Rare Diseases & Orphan Products

BREAKTHROUGH SUMMIT 2016

NORD® Rare Summit

OCTOBER 17-18, 2016
HYATT REGENCY CRYSTAL CITY
ARLINGTON, VA

Where Today's Critical Issues and Conversations Lead to Tomorrow's Cutting-Edge Ideas & Advancements

FDA Speakers Spark Progress for Rare Diseases!

Keynote Speaker

ROBERT CALIFF, MD,
Commissioner,
FDA

PETER MARKS, MD, PhD,
Director, Center for Biologics Evaluation and Research,
FDA

JANET WOODCOCK, MD,
Director, Center for Drug Evaluation and Research,
FDA

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REGISTER BY AUGUST 26th AND SAVE UP TO $400!

WHY YOU SHOULD ATTEND

Invitation from NORD

As a partner in the fight against rare diseases, I hope you will attend this year's Rare Diseases and Orphan Products Breakthrough Summit. NORD's Summit is the only annual event that brings together the entire rare disease community in one venue to discuss new opportunities for collaboration to advance treatments and therapies.

The following 2016 agenda will help you map out the ways to engage with timely content, people, and activities during the conference. In addition to our esteemed faculty, we are excited to announce two new features this year: our enhanced appointment setting software and Lunch & Learn roundtable discussions. We know how important it is to connect with other stakeholders to build partnerships and collaborations, so we hope to see you there.

Peter L. Saltonstall,
President and CEO,
NORD

With Special Appreciation for the 2016 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.

Larry Bauer, Regulatory Scientist, CDER, FDA

Katharine Chowdhury, OOPD, FDA

Althea Cuff, Science Policy Analyst, CDER, FDA

Jonathan Goldsmith, MD, FACP, Associate Director Rare Diseases Program/OND, CDER, FDA

Lucas Kempf, MD, Medical Officer, Rare Diseases Program, CDER, FDA

Kathryn O'Connell, MD, PhD, Rare Disease Program, Office of New Drugs, CDER, FDA

Gayatri Rao, MD, JD, Director, OOPD, FDA

Julienne Vaillancourt, RPh, MPH, Captain, U.S. Public Health Service, Regulatory Reviewer, CBER, FDA

Charting the Course in Rare Diseases

CONNECT

Network with over 500 patients, advocates, legislative and regulatory leaders, industry executives and researchers.

EXPLORE

Immerse yourself in rare disease learning and action-oriented strategy by choosing from over 6 tracks and 30 sessions.

ADVANCE

Equip yourself with breaking updates and innovative ideas that move the dial forward in rare disease research and treatments.

INSPIRE

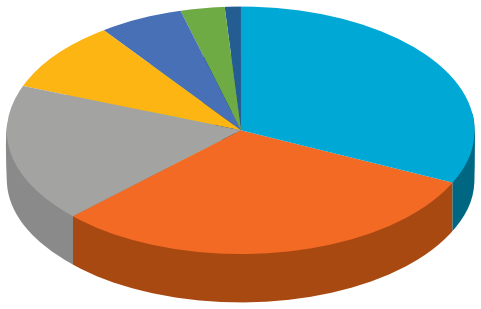
Join seasoned and emerging visionaries as we trailblaze the road ahead in diagnosis, treatment and access for patients.

TRANSFORM

Together, we can create meaningful change in the lives of rare disease patients.

The **Largest Multi-Stakeholder** Gathering in the Rare Disease Community

Previous Participant Profile



New and Exciting for 2016!

RARE TO RARE NETWORKING

Maximize your time throughout the event and utilize appointment-setting software to facilitate 1:1 meetings and build meaningful connections.

