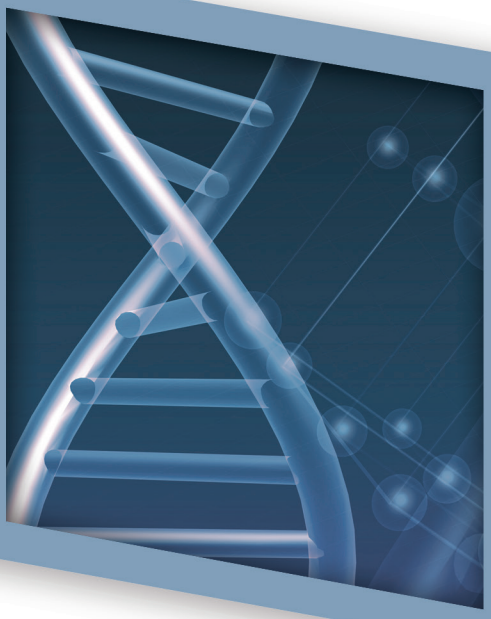
US CONFERENCE ON

# Rare Diseases and Orphan Products

## Shaping the Future Now

**OCTOBER 22-24, 2012**

Washington, DC, USA



NORD
National Organization for Rare Disorders

DIA®
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FDA

NATIONAL INSTITUTES OF HEALTH

EURORDIS
Rare Diseases Europe

Duke Department of Pediatrics
Duke University School of Medicine

This activity has been planned and implemented in accordance with the Essential Areas and policies of the Accreditation Council for Continuing Medical Education (ACCME) through the joint sponsorship of Postgraduate Institute for Medicine (PIM) and the Drug Information Association. PIM is accredited by the ACCME to provide continuing medical education for physicians.

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**LEARNING OBJECTIVES**

At the conclusion of this conference, participants should be able to:

- Define how the evolving health care environment will affect orphan product development and investment
- Discuss how the government and private sector are addressing special challenges faced by patients and companies
- Explain the unique challenges faced by patients with rare diseases and the organizations that represent the patients
- Discuss how to develop better communication among the investigator, patient, industry, investor and government influencers in the rare disease/orphan product community
- Discuss updates on recent initiatives for rare disease research and drug development by incorporating the perspectives of multiple stakeholder groups
- Synthesize didactic information with examples from case studies used to illustrate early research and development, natural history studies, clinical trial design and statistical analysis, endpoint development, and post-marketing opportunities
- Recognize the importance of collaboration in rare disease drug development through examples of success stories

## DIA's Certificate Program Statement

This program is part of DIA's Certificate Program and is awarded the following:

• Clinical Research Certificate Program: 12 Elective Units

*For more information go to www.diahome.org/certificateprograms*

## Disclosure of Conflicts of Interest

The Postgraduate Institute for Medicine (PIM) and DIA require instructors, planners, managers and other individuals who are in a position to control the content of this activity to disclose any real or apparent conflict of interest they may have as related to the content of this activity. All identified conflicts of interest are thoroughly vetted by PIM and DIA for fair balance, scientific objectivity of studies mentioned in the materials or used as the basis for content, and appropriateness of patient care recommendations.

