENT **MEETING SCHEDULER**
ENHANCING CONNECTIONS & PARTNERSHIP BUILDING

MULTIPLE **FOCUSED TRACKS**
WITH SOMETHING FOR EVERYONE

ROUNDTABLE **DISCUSSIONS**
KEY TOPICS OF INTEREST

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

AGENDA AT A GLANCE

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

PROMOTING EARLIER DIAGNOSIS	NEXT-GENERATION TREATMENTS & ADVANCING CLINICAL TRIALS	SUCCESSFUL STRATEGIES FOR PATIENT ORGANIZATIONS

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

THE POWER OF DATA-SHARING	DEVELOPING A GLOBAL STRATEGY FOR RARE DISEASES & ORPHAN DRUGS	SUCCESSFUL STRATEGIES FOR PATIENT ORGANIZATIONS

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

BREAKTHROUGH SPEAKERS

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 A. MOSCICKI, M.D.

Deputy Center Director for Science Operations, Center for Drug Evaluation and Research (CDER), FDA

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

MICHELLE DROZD, Sc.M.

Deputy Vice President, Policy and Research, PhRMA

*via special recorded video message

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**
Mike Porath, Founder and CEO, **TheMighty.com**

9:00 **FDA KEYNOTE ADDRESS**
Scott Gottlieb, M.D., Commissioner, **FDA**

9:30 **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 Mitty Chair Director, Division of Medical Ethics, **NYU Langone Medical Center**

Fireside Chat:
Henry R. Moehring, President and CEO, **Alpha-1 Foundation**
Heather Golding, Vice President Legal and Compliance, **Sobi North America**
Gina Parziale, C.F.R.E., Executive Director, **Alport Syndrome Foundation**
Mathieu Boudes, Operations & Projects Manager, **EURORDIS**

10:15 Networking & Refreshment Break
NETWORKING APPOINTMENT TIMES AVAILABLE

11:00 **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 Slotnik, J.D., Partner, **Health Policy Strategies, Inc.**

Panelists:
Erin Tite, Parent of patient
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**

12:15 LUNCH & LEARN BREAKOUT ROUNDTABLES

Sponsor booths open and poster presenters available at their posters; Reserve your seat when registering.

1 RARE CANCER ORGANIZATION NETWORKING
Jim Palma, Executive Director, **TargetCancer Foundation**

2 THE PROMISE OF IMMUNOTHERAPY FOR CANCER PATIENTS

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

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

5 ASSURING PATIENT ACCESS THROUGH PAPs
Jenica Stroock, Director, Corporate Responsibility, **Pfizer**

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

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

8 NATURAL HISTORY STUDIES
Gumei Liu, M.D., Ph.D., Office of Orphan Products Development, **FDA**
Vanessa Boulanger, Director of Research Programs, **NORD**
Allison Seebald, Research Programs Associate, **NORD**

9 DEVICES FOR RARE PEDIATRIC DISEASES
Vasum Peiris, M.D., M.P.H., Chief Medical Officer of Pediatrics and Special Populations, Center for Devices and Radiological Health, **FDA**

10 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**

11 RARE PEDIATRIC DISEASE PRIORITY REVIEW VOUCHERS & DESIGNATIONS
Larry Bauer, Regulatory Scientist, Rare Diseases Program, Office of New Drugs, CDER, **FDA**
Althea Cuff, Senior Regulatory Health Project Manger, Rare Diseases Program, Office of New Drugs, CDER, **FDA**
Carla Epps, M.D., M.P.H., Medical Officer, CDER, **FDA**

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

13 GENE THERAPY POLICIES AT CBER
Ilan Irony, M.D., Deputy Director Division of Clinical Evaluation and Pharmacology/ Toxicology, CBER, **FDA**

14 CREATIVE PROTOCOL DESIGNS TO ACCELERATE REGULATORY APPROVALS
Jules T. Mitchel, MBA, Ph.D., President, **Target Health Inc.**

15 ASSESSING THE VALUE OF ORPHAN DRUGS
Dennis Jackman, Senior VP, Public Affairs, **CSL Behring Biopharmaceuticals**

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

17 BOARD MANAGEMENT FOR PATIENT ORGANIZATIONS
Meegan Carey, Executive Director, **PSC Partners**

18 MANAGING VOLUNTEERS IN PATIENT ORGANIZATIONS
Kristen Angell, Associate Director of Membership, **NORD**

19 SOCIAL MEDIA STRATEGIES TO MAKE YOUR MESSAGE HEARD
Shazia Ahmad, Director, Patient and Physician Services, **UBC**

20 ROLE OF PATIENT ORGANIZATIONS IN ADVOCATING FOR NEW DRUG APPROVALS
Terry Jo Bichell, **Foundation for Angelman Syndrome Therapeutics; A-BOM & Angelman Syndrome Foundation**

21 EDUCATING MEDICAL PROFESSIONALS ABOUT RARE DISEASES
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**
Ronald J. DeBellis, Pharm.D., FCCP, Chief Scientific Officer, **NORD**, Professor of Clinical Sciences, **Keck Graduate Institute School of Pharmacy**

22 GENOME SEQUENCING & RARE DISEASE DIAGNOSIS

23 COMMERCIALIZING GENE THERAPIES

1:30 CHOOSE ONE OF THREE BREAKOUT SESSIONS (A – C)

BREAKOUT A

PROMOTING EARLIER DIAGNOSIS

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 Medicin**e 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**

BREAKOUT B

NEXT-GENERATION TREATMENTS & ADVANCING CLINICAL TRIALS

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 C

SUCCESSFUL STRATEGIES FOR PATIENT ORGANIZATIONS

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., C.G.C., **Children's Hospital of Philadelphia**