"The NORD Summit was the singular opportunity that I have had to interact and network with others from patient orgs dealing with rare diseases. I learned as much in the networking breaks as I learned in the sessions. I leave with new knowledge I don't think I could have gained anywhere else." — Director of Research, **Hemophilia Federation of America**

AGENDA AT A GLANCE

DAY ONE MONDAY, OCTOBER 17, 2016

7:00	Conference Registration and Continental Breakfast
8:00	**NORD's Welcome & Opening Remarks** — Peter Saltonstall, President and CEO, **NORD**
8:15	**PATIENT KEYNOTE ADDRESS**
9:00	**Exploring Frontiers — Telemedicine and Rare Diseases**
9:30	**KEYNOTE ADDRESS**
10:15	Networking and Refreshment Break SPONSORED BY: VERTEX
11:00	**Potential Advances through Genetic Innovation**
12:15	**LUNCH AND LEARN BREAKOUT ROUNDTABLES** SPONSORED BY: Pfizer
1:30	**CHOOSE BETWEEN THREE BREAKOUT SESSIONS (A-C)**

A The Crucial Role of Data in Advancing Diagnosis and Clinical Drug Development

B Collaborations Across Borders — Addressing Rare Diseases as a Global Public Health Challenge

C Focus on Pediatric Diseases – Advancing Research and Treatments

2:45	Networking and Refreshment Break
3:30	**The Challenge of Access and Reimbursement — Rising Concerns Regarding Affordability, Innovation and Quality of Care**
4:30	**The Landscape for Investment**
5:30	Close of Day One / Networking Cocktail Reception Commences SPONSORED BY: DOHMEN LIFE SCIENCE SERVICES, fff enterprises, ALEXION

DAY TWO TUESDAY, OCTOBER 18, 2016

7:30	Continental Breakfast Opens
8:00	**Day Two Insights** — Peter Saltonstall, President and CEO, **NORD**
8:10	**Update from NORD Board of Directors** — Marshall L. Summar, MD, Division Chief, Genetics and Metabolism, **Children's National Health System**
8:20	**NATIONAL ELECTION IMPLICATIONS KEYNOTE ADDRESS**
9:00	**Driving Progress Through Policy**
10:00	Networking and Refreshment Break SPONSORED BY: Pfizer
10:45	**CHOOSE BETWEEN THREE TRACKS (I-III)**

I Trending Topics from FDA

II Strategies to Address Patient Challenges

III Breaking Down Barriers to Access

1:00	Networking Luncheon
2:15	**NORD and Trio Health Partnership to Improve Quality of Care and Outcomes**
3:00	**Predicting the Pipeline — Orphan Product Development and Progress in 2017**
3:45	**The FDA Commitment to Rare Diseases**
4:45	Closing Remarks — Peter Saltonstall, President and CEO, **NORD**
5:00	Close of Conference

SUMMIT SPEAKERS


David Altarac, MD, MPA,
Head of Global Regulatory Affairs,
Shire


Jim Anderson, Senior Director, Business Development, Head, Reimbursement, Access & Distribution Solutions, Emerging Biopharma,
QuintilesIMS


David Arons, JD,
Chief Executive Officer,
National Brain Tumor Society, Member, Blue Ribbon Panel,
National Moonshot Cancer Initiative


Larry Bauer,
Regulatory Scientist, CDER,
FDA


Michael Binks, MD,
Vice President, Clinical Research, Rare Disease Research Unit,
Pfizer Inc


Catherine Blansfield, MA, BS, RN,
Vice President of Access and Outcomes,
NORD


Tim Boyd,
Associate Director of State Policy,
NORD


Philip J. Brooks, PhD,
Program Director, Division of Clinical Innovation, NCATS,
NIH


Loreen M. Brown, MSW,
Senior Vice President, Product Strategy and Commercialization Excellence,
Lash Group, a part of AmerisourceBergen


Robert Califf, MD,
Commissioner,
FDA


Brent Clough,
CEO,
Trio Health


Mark Dant,
President and CEO,
National MPS Society


Peter Dehnel, Medical Director,
Blue Cross and Blue Shield of Minnesota


Mary Dunkle,
Vice President Educational Initiatives,
NORD


Caitlin Dwyer, MSW, LSW,
Social Worker,
Division of Nephrology,
Children's Hospital of Philadelphia


David Flannery, MD, FACMG, FAAP,
Medical Director,
American College of Medical Genetics


Kelly East, MS, CGC, Certified Genetic Counselor, Clinical Applications Lead,
HudsonAlpha Institute for Biotechnology


Karen L. Erickson, Associate Executive Director, Community Engagement,
The Alpha-1 Foundation


Eric Floyd, MS, MBA, PhD,
Chief Scientific Officer,
Dohmen Life Science Services


Pamela K. Gavin,
Chief Operating Officer,
NORD


Jim Geraghty,
Entrepreneur-In-Residence,
Third Rock Ventures


Jonathan Goldsmith, MD, FACP,
Associate Director Rare Diseases Program/OND, CDER,
FDA