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PROGRAM COMMITTEE

**Peter L. Saltonstall**
President and CEO
National Organization for Rare Disorders (NORD)

**Ronald J. Bartek**
President/Director/Co-Founder
Friedreich's Ataxia Research Alliance

**Larry Bauer, RN, MA**
Senior Regulatory Project Manager
Office of New Drugs, Rare Diseases Program
CDER, FDA

**Deborah Dolan, MBA**
Vice President, Key Accounts
AmerisourceBergen Corporation

**Diane Edquist Dorman**
Vice President of Public Policy
National Organization for Rare Disorders (NORD)

**Jayne C. Gershkowitz**
Senior Director
Patient Advocacy & Public Policy
Amicus Therapeutics

**Stephen C. Groft, PharmD**
Director, Office of Rare Diseases Research
National Institutes of Health

**Dennis Jackman**
Senior Vice President, Public Affairs
CSL Behring, L.L.C.

**Priya Kishnani, M.D.**
Professor of Pediatrics
Division Chief, Medical Genetics
Duke University Medical Center

**Anne R. Pariser, MD**
Associate Director for Rare Diseases, Office of New Drugs
CDER, FDA

**Wayne L. Pines**
President, Regulatory Services and Healthcare
APCO Worldwide, Inc.

**Gayatri Rao, JD, MD**
Director
Office of Orphan Products
OMPT, FDA

**Kelly C. Slone**
Director, Medical Industry Group
National Venture Capital Association

## WHO SHOULD ATTEND:

- Researchers from academia and drug and device companies
- Patient organizations and those interested in creating one
- Senior managers from drug and device companies interested in rare diseases
- Investors focused on the future of orphan product development
- Policy experts who are concerned about federal or state policies that affect patients with rare diseases
- Providers of services to the rare disease community, including insurance providers and health care professionals
- Government officials responsible for rare disease research and orphan product oversight

***Monitor the website for CE information.***

**Worldwide Headquarters**
Drug Information Association, Inc.
800 Enterprise Road, Suite 200
Horsham, PA 19044, USA
**Regional Offices**
Basel, Switzerland Tokyo, Japan Mumbai, India Beijing, China

## The Meeting is For All Stakeholders in the Rare Disease/Orphan Product Community

This is the annual meeting where all stakeholders - patients, patient organizations, researchers, drug and device companies, investors, thought leaders and government – meet to focus on rare diseases and orphan product research, development and access. At this year's meeting, we seek to gain a common understanding of the current and emerging challenges, opportunities, and strategies for working together effectively to shape a better future for rare diseases and orphan drugs. It is important to remember none of us can get there alone. The 2011 meeting brought together more than 300 stakeholders, and this year's event promises even more.

The format of the meeting includes both plenary and smaller group sessions. High-level plenary sessions will open a collaborative dialogue among leading researchers, company officials, investors, patient organizations and government leaders. Smaller group sessions are organized around three central themes: policy, research and regulation and special challenges. These sessions are intended to promote advancements in science, care and other considerations that will address the needs of the 30 million Americans with rare diseases. Topics include the current and emerging drug development environment, reenactment of the Prescription Drug and Medical Device User Fee Act, an update on NIH's new National Center Advancing Translational Science, and many others.

## HIGHLIGHTS

**Research and Regulation:**

- Senior staff from the FDA and NIH will discuss how to develop a new product and how to comply with NIH grant and FDA requirements for investigational new drugs and marketing applications
- Speakers will address the unique challenges faced by companies in the development of orphan products, and how to develop efficient clinical rare disease programs and avoid common pitfalls
- Learn how to work with federal agencies on grants and orphan designations

**Policy:**

- Hear about policies affecting rare diseases in this evolutionary time: the opportunities, challenges and possible solutions
- Discuss the legislation that, by the time of the meeting, Congress is likely to have enacted, including special provisions applicable to orphan products
- Learn some of the administrative initiatives that FDA is considering that will affect product development and government reviews
- Learn strategies on how you can become a more effective advocate

**Special Challenges in Rare Diseases:**

- Learn how the government and private sector are addressing the special challenges faced by patients and companies in the new health care environment
- Meet individuals who face the same challenges that you do
- Gain a better understanding of how the FDA and NIH work and how they interact with the drug and device development processes
- Learn ways to de-risk your investments in orphan products and understand better the timelines for research and regulatory reviews

## DAY 1 | OCTOBER 22, 2012

**7:30-8:30 AM** REGISTRATION AND CONTINENTAL BREAKFAST

**8:30-9:00 AM** WELCOME AND BRIEF OPENING REMARKS

**Peter L. Saltonstall**
President and CEO
National Organization for Rare Disorders (NORD)

**Anne R. Pariser, MD**
Associate Director for Rare Diseases,
Office of New Drugs
CDER, FDA

**Flaminia Macchia**
Director European Public Affairs
European Rare Diseases Organization (EURORDIS)

**9:00-9:30 AM** KEYNOTE 1 – THEME 1 (RESEARCH AND REGULATION)

**Robert M. Califf, MD, MACC**
Vice Chancellor for Clinical and Translational Medicine
Director, Duke Translational Medicine Institute