2:45 Networking & Refreshment Break

NETWORKING APPOINTMENT TIMES AVAILABLE

3:30 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**

4:30 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**

5:45 NETWORKING RECEPTION

DAY 2 TUESDAY, OCTOBER 17, 2017

7:00 Continental Breakfast Opens
NETWORKING APPOINTMENT TIMES AVAILABLE

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 Raskin, **Congential 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
NETWORKING APPOINTMENT TIMES AVAILABLE

10:30 CURRENT TOPICS FROM THE FDA
Moderator: Robert Temple, M.D., Deputy Center Director for Clinical Science, CDER, **FDA**

10:30 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., 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**

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Jim Palma, Executive Director, **TargetCancer Foundation**

2 THE PROMISE OF IMMUNOTHERAPY FOR CANCER PATIENTS

3 ATTRACTING YOUNG RESEARCHERS TO RARE DISEASES
Rashmi Gopal-Srivastava, Ph.D., Director, Extramural Research Program, ORDR, NCATS, **NIH**

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

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8 NATURAL HISTORY STUDIES
Gumei Liu, M.D., Ph.D., Office of Orphan Products Development, **FDA**

Vanessa Boulanger, Director of Research Programs**, NORD**

Allison Seebald, Research Programs Associate, **NORD**

9 DEVICES FOR RARE PEDIATRIC DISEASES
Vasum Peiris, M.D., M.P.H., Chief Medical Officer of Pediatrics and Special Populations, Center for Devices and Radiological Health, **FDA**

10 NAVIGATING EXPANDED ACCESS
Lucas Kempf, M.D., Medical Officer, Rare Diseases Program, Office of New Drugs, CDER, **FDA**

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

Althea Cuff, Senior Regulatory Health Project Manager, Rare Diseases Program, office of New Drugs, CDER, **FDA**

Carla Epps, M.D., M.P.H., Medical Officer, Office of Orphan Products Development, CDER, **FDA**

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Diane Maloney, J.D., Associate Director for Policy, CBER, **FDA**

Julienne Vaillancourt, R.Ph., M.P.H., CAPT, US PHS CC, Policy Advisor and Rare Disease Liaison, Office of the Center Director, Associate Director for Policy, CBER, **FDA**

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

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Jules T. Mitchel, MBA, Ph.D., President, **Target Health Inc.**

15 ASSESSING THE VALUE OF ORPHAN DRUGS
Dennis Jackman, Senior Vice President, Public Affairs, **CSL Behring Biopharmaceuticals**

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

17 ROLE OF CRITICAL PATH INNOVATION MEETINGS (CPIM) IN RARE DISEASE DRUG DEVELOPMENT
Chekesh S. Clingman, Ph.D., M.B.A., US Public Health Service, Associate Director for Strategic Partnerships, CDER, Office of Translational Sciences, **FDA**

18 BOARD MANAGEMENT FOR PATIENT ORGANIZATIONS
Meegan Carey, Executive Director, **PSC Partners**

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Kristen Angell, Associate Director of Membership, **NORD**

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Terry Jo Bichell, **Foundation for Angelman Syndrome Therapeutics; A-BOM & Angelman Syndrome Foundation**

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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**

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

23 GENOME SEQUENCING & RARE DISEASE DIAGNOSIS

24 COMMERCIALIZING GENE THERAPIES

2:00 INVESTOR PERSPECTIVE: THE OUTLOOK FOR INVESTMENT IN ORPHAN PRODUCTS

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

Tony Gibney, Managing Partner, **Leerink Partners**

Kris Jenner, Founding Partner, **Rock Springs Captial**

Jonathan Leff, Partners Private Transactions Team, **Deerfield Management**

Maha Katabi, Partner, **Sectoral Asset Management**

3:00 RARE DISEASE DRUG, BIOLOGIC AND DEVICE DEVELOPMENT: ACHIEVEMENTS AND OPPORTUNITIES

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

Richard Moscicki, M.D., Deputy Center Director for Science Operations, CDER, **FDA**

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**

4:00 CLOSING REMARKS
Peter L. Saltonstall, President and Chief Executive Officer, **NORD**

6:00 MUSIC, MEDICINE AND MOVING FORWARD
A Cocktail Reception Hosted by Children's National Rare Disease Institute to celebrate the NORD Summit

Children's National
Rare Disease Institute

POSTER HIGHLIGHTS

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**

RARE TO RARE NETWORKING

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
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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
- Clinical Development
- R&D
- Reimbursement
- Health Policy / Affairs
- Regulatory Affairs
- 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

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REGISTER TODAY!

	Advantage Rate Expired 8/25/17	Standard Rate Register by 10/15/17	Register Onsite
NORD Patient Organization Members	$349	$649	$749
Non-Profits/Patients/Academics	$399	$699	$799
NORD Corporate Council Members	$1,699	$2,099	$2,199
Government	$499	$499	$599
Students, Residents and Fellows	$399	$699	$799
Physicians, Pharmacists and Nurses (2-day Summit)*	$499	$699	$799
Industry (Pharma, Service Providers, Co-Pay Foundations)	$2,199	$2,499	$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.

NORD® Rare Summit

Register at NORDSummit.org.

VENUE

Marriott Wardman Park
2660 Woodley Rd NW,
Washington, DC 20008
Phone Reservations: 1-877-212-5752 (mention NORD)
Please book your room by September 22, 2017.

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

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

National Organization for Rare Disorders
55 Kenosia Avenue
Danbury, CT 06810

NORD Rare Summit

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BREAKTHROUGH SPEAKERS

Scott Gottlieb, M.D.
Commissioner
FDA

Mike Porath
Founder/CEO
The Mighty

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