Steven Grossman,
President,
HPS Group, LLC


Alberto Gutierrez,
Director of the Office of In Vitro Diagnostics, CDRH,
FDA


Amanda L. Hayward, PhD,
Independent


Nicole Hebbert, Vice President, Patient Access and Engagement,
UBC: An Express Scripts Company


John Jenkins, MD,
Director,
Office of New Drugs,
FDA


Valerie Jensen, RPh,
Associate Director of the Drug Shortage Staff, CDER,
FDA


Jane Juusola, PhD, FACMG
Director, Whole Exome Sequencing Program,
GeneDx


Lucas Kempf, MD, Medical Officer, Rare Diseases Program, CDER,
FDA


Joan Keutzer, PhD, Vice President and Head of Integrated Solutions for Rare Diseases, **Sanofi Genzyme**


Richard Klein,
Director, Patient Liaison Program,
Office of Health and Constituent Affairs, OHCA, **FDA**


Carrie Koenig,
Program Coordinator,
Hemophilia Federation of America


Peter Kolchinsky,
Managing DIrector & Portfolio Manager,
RA Capital Management


Mike Lanthier,
Operation Research Analyst,
Office of Planning, Office of the Commissioner, **FDA**


John Leonard, MD,
Chief Medical Officer,
Intellia Therapeutics


Ted W. Love, MD,
Chief Executive Officer,
Global Blood Therapeutics


Sue Lim, MD,
Team Lead for Therapeutic Biologics, OND Therapeutic and Biologics Team, CDER, **FDA**


Peter Marks, MD, PhD,
Director, CBER,
FDA


Jeffrey Marrazzo,
Co-Founder and Chief Executive Officer,
Spark Therapeutics

Paul Melmeyer,
Associate Director of Public Policy,
NORD

Matthew Might, Associate Professor, School of Computing, **University of Utah;** Associate Professor, Visiting, Biomedical Informatics, **Harvard Medical School;** Strategist, Executive Office of the President,
The White House

Jules T. Mitchel,
President and Co-Founder,
Target Health Inc.

Richard Moscicki, MD,
Deputy Center Director for Science Operations,
FDA

Haja El Mubarak,
Division of Microbiology Devices, CDRH,
FDA

Jan Nielsen,
Division President, Sonexus™ Access & Patient Support,
Cardinal Health

Michael A. Pacanowski, MPH, PharmD,
Associate Director for Genomics and Targeted Therapy, CDER,
FDA

Richard Peters, MD, PhD, Senior Vice President, Head, Global Rare Diseases Franchise and Head, JAPAC Business Unit, **Sanofi Genzyme**

Lisa Phelps, MPH,
Director of Marketing & Community Relations,
NORD

Gayatri Rao, MD, JD,
Director, OOPD,
FDA

Kate Rawson,
Senior Editor,
Prevision Policy

Lori Reilly,
Executive Vice President, Policy and Research,
PhRMA

Martha Rinker,
Vice President of Public Policy,
NORD

Durhane Wong-Rieger, President,
Canadian Organization for Rare Disorders

Peter L. Saltonstall,
President and CEO,
NORD

Jayson Slotnik, JD,
Principal and Founding Member,
Health Policy Strategies, Inc.

Noel Southall,
Informatics, NCATS,
NIH

Marshall L. Summar,
MD, Division Chief, Genetics and Metabolism, **Children's National Health System**

Gina Szajnuk,
Co-founder & Executive Director,
Rare and Undiagnosed Network (RUN)

Michelle Tarver, MD, PhD,
CDRH,
FDA

Lisa Terrizzi, JD,
General Counsel,
NORD

Charles A. Thompson,
Global Lead, Pfizer Pediatric Center of Excellence,
Pfizer Inc

Tiina K. Urv, PhD,
Program Director, Division of Clinical Innovation, NCATS,
NIH

Pujita Vaidya, MPH, Acting Director, Decision Support and Analysis Team, Office of Strategic Programs, CDER,
FDA

Paul Weckstein, JD,
Co-Director,
The Center for Law and Education

John J. Whyte, MD, MPH, Director of Professional Affairs and Stakeholder Engagement, CDER, **FDA**