**9:30-10:00 AM** REFRESHMENT BREAK

**10:00-10:30 AM** KEYNOTE 2 – THEME 1 (RESEARCH AND REGULATION)

**TRND and Translational Development**

**Christopher P. Austin, MD**
Director, National Center for Advancing Translational Sciences (NCATS)

**10:30-11:30 AM** PLENARY SESSION – THEME 1 (RESEARCH AND REGULATION)

### Shaping the Future

Session Chair

**Stephen C. Groft, PharmD**
Director, Office of Rare Diseases Research
National Institutes of Health

Speakers

**Tissue Chips to Predict Drug Safety**

**Danilo A. Tagle, PhD**
Associate Director for Special Initiatives
National Center for Advancing Translational Sciences (NCATS), NIH

**NIH Drug Repurposing Program**

**Bonnie B. Dunn, PhD**
Program Director
National Center for Advancing Translational Sciences (NCATS), NIH

**11:30 AM–1:00 PM** LUNCH

**Luncheon Speaker – Theme 1 (Research and Regulation)**

### Cystic Fibrosis and Comprehensive Approach to Therapy Development

**Preston W. Campbell, III, M.D.**
Executive Vice President for Medical Affairs
Cystic Fibrosis Foundation

**1:00-2:30 PM** THEMES

#### Theme 1 (Research and Regulation)

**Well-Designed & Well-Conducted Clinical Trials**

Session Chair

**Gayatri Rao, JD, MD**
Director
Office of Orphan Products
OMPT, FDA

Speakers

**Trial Design and Statistical Considerations**

**Lisa M. LaVange, PhD**
Director, Office of Biostatistics
Office of Translational Sciences
CDER, FDA

**Ivacaftor Example**

**Anthony G. Durmowicz, MD**
Medical Officer, Office of New Drugs
CDER, FDA

**Panel Q&A**

#### Theme 2 (Policy)

**Impact of FDASIA on Orphan Product Development**

Moderator

**Wayne L. Pines**
President, Regulatory Services and Healthcare
APCO Worldwide, Inc.

Speakers

**John K. Jenkins, MD**
Director, Office of New Drugs
CDER, FDA

**Andrew J. Emmett, MPH**
Managing Director, Science and Regulatory Affairs
Biotechnology Industry Organization (BIO)

**The Impact of FDASIA on Orphan Device/ HUD Development**

**Cassie A. Scherer, JD**
Policy Advisor, Office of the Center Director, CDRH, FDA

#### Theme 3 (Special Challenges in Rare Diseases)

**The Challenges of Reimbursement for the Rare Disease Patient**

Moderator

**Pamela Gavin, MBA**
Senior Vice President, NORD

Speakers

**Dennis Jackman**
Senior Vice President, Public Affairs
CSL Behring, L.L.C.

**Tamir Orbach**
Former PNH patient,
Former President of the PNH Research and Support Foundation,
Former Vice President of the Aplastic Anemia & MDS International Foundation

**Challenges of Reimbursement and the Rare Disease Patient, the Specialty Pharmacy Perspective**

**Bill Martin**
Vice President, Pharma Strategies and Account Management
Express Scripts, Inc.

**2:30-3:00 PM** REFRESHMENT BREAK

**3:00-4:00 PM** PRESENTATION OF POSTERS SESSION

Session Chair

**Anne R. Pariser, MD**
Associate Director for Rare Diseases, Office of New Drugs
CDER, FDA

Poster Presenters

**The CLARITY Challenge: Children's Leadership Award for the Reliable Interpretation and appropriate Transmission of Your genomic Information**

**Catherine Astrid Brownstein, PhD, MPH**
Boston Children's Hospital
Department of Genetics and Program in Genomics

**Elucidating the mechanism of the allergic inflammation in Eosinophilic Esophagitis**

**Mari Kent**
James Madison University
Laboratory of Immunopathogenesis, O&O Alpan LLC

**Characterizing New Molecular Entity Approvals with Orphan Product Designation, 1986-2011**

**Kathleen Miller, MS**
Doctoral Student
University of North Carolina At Chapel Hill

**Development of a keystone translational biomarker for orphan mitochondrial diseases**

**Will Matthew Klein, MD, MS**
Senior Vice President
Edison Pharmaceuticals

**Accelerating FDA approvals of new molecular entities for rare diseases: review of data from the Database Analysis Search Host**

**Katie Sinicrope**
Research Analyst
Eastern Research Group, Inc.

**Newborn Screening of Rare Diseases: Selected Programs at the National Institutes of Health that Span Screening to Research**

**Tiina K. Urv, PhD**
Program Director
National Institute of Child Health and Human Development (NICHD)

**4:00-5:30 PM** THEMES

## Theme 1 (Research and Regulation)

**Small Clinical Trials**

Session Chair

**Larry Bauer, RN, MA**
Senior Regulatory Project Manager
Office of New Drugs, Rare Diseases Program
CDER, FDA

Speakers

**Principles and Design Features, N of 1, Non-Traditional Study Designs**

**Robert J. Temple, MD**
Deputy Center Director for Clinical Science
CDER, FDA

**Case Example #1 glucarpidase**

**Patricia A. Dinndorf, MD**
Medical Officer, FDA Office of Hematology Oncology Products, Division of Hematology Products
CDER, FDA

**Case Example #2 Mifepristone**

**Marina S. Zemskova, MD**
Medical Officer, DMEP (Division of Metabolic and Endocrine Drugs), OND
CDER, FDA

Panel Q & A

## Theme 2 (Policy)

**Comparative Effectiveness Research and Health Technology Assessments (HTA)**

Moderators

**Douglas R. Paul, PharmD, PhD**
Vice President & Partner
Medical Marketing Economics, LLC

Speakers

**Lauren Barnes, MHS**
Senior Vice President
Avalere Health

**Donald Han**
Senior Director Market Access- Rare Diseases
Pfizer

**Michael S. Lauer, MD, FACC, FAHA**
Director, Division of Cardiovascular Sciences (DCVS) National Heart, Lung, and Blood Institute

## Theme 3 (Special Challenges in Rare Diseases)

**Investing in Orphan Products: Is the Environment getting Better Or Worse**

Moderator

**Thomas M. Burton, JD**
Staff Reporter
The Wall Street Journal

Speakers

**David I. Scheer, MS**
Chairman of the Board, Aegerion Pharmaceuticals
President, Scheer & Company, Inc.

**Fritz Bittenbender**
Vice President of Alliance Development and State Government Relations, Biotechnology Industry Organization (BIO)

**Ritu S. Baral**
Principal, Senior Biotechnology Analyst
Canaccord Genuity

**5:30-6:30 PM** NETWORKING RECEPTION

## DAY 2 | OCTOBER 23, 2012

7:30 – 8:30 AM

CONTINENTAL BREAKFAST

8:30-8:35 AM OPENING REMARKS

**Paul Pomerantz**
DIA Worldwide Executive Director

8:35-9:15 AM KEYNOTE – THEME 2 (POLICY)

**Stephen P. Spielberg, MD, PhD**
Deputy Commissioner,
Medical Products and Tobacco
Office of the Commissioner, FDA

9:15-10:30 AM PLENARY SESSION – THEME 2 (POLICY)

### Shaping the Future of Health Policy Now

Moderator

**Cole Werble, MA**
Founding Member, Prevision Policy, LLC

Speakers

**David P. Meeker MD**
President and Chief Executive Officer, Genzyme

**Charles A. Mohan, Jr.**
CEO/Executive Director
The United Mitochondrial Disease Foundation

10:30-11:00 AM POSTER SESSION / NETWORKING

11:00 AM-12:30 PM LUNCH

**Luncheon Speaker – Theme 2 (Policy)**
**Speaker Invited**

12:30–2:00 PM THEMES

### Theme 1 (Research and Regulation)

**Natural History Studies**

Session Chair

**David J. Eckstein, PhD**
Senior Health Scientist Administrator
Office of Rare Diseases Research
National Center for Advancing Translational Sciences
National Institutes of Health