Celia Witten, MD, PhD,
Deputy Director, CBER,
FDA

Janet Woodcock, MD,
Director, CDER,
FDA

Nora Yang, PhD, MBA, Director, Portfolio Management and Strategic Operations, Therapeutics for Rare and Neglected Diseases (TRND), NCATS, **NIH**

Lynne P. Yao, MD,
Acting Director, Division of Pediatric and Maternal Health/ODE IV/CDER,
FDA

DAY ONE MONDAY, OCTOBER 17, 2016

7:00 Conference Registration and Continental Breakfast

Networking Appointment Times Available

8:00 NORD's Welcome and Opening Remarks

Peter L. Saltonstall, President and CEO, **NORD**

8:15 **PATIENT KEYNOTE ADDRESS**

Kristen Gray, Co-Founder, **Charlotte and Gwenyth Gray Foundation**

9:00 **Exploring Frontiers — Telemedicine and Rare Diseases**

David Flannery, MD, FACMG, FAAP, Medical Director, **American College of Medical Genetics**

9:30 **KEYNOTE ADDRESS**

Robert Califf, MD, Commissioner, **FDA**

10:15 Networking and Refreshment Break Sponsored by: VERTEX

Networking Appointment Times Available

11:00 **Potential Advances through Genetic Innovation**

Today's genetic capabilities and developing technologies hold great promise for the rare disease community. With genome and exome sequencing, new applications of gene therapy and the promise of gene editing, the potential for advances through genetic innovation is growing.

- Advance diagnosis for rare disease patients through genome/exome sequencing
- Leverage gene therapy to find new treatment options for rare disease patients
- Understand the promise of targeted genome editing with CRISPR/Cas9
- Regulatory considerations for gene therapy

Moderator:

Nora Yang, PhD, MBA, Director, Portfolio Management and Strategic Operations, Therapeutics for Rare and Neglected Diseases (TRND), NCATS, **NIH**

Panelists:

Jane Juusola, PhD, FACMG, Director, Whole Exome Sequencing Program, **GeneDx**

John Leonard, MD, Chief Medical Officer, **Intellia Therapeutics**

Jeffrey Marrazzo, Co-Founder and Chief Executive Officer, **Spark Therapeutics**

Celia Witten, MD, PhD, Deputy Director, CBER, **FDA**

12:15 **LUNCH AND LEARN BREAKOUT ROUNDTABLES*** SPONSORED BY: Pfizer

**Reserve your table seat when registering*

1. **Use of EHRs for Rare Disease Patients**
   Jeremy Morgan, Initiated Research and CoRDS Program Director, **Sanford Research**
   Angela VanVeldhuizen, CoRDS Project Manager, **Sanford Research**
   Brent Clough, CEO, **Trio Health**
2. **The Role of the Patient Organizations in Advocating for New Drug Approvals**
   Mladen Bozic, Head, Global Regulatory Policy and Intelligence, **Shire**
   Kate Rawson, Senior Editor, **Prevision Policy**
   Wayne Pines, President Healthcare, **APCO Worldwide**
3. **Potential Challenges of Switching when Biosimilars are Approved**
   Larry LaMotte, Vice President of Public Policy, **Immune Deficiency Foundation**
4. **Optimal Use of Social Media by Patient Organizations**
   Kelly Williams, Vice President, Communications and Marketing, **Pulmonary Hypertension Association**
   Jennifer Huron, Associate Director, Communications & Marketing, **NORD**
   Lisa Phelps, MPH, Director of Marketing & Community Relations, **NORD**
5. **Navigating and Accessing Hubs for Rare Disease Patients**
   Nancy Pilcher, RN, Director, Business Development, **Lash Group, part of AmerisourceBergen**
   Barbara Tucciarone, Operations Specialist, **NORD**
   Catherine Blansfield, MA, BS, RN, Vice President of Access and Outcomes, **NORD**
   Rob Osborne, Senior Director of Business Development, **Accredo**
6. **Changes in Compassionate Use Programs for Drugs**
   John J. Whyte, MD, MPH, Director of Professional Affairs and Stakeholder Engagement, CDER, **FDA**
   Paul Melmeyer, Associate Director of Public Policy, **NORD**
7. **Optimizing Communications among Patient Organizations and Industry**
   Amit Rakhit, MD, MBA, Chief Medical & Portfolio Officer, **Ovid Therapeutics**
   Jamie Ring, Head Patient Advocacy, **Spark Therapeutics**
   Ellen Salkeld, Board Member, **Aplastic Anemia & MDS International Foundation**
8. **Facilitating Clinical Trial Recruitment**
   Jessica Perry, Associate Director, Patient Recruitment Programs, **QuintilesIMS**
9. **Partnering for Progress (Centers of Excellence, Consortia, etc)**
   Charles A. Thompson, Global Lead, Pfizer Pediatric Center of Excellence, **Pfizer Inc**
   Ron DeBellis, Chief Scientific Officer, **NORD**
10. **The Future of Gene Therapy for Rare Diseases**
    Celia Witten, MD, PhD, Deputy Director, CBER, **FDA**
    Frank Sasinowski, Director, **Hyman Phelps & McNamara**
11. **How Drug Shortages Affect Rare Disease Patients**
    Valerie Jensen, RPh, Associate Director of the Drug Shortage Staff, CDER, **FDA**
12. **Advancing Pediatric Research and Treatment Development**
    Lynne Yao, MD, Acting Director, Division of Pediatric and Maternal Health/ODE IV/CDER, **FDA**
13. **Developing Additional Screening Diagnostics**
    Alberto Gutierrez, Director of the Office of In Vitro Diagnostics, CDRH, **FDA**
14. **Co-development and Co-branding of Treatments**
    Derek Gavin, Director of Development, **NORD**
15. **Promote a Culture of Patient Involvement in Medical Technology**
    Haja El Mubarak, Division of Microbiology Devices, CDRH, **FDA**
16. **Development and Regulation of Combination Products**
    James Bertram, CDRH Product Jurisdictional Officer, **FDA/CDRH/ODE**
17. **Genome Sequencing for Rare Disease Diagnosis**
    Kelly East, MS, CGC, Certified Genetic Counselor, Clinical Applications Lead, **HudsonAlpha Institute for Biotechnology**
    Nedra Whitehead, Director, Center for Genomics in Public Health, **RTI International**
18. **Educating Medical Professionals about Rare Diseases**
    Mary Dunkle, Vice President Educational Initiatives, **NORD**
    Karren Williams, Executive Director Global Medical Communications, **Akcea Therapeutics**
19. **How Off-label Reimbursement Issues will Affect Rare Disease Patients**
    Steven A. Grossman, President, **HPS Group**
    Pamela K. Gavin, Chief Operating Officer, **NORD**
20. **Facilitation of Earlier Diagnosis of Rare Diseases**
    Joan Keutzer, PhD, Vice President and Head of Integrated Solutions for Rare Diseases, **Sanofi Genzyme**
    Eden Haverfield, PhD, FACMG, Medical Geneticist, **Invitae**

Networking Appointment Times Available

"This has been a successful meeting between my spiritual and emotional dedication to fellow rare disease patients and the scientific & regulatory experts with their valuable lot of tools to help us navigate the confusing aspects of being a good advocate."

— Director of Support Groups, **Cushing's Support and Research Foundation**

BREAKOUT A

The Crucial Role of Data in Advancing Diagnosis and Clinical Drug Development

Rare diseases pose special challenges related to data collection and analysis, but the potential value of data for this community has been well documented. This panel will discuss innovative ways to collect, share or use data to advance rare disease diagnosis and treatment.

- Understand the pivotal role of natural history and registry studies in orphan product development
- Hear case studies regarding the use of data for patients with ultra-rare diseases
- Discuss current trends in targeting the molecular basis of disease

Moderator:

Marshall L. Summar, MD,
Division Chief, Genetics & Metabolism,
Children's National Health System

Panelists:

Matthew Might, Associate Professor of Computing, **University of Utah;** Adviser, **Precision Medicine Initiative;** Visiting Professor, **Harvard Medical School**

Michael A. Pacanowski, MPH, PharmD, Associate Director for Genomics and Targeted Therapy, CDER, **FDA**

Michael Binks, MD, Vice President, Clinical Research, Rare Disease Research Unit, **Pfizer Inc**

Eden Haverfield, PhD, FACMG, Medical Geneticist, **Invitae**

BREAKOUT B

Collaborations Across Borders — Addressing Rare Diseases as a Global Public Health Challenge

Rare diseases affect patients around the world in such a way that singular country efforts are no longer enough to address the growing public health challenge. Collaborations must happen at a global scale. This panel features discussions from global leaders on the challenges and opportunities of a global, collaborative approach.