Speakers

**White paper findings/overview of May workshop**

**Marc K Walton, MD, PhD**
Associate Director for Translational Medicine
Office of Translational Sciences
CDER, FDA

**Case studies**

**Jeffrey Krischer, Ph.D.**
Professor, Department of Pediatrics
University of South Florida College of Medicine

**Case study**

**Marshall L. Summar, MD**
Chief, Genetics and Metabolism
Margaret O'Malley Professor of Genetic Medicine
Professor of Pediatrics, George Washington University
Children's National Medical Center

**Panel Q & A**

### Theme 2 (Policy) and Theme 3 (Special Challenges in Rare Diseases)

**Role of Academic Centers in Orphan Product Development**

Moderator

**Susan Riley Keyes, PhD, JD**
Vice President
Nemucore Medical Innovations, Inc

Speakers

**Priya Kishnani, M.D.**
Professor of Pediatrics
Division Chief, Medical Genetics
Duke University Medical Center

**Andrew E. Mulberg, MD, FAAP, CPI**
Division Deputy Director
Division of Gastroenterology and Inborn Error Products
Office of Drug Evaluation III
CDER, FDA

**Lisa Coles, PhD, MS**
Post-doctoral Fellow
Center for Orphan Drug Research,
College of Pharmacy, University of Minnesota

2:00-2:15 PM REFRESHMENT BREAK

2:15-3:45 PM THEMES

## Theme 1 (Research and Regulation)

**Endpoint Development**

Session Chair

**Emanuela Lacana, PhD**
Lead Interdisciplinary Scientist
CDER, FDA

Speakers

**Clinical Outcome Assessments/PROs**

**Elektra J. Papadopoulos, MD, MPH**
Medical Officer; Study Endpoints and Labeling Development; Office of New Drugs
CDER, FDA

**EoE Case Study**

**Gumei Liu, MD, PhD**
Commissioner's Fellow
CDER, FDA

**Panel Q&A**

## Theme 2 (Policy) and Theme 3 (Special Challenges in Rare Diseases)

**The New Relationship with the Patient Community – FDA/NIH and the Patient**

Moderator

**Jayne C. Gershkowitz**
Senior Director
Patient Advocacy & Public Policy
Amicus Therapeutics

Speakers

**Diane Edquist Dorman**
Vice President Public Policy
National Organization for Rare Disorders (NORD)

**Richard Klein**
Public Health Specialist
Office of Special Health Issues
OC, FDA

**Theresa M. Mullin, PhD**
Associate Director, Office of Planning and Informatics
CDER, FDA

**Incorporating Patient Views on Benefits vs. Risk in Medical Device Approvals**

**Philip Desjardins, JD**
Associate Director for Policy
CDRH, FDA

3:45 – 4:00 PM REFRESHMENT BREAK

4:00 – 5:20 PM THEMES

## Theme 1 (Research and Regulation)

**Post-Marketing Period-Opportunities for Continued Learning**

Session Chair

**Rashmi Gopal-Srivastava, Ph.D.**
Director, Extramural Research Program
Office of Rare Diseases Research (ORDR)
NCATS, National Institutes of Health

Speakers

**Immune Tolerance**

**Priya Kishnani, MD**
Professor of Pediatrics;
Division Chief, Medical Genetics,
Duke University Medical Center

**PKU-Continued Challenges and Gaps**

**Melissa A. Parisi, MD, PhD**
Chief, Intellectual and Developmental Disabilities Branch
Center for Developmental Biology and Perinatal Medicine
Eunice Kennedy Shriver National Institute of Child Health and Human Development

**Panel Q&A**

## Theme 2 (Policy) and Theme 3 (Special Challenges in Rare Diseases)

**The New Relationship with the Patient Community – Industry and the Patient**

Moderator

**Mary Cobb, MS**
Senior Vice President, Membership and Organizational Strategy
National Organization For Rare Disorders (NORD)

Speakers

**Ronald J. Bartek**
President/Director/Co-Founder
Friedreich's Ataxia Research Alliance

**Geraldine Carroll**
Director, Patient Advocacy Relations
Allergan

**David Caponera, RRT, MHA**
Vice President, Patient Advocacy & Reimbursement
Aegerion Pharmaceuticals

**John W. Walsh**
President, CEO and Co-Founder
Alpha-1 Foundation

5:30-6:00 PM SPECIAL SESSION

### Facing the Crisis in Biomedical Innovation: A Venture Investor's Perspective

**Jonathan S. Leff, MBA**
Managing Director, Warburg Pincus

## DAY 3 | OCTOBER 24, 2012

**7:30-8:30 AM** CONTINENTAL BREAKFAST

**8:30-9:00 AM** KEYNOTE – THEME 3 (SPECIAL CHALLENGES IN RARE DISEASES)

**John J. Castellani**
President & CEO
Pharmaceutical Research and Manufacturers of America (PhRMA)

**9:00-10:15 AM** PLENARY SESSION – THEME 3 (SPECIAL CHALLENGES IN RARE DISEASES)

### Access and Reimbursement

Moderator

**Peter L. Saltonstall**
President and CEO
National Organization for Rare Disorders (NORD)

Speakers

**Mark McClellan, MD, PhD**
Director, Engelberg Center for Health Care Reform
and Leonard D. Schaeffer Chair in Health Policy Studies
The Brookings Institution

**Richard H. Bagger**
Senior Vice President, Corporate Affairs & Strategic Market Access
Celgene Corporation

**Miriam O'Day**
Senior Director Public policy
Alpha-1 Foundation

**10:15-10:30 AM** REFRESHMENT BREAK

**10:30–11:30 AM** THEMES

#### Theme 1 (Research and Regulation)

**Working with the FDA**

Session Chair

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

Speakers

**Orphan Designations & Grants**

**Gayatri Rao, JD, MD**
Director
Office of Orphan Products
OMPT, FDA

**Importance of Meetings with FDA**

**Larry Bauer, RN, MA**
Senior Regulatory Project Manager
Office of New Drugs, Rare Diseases Program
CDER, FDA

**Panel Q & A**

#### Theme 2 (Policy) and Theme 3 (Special Challenges in Rare Diseases)

**NIH Updates**

Moderator

**Stephen C. Groft, PharmD**
Director, Office of Rare Diseases Research
National Institutes of Health

Speakers

**NIH Undiagnosed Diseases Program and Planned Extension to the Extramural research Community**

**Cynthia J. Tifft, MD, PhD**
Deputy Clinical Director
National Human Genome Research Institute

**Whole Genome Sequencing as a Diagnostic Tool for Rare Genetic Diseases**

**David Adams, MD, PhD**
Senior Staff Clinician
Pediatrics, Clinical and Biochemical Genetics
Unknown Diseases Program, NIH
MGB/NHGRI/NIH

**Glybera: Progress Towards Drug Approval for Gene Therapy**

**Robert Kotin, PhD**
Senior Investigator and Head of the Laboratory of Molecular
Virology and Gene Therapy
Genetics and Developmental Biology Center
NHLBI, NIH

**11:30 AM-12:45 PM** LUNCH

**Luncheon Speaker – Theme 3 (Special Challenges in Rare Diseases)**

**Christopher Jennings**
President,
Jennings Policy Strategies

## 12:45-2:00 PM THEMES

### Theme 1 (Research and Regulation)

**Translational/Late Phase Development**

Session Chair

**Emanuela Lacana, PhD**
Lead Interdisciplinary Scientist
CDER, FDA

Speakers

**Strategies & planning next steps, repurposing**

**John C. McKew, Ph.D.**
Chief Therapeutics Development Branch
Therapeutics for Rare and Neglected Diseases (TRND)
BrIDGs (former NIH-RAID)