- Evaluate the various global approaches to clinical trials
- Prepare for a diverse set of global access challenges including payer reimbursement, patient support services and affordability
- Navigate the differing patient privacy and compliance issues that affect clinical trials development in each country
- Learn how regulatory agencies are collaborating to create a more seamless process
- Initiation of the EMA/FDA Rare Disease Cluster

Moderator:

Lisa Phelps, MPH, Director of Marketing & Community Relations, **NORD**

Panelists:

David Altarac, MD, MPA,
Head of Global Regulatory Affairs, **Shire**

Jonathan Goldsmith, MD, FACP, Associate Director Rare Diseases Program/OND, CDER, **FDA**

Jules T. Mitchel, President and Co-Founder, **Target Health Inc.**

Durhane Wong-Rieger, President,
Canadian Organization for Rare Disorders

BREAKOUT C

Focus on Pediatric Diseases — Advancing Research and Treatments

While more than half of the patients with rare diseases are children and all pediatric cancers are rare, pediatric diseases fall far behind adult rare diseases with respect to current research and approved therapies.

- Advance research and development of safe, effective treatments for children with rare diseases
- Gain a deeper understanding of the Rare Pediatric Disease Priority Review Voucher
- Learn what the Cancer Moonshot Initiative may mean for children and pediatric oncology

Moderator:

Tiina K. Urv, PhD,
Program Director, Division of Clinical Innovation, NCATS,
NIH

Panelists:

Lynne P. Yao, MD, Acting Director,
Division of Pediatric and Maternal Health/ODE IV/CDER,
FDA

Larry Bauer,
Regulatory Scientist, CDER,
FDA

David Arons, JD, Chief Executive Officer,
National Brain Tumor Society;
Member, Blue Ribbon Panel,
National Moonshot Cancer Initiative

Mark Dant,
President and CEO,
National MPS Society

2:45 Networking and Refreshment Break

Networking Appointment Times Available

3:30 The Challenge of Access and Reimbursement — Rising Concerns Regarding Affordability, Innovation and Quality of Care

Access and affordability for rare disease patients continue to pose unique challenges. This panel features thought-leaders from each sector of the healthcare community who come together to discuss how the Affordable Care Act, healthcare marketplace and rising cost of medications affect the rare disease community.

- Maintain focus on the cost of diseases versus the cost of medicine to transform patient care and access
- Discover innovations in manufacturer-driven patient support programs and healthcare insurance benefit design to determine the downstream effect on rare disease patients
- Understand how the post-ACA healthcare marketplace poses unique challenges and difficulties for rare disease patients including limited affordable options in coverage

Moderator:

Catherine Blansfield, MA, BS, RN,
Vice President of Access and Outcomes,
NORD

Panelists:

Lori Reilly,
Executive Vice President, Policy & Research,
PhRMA

Loreen M. Brown, MSW, Senior Vice President, Product Strategy and Commercialization Excellence,
Lash Group, a part of AmerisourceBergen

Karen L. Erickson, Associate Executive Director, Community Engagement, **The Alpha-1 Foundation**

Peter Dehnel, Medical Director,
Blue Cross and Blue Shield of Minnesota

4:30 The Landscape for Investment

In recent years, the investment community has felt comfortable investing in orphan medical products, even though the target population by definition is limited. Whether this trend continues depends on how investors view the future of orphan products and whether the environment is conducive.