**Examples from TRND Experience**

**G. Sitta Sittampalam, PhD**
Senior Scientist & Project Manager
National Center for Advancing Translational Sciences (NCATS/NIH)
Therapeutics for Rare & Neglected Diseases (TRND)

**Panel Q & A**

### Theme 2 (Policy)

**Risk Tolerance for the Rare Disease Patient**

Moderator

**Diane Edquist Dorman**
Vice President Public Policy
National Organization for Rare Disorders (NORD)

Speakers

**Markham C. Luke, MD PhD**
Deputy Director, Office of Device Evaluation CDRH, FDA

**Patrick Frey**
Director, Office of Planning and Analysis
CDER, FDA

**Kevin D. Romer, MBA**
President
National Tay-Sachs and Allied Diseases Association

### Theme 3 (Special Challenges in Rare Diseases)

Moderator

**Steve Usdin,**
Washington editor, BioCentury
Co-host, BioCentury This Week

**12:45–1:25 PM**

**Internationalization of Rare Disease Community**

Speakers:

**The Importance of the International Dimension in Rare Disease Treatment**

**Paul Perreault**
President, CSL Behring

**José T. Morales**
Partner, Accenture, Inc.
Board Member, Cystinosis Research Network

**1:25–2:00 pm**

**The Ethical Challenges of Genetic Testing**

Speakers:

**Gail H. Javitt, MPH, JD**
Counsel
Sidley-Austin LLP

## 2:00-2:15 PM REFRESHMENT BREAK

## 2:15-3:30 PM COLLABORATION EXAMPLE: THE NP-C STORY

Session Chair

**Forbes D. Porter, MD, PhD**
Senior Investigator
Acting Program Director
Clinical Director
Eunice Kennedy Shriver National Institute of Child Health and Human Development
National Institutes of Health, DHHS

Speakers

**Example of collaboration, multidisciplinary & stakeholder approach**

**Elizabeth Ottinger, PhD**
Project Manager Team Lead
Therapeutics for Rare & Neglected Diseases (TRND)
National Center for Advancing Translational Sciences (NCATS)
National Institutes of Health (NIH)

**Lessons Learned**

**Charles H. Vite, DVM, PhD**
Associate Professor Veterinary Neurology, School of Veterinary Medicine
University of Pennsylvania

**Nuria Carrillo, MD**
Therapeutics for Rare and Neglected Diseases (TRND)

**Panel Q & A**

**Panel includes speakers above and:**

**Lynne P. Yao, MD**
Acting Associate Director, Office of New Drugs,
Pediatric and Maternal Health Staff,
CDER/FDA

**3:30-4:30 PM** CLOSING PLENARY

## What the Future Looks Like for the Rare Disease Patient

MODERATOR

**Wayne L. Pines**
President, Regulatory Services and Healthcare
APCO Worldwide, Inc.

SPEAKERS

**Peter L. Saltonstall**
President and CEO
National Organization for Rare Disorders (NORD)

**Janet Woodcock, MD**
Director, Center for Drug Evaluation and Research (CDER)
Food and Drug Administration (FDA)

**Jonathan S. Leff, MBA**
Managing Director, Warburg Pincus

**Tamir Orbach**
Former PNH patient,
Former President of the PNH Research and Support Foundation,
Former Vice President of the Aplastic Anemia & MDS International Foundation

**Maria Mavris**
Director Therapeutics Development
European Rare Diseases Organization
(EURORDIS)

**4:30 PM** CONFERENCE ADJOURNED

## REGISTRATION FORM
Register online or fax this page to +1.215.442.6199

### US Conference on Rare Diseases and Orphan Products: Shaping the Future Now

Event #12017 • October 22-24, 2012

Capitol Hilton, Washington, DC, USA

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| Government (Full-time) | | US $500 ❑ |
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October 22-25 I Washington, DC

Register for *Clinical Trial Endpoints: Methods and Practice in Developing Measurements* and *US Conference on Rare Diseases & Orphan Products* and Save $200.

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**GROUP DISCOUNTS*** Register 3 individuals from the same company and receive complimentary registration for a 4th! **All 4 individuals must register and prepay at the same time – no exceptions.** DIA will apply the value of the lowest applicable fee to this complimentary registration; it does NOT include fees for optional events or DIA membership. You may substitute group participants of the same membership status at any time; however, administrative fees may be incurred. **Group registration is not available online and does not apply to the already-discounted fees for government or charitable nonprofit/academia.** To take advantage of this offer, please make a copy of this registration form for EACH of the four registrants from your company. Include the names of all four group registrants on each of the forms and return them together to DIA.

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**3.** _____

Payment options: Register online at www.diahome.org or check payment method.

❑ **CREDIT CARD** number may be faxed to: +1.215.442.6199. You may prefer to pay by check or bank transfer since non-U.S. credit card payment will be subject to the currency conversion rate at the time of the charge.

☐ Visa ☐ MC ☐ AMEX Exp Date_____

Card # _____

Name (printed) _____

Signature_____

❑ **CHECK** drawn on a US bank payable to and mailed along with this form to: Drug Information Association Inc, P.O. Box 95000-1240, Philadelphia, PA 19195-1240, USA. Please include a copy of this registration form to facilitate identification of attendee.

❑ **BANK TRANSFER** When DIA completes your registration, an email will be sent to the address on the registration form with instructions on how to complete the Bank Transfer. Payment should be made in US dollars. Your name and company, as well as the Event I.D. # must be included on the transfer document to ensure payment to your account.

**TRAVEL AND HOTEL** The most convenient airport is Ronald Reagan National Airport and attendees should make airline reservations as early as possible. The Capitol Hilton is holding a block of rooms at the **reduced rate below until October 1, 2012**, for the DIA event attendees. Room availability at this rate is guaranteed only until this date or until the block is filled.

**Single $275 Double $275**

Attendees must make their own hotel reservations. Contact the Capitol Hilton by telephone at +1.800.HILTONS and mention the DIA event. The hotel is located at 1001 16th Street NW, Washington, DC, USA 20036.

**CANCELLATION POLICY: On or before October 15, 2012**
**Administrative fee that will be withheld from refund amount:**
**Member or Nonmember = $200**
**Government or Charitable Nonprofit/Academia (Member or Nonmember) = $100**
Cancellations must be in writing and be received by the cancellation date above. Registrants who do not cancel by that date and do not attend will be responsible for the full registration fee paid. Registrants are responsible for cancelling their own hotel and airline reservations. You may transfer your registration to a colleague at any time but membership is not transferable. Please notify DIA of any such substitutions as soon as possible. Substitute registrants will be responsible for nonmember fee, if applicable.

**DIA reserves the right to alter the venue, if necessary. If an event is cancelled, DIA is not responsible for any airfare, hotel or other costs incurred by registrants.**

**Participants with Disabilities:** DIA event facilities and overnight accommodations are accessible to persons with disabilities. Services will be made available to sensory-impaired persons attending the event if requested at least 15 days prior to event. Contact the DIA office to indicate your needs.

### TABLETOP EXHIBIT INFORMATION
Attendees may visit the tabletop exhibits during the event and receptions.
Contact **Shannon Lewis Exhibits Associate,** Phone **+1.215.442.6149**
Fax **+1.215.442.6199,** email **Shannon.Lewis@diahome.org**

### EVENT INFORMATION
Contact **Carrie Dunn, Program Developer,** Phone **+1. 215.442.6181**
Fax **+1.215.442.6199,** email **Carrie.Dunn@diahome.org**

**Please check the applicable category:**

❑ Academia ❑ Government ❑ Industry ❑ CSO ❑ Student ❑ Patient Organizations/Patients (Call for registration information)

Last Name _____

First Name _____ M.I.

Degrees _____ ❑ Dr. ❑ Mr. ❑ Ms.

Job Title _____

Company _____

Address (As required for postal delivery to your location) _____ Mail Stop

City _____ State Zip/Postal Country

email **Required for confirmation** _____

Phone Number _____ Fax Number **Required for confirmation**