- Examine how the investment community views orphan products
- Determine what factors investors take into account in deciding how to invest
- Identify whether investors are optimistic about the prospects for 2017 and beyond

Moderator:

Jim Geraghty, Entrepreneur-In-Residence,
Third Rock Ventures

Panelists:

Ted W. Love, MD, Chief Executive Officer,
Global Blood Therapeutics

Peter Kolchinsky, Managing DIrector & Portfolio Manager,
RA Capital Management

Amanda L. Hayward, PhD,
Independent

5:30 Close of Day One

Networking Cocktail Reception

(immediately following the close of day one)

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DAY TWO TUESDAY, OCTOBER 18, 2016

7:30 Continental Breakfast Opens

Networking Appointment Times Available

8:00 Day Two Insights

Peter L. Saltonstall,
President and CEO,
NORD

8:10 Update from NORD Board of Directors

Marshall L. Summar, MD,
Division Chief, Genetics & Metabolism,
Children's National Health System

8:20 **NATIONAL ELECTION IMPLICATIONS KEYNOTE ADDRESS**

Kate Rawson, Senior Editor, **Prevision Policy**

9:00 **Driving Progress Through Policy**

Moderator:
Martha Rinker, Vice President of Public Policy, **NORD**

Part I: State-Based Advocacy

- Leverage lessons learned from successes and challenges of state-based advocacy initiatives
- Garner insights into future state advocacy programs

Tim Boyd, Associate Director of State Policy, **NORD**

Part II: National Policy Outlook

- Examine the implications stemming from PDUFA VI

Paul Melmeyer, Associate Director of Public Policy, **NORD**

10:00 Networking and Refreshment Break

SPONSORED BY: Pfizer

Networking Appointment Times Available

10:45 **CHOOSE FROM THREE TRACKS (I-III)**

TRACK I

Trending Topics from FDA

10:45 Track Chair Opening Remarks

Richard Moscicki, MD,
Deputy Center Director for Science Operations,
FDA

10:50 **Biosimilars Update**

Sue Lim, MD, Team Lead for Therapeutic Biologics, OND Therapeutic and Biologics Team, CDER, **FDA**

11:10 **Externally-Led PFDD**

Pujita Vaidya, MPH, Acting Director, Decision Support and Analysis Team, Office of Strategic Programs, CDER, **FDA**

11:30 **Current Perspectives on Orphan Exclusivity Incentives**

Gayatri Rao, MD, JD, Director, OOPD,
FDA

11:50 **The Importance of the Patient's Input at Advisory Committees**

Richard Klein, Director,
Patient Liaison Program, Office of Health and Constituent Affairs, OHCA, **FDA**

TRACK II

Strategies to Address Patient Challenges

10:45 Track Chair Opening Remarks

Richard Peters, MD, PhD, Senior Vice President, Head, Global Rare Diseases Franchise and Head, JAPAC Business Unit, **Sanofi Genzyme**

10:50 **Ensuring Patients' Access to Education — A Civil Rights Issue**

Lisa Terrizzi, JD, General Counsel, **NORD**
Paul Weckstein, JD, Co-Director,
The Center for Law and Education

11:10 **Rare Diseases and Emergency Room Visits**

Carrie Koenig, Program Coordinator,
Hemophilia Federation of America

11:25 **Transition to Adulthood for Pediatric Patients**

Caitlin Dwyer, MSW, LSW, Social Worker, Division of Nephrology, **Children's Hospital of Philadelphia**

11:40 **Advocacy for the Undiagnosed**

Gina Szajnuk, Co-Founder and Executive Director,
Rare and Undiagnosed Network (RUN)

12:00 **The Challenge of Drug Shortages for the Rare Disease Patient**

Valerie Jensen, RPh, Associate Director of the Drug Shortages Staff, CDER, **FDA**

TRACK III

Breaking Down Barriers to Access

10:45 Track Chair Opening Remarks

Jim Anderson, Senior Director, Business Development, Head, Reimbursement, Access & Distribution Solutions, Emerging Biopharma, **QuintilesIMS**

10:50 **Off-Label Usage and Reimbursement Concerns**

Steven Grossman, President, **HPS Group, LLC**

11:10 **The Impact of Preferred Drug Lists and Formulary Exclusions**

Jan Nielsen, Division President, Sonexus™ Access & Patient Support,
Cardinal Health

11:30 **The Role of Specialty Pharmacies and Hubs in Product and Patient Access**

Nicole Hebbert, Vice President,
Patient Access and Engagement,
UBC: An Express Scripts Company

11:50 **The Challenges of Orphan Product Pricing**

Jayson Slotnik, JD,
Principal and Founding Member,
Health Policy Strategies, Inc.

TRACK I CONTINUED

12:10 **Case Studies and Flexibility on Recent Approvals**

John Jenkins, MD,
Director, Office of New Drugs,
FDA

12:30 **Closing Panel Discussion**

TRACK II CONTINUED

12:15 **Rare Disease Patients and Medical Devices**

Michelle Tarver, MD, CDRH,
FDA

12:30 **Closing Panel Discussion**

TRACK III CONTINUED

12:10 **Compassionate Use and Expanded Access**

Lucas Kempf, MD,
Medical Officer, Rare Diseases Program, CDER,
FDA

12:30 **Closing Panel Discussion**

1:00 CLOSE OF TRACKS AND NETWORKING LUNCHEON *Networking Appointment Times Available*

2:15 **NORD and Trio Health Partnership to Improve Quality of Care and Outcomes**

Pamela K. Gavin, Chief Operating Officer, **NORD**

Brent Clough, CEO, **Trio Health**

3:00 **Predicting the Pipeline — Orphan Product Development and Progress in 2017**

Orphan drug approvals have steadily increased over the past few years, with the FDA and NIH providing incentives and funding for research and development of new treatments for rare diseases. As the industry looks forward into 2017 and beyond — What else can be expected? Will the orphan drug approvals continue to rise? Are certain rare diseases or therapeutic areas more likely to gain investment than others? This panel takes stock of the 2016 orphan drug development landscape and looks forward into the pipeline of potential product development and approvals for the coming year.

- Explore the rare diseases and therapeutic areas that have the greatest need for drug developments as well as the strongest likelihood of drug approval for 2017
- Assess whether these disease states in need line up with the areas of interest from the investment community
- Discover how to best incorporate patient advocates into the drug development paradigm

Moderator:
Philip J. Brooks, PhD, Program Director,
Division of Clinical Innovation, NCATS, **NIH**

Panelists:
Eric Floyd, MS, MBA, PhD, Chief Scientific Officer,
Dohmen Life Science Services

Mike Lanthier, Operation Research Analyst,
Office of Planning, Office of the Commissioner, **FDA**

Noel Southall, PhD, Leader, Informatics,
Division of Pre-Clinical Innovation, NCATS, **NIH**

3:45 **The FDA Commitment to Rare Diseases**

Moderator:
Peter L. Saltonstall,
President and CEO,
NORD

Panelists:
Janet Woodcock, MD,
Director, CDER,
FDA

Peter Marks, MD, PhD,
Director, CBER,
FDA

5:00 **Closing Remarks**

Peter L. Saltonstall,
President and CEO,
NORD

5:00 CLOSE OF CONFERENCE

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 presented, illustrating key themes:

- **Innovative Research**
- **Medical Education Advancement**
- **Patient Community Building**
- **Other Life-Transforming Treatments and Advancements**

For any questions regarding the poster submissions, please visit www.nordsummit.org.

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3 Ways to Register

Website: www.nordsummit.org

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Phone: Roberts Apse
339-298-2290

* For additional pricing information, please contact Roberts Apse at 339-298-2290 or roberts.apse@cbinet.com

Fee includes continental breakfast, lunch, wine and cheese reception, refreshments and conference documentation.
Credit Card (Visa, MC, AMEX, Discover) or checks accepted. Please make checks (in U.S. funds drawn on a U.S. bank) payable to: CBI. (No personal checks accepted.) PLEASE NOTE: All advertised discounts are taken from the full, Standard Rate.

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NORD and CBI will offer a credit to those who cancel prior to the cut-off date to an alternative conference hosted by CBI within a six-month timeframe. In case of a conference cancellation, 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.*

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NORD stands behind the quality of its events, as does CBI for its conferences. If you are not satisfied with the quality of this event, a credit will be awarded towards a comparable conference hosted by CBI of your choice. Please contact (800) 817-8601 for further information.

* The opinions of the conference faculty do not necessarily reflect those of the companies they represent, NORD or CBI.

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.

To apply, please go to www.nordsummit.org/summit-event-details/ 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 summit. The overall theme of the poster sessions is "Life-Transforming Treatments;" however, the four themes of "Innovative Research", "Medical Education Advancement", "Patient Community Building" and "Other" are the suggested specific topic areas within that over-arching theme that the planning committee would like addressed. For any questions regarding the poster submissions or to submit an abstract please contact...

Ashley Worrell
339-298-2113
ashley.worrell@cbinet.com

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