US Conference on Rare Diseases & Orphan Products:
The New Era in Health Care

October 7-9 | North Bethesda, MD

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NORD

30 YEARS | EMPOWERING THE RARE DISEASE COMMUNITY

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EURORDIS Rare Diseases Europe

The One Conference for all Stakeholders in the Rare Disease/ Orphan Product Community

PROGRAM COMMITTEE

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National Organization for Rare Disorders (NORD)

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Register at
diahome.org/RareDiseases2013

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OVERVIEW:

This year, the third annual conference of stakeholders in the rare disease/ orphan product community comes at an opportune time. Two new laws – the Affordable Care Act (ACA) and the FDA Safety and Innovation Act (FDASIA) – are being implemented. Together, these laws will shape the future for the rare disease orphan product community for years to come. This annual conference convenes all stakeholders in the rare disease/orphan product community – patients, patient organizations, researchers, venture capital firms, drug and device companies, investors, thought leaders and government – to focus on rare diseases and orphan product research, development and access.

The format of the conference 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 the four central themes. In addition, there will be a significant poster session that will highlight the latest research.

FEATURES FOUR MAJOR THEMES:

Research & Regulation:

- Government 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
- FDA will explain how the FDASIA provisions related to orphan products are being implemented

Access and Reimbursement:

- Experts in reimbursement will explain how access to existing and new therapies is being affected in the new health care delivery environment

The Role of the Patient in the Research and Regulatory Process:

- Learn how the new patient-centric program is being implemented at the FDA, with the patient voice being inserted more frequently into Benefit-risk decisions
- Learn how individual patients are working with the FDA and drug companies on specific products being developed

The Implementation of the Affordable Care Act:

- Representatives from the Center for Medicare and Medicaid will explain how the Affordable Care Act is being implemented and how it affects the rare disease/orphan product community

Thank you to our Media Partners:

RDR Rare Disease REPORT

Rare Diseases

CONTINUING EDUCATION

The Drug Information Association is accredited by the Accreditation Council for Pharmacy Education as a provider of continuing pharmacy education. This program is designated for up to 4.5 contact hours or .45 continuing education units (CEU's). Type of Activity: Knowledge

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If you would like to receive a statement of credit, you must attend the conference (and tutorial if applicable), sign in each day and if applicable, at the three designated ACPE-certified sessions (see PHARMACY CREDIT ALLOCATION), and complete the online credit request process through My Transcript. To access My Transcript, please go to www.diahome.org, select "Login to My DIA" and you will be prompted for your user ID and password. Select "My Transcript" (left side bar) and "Credit Request" to process your credit request. Participants will be able to download a statement of credit upon successful submission of the credit request. My Transcript will be available for credit requests on **Wednesday, October 23, 2013**.

Disclosure Policy

It is Drug Information Association policy that anyone in a position to control the content of a continuing education activity must disclose to the program audience (1) any real or apparent conflict(s) of interest related to the content of their presentation and/or the educational activity, and (2) discussions of unlabeled or unapproved uses of drugs or medical devices. Faculty disclosures will be included in the course materials.

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IACET CREDIT ALLOCATION

Tutorial: Overview of The Regulatory frameworks and Opportunities for Orphan Medicinal Products (OMPs): .3 IACET CEUs

US Conference on Rare Diseases and Orphan Products: 1.7 IACET CEUs

PHARMACY CREDIT ALLOCATION

October 8; Track 3: Paying for Orphan Therapies: 1.5 contact hours or .15 CEUs, 0286-0000-13-085-L04-P

October 8; Track 2 and 3: Assuring Patient Access to Treatments: 1.5 contact hours or .15 CEUs, 0286-0000-13-086-L05-P

October 9; Track 3: Managing Orphan Drug Recalls and Shortages: 1.5 contact hours or .15 CEUs, 0286-0000-13-087-L01-P

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NORD

The National Organization for Rare Disorders (NORD) is committed to improving the lives of the 30 million Americans living with rare diseases and assisting the organizations that serve them. Established in 1983, NORD provides programs of advocacy, education, research and patient/family services.

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 the special challenges faced by patients and companies under the Affordable Care Act and other changes in how health care is delivered and financed
- Express ideas on best practices for patients with rare diseases and the organizations that represent patients
- Discuss how to enhance communication among the investigator, patient, industry, investor and government influencers in the rare disease/orphan product community
- Discuss the latest initiatives in rare disease and orphan drug/device research and development
- Identify how FDA is implementing new legislation related to the review and approval of orphan drugs and humanitarian devices
- Discuss case studies which illustrate how industry, government and patients are collaborating to advance the development and approval of new therapies
- Explain the new initiatives in developing natural history studies, new methods of conducting clinical trial design and statistical analysis, endpoint development, and post-marketing opportunities
- Recognize the importance of collaboration in rare disease drug and device development
- Discuss the challenges faced in managing orphan drug shortages, paying for orphan therapies, and working with patients on obtaining access to special medicines

DIA'S CERTIFICATE PROGRAM

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

- Clinical Research Certificate Program: 12 Elective Units
- Clinical Safety and Pharmacovigilance Certificate Program: 4 Elective Units
- Regulatory Affairs Certificate Program: 12 Elective Units

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

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SUNDAY, OCTOBER 6

1:30-5:00 PM **TUTORIAL**

Overview of The Regulatory frameworks and Opportunities for Orphan Medicinal Products (OMPs)

TUTORIAL INSTRUCTOR:

Martine Zimmermann, PharmD
Vice President, Global Regulatory Affairs
Alexion Pharma International Sàrl

The tutorial will describe regulatory frameworks from FDA and EMA and incentives for development of Orphan Medicinal Products (OMPs). It will cover details regarding incentives for developments of OMPs, opportunities and challenges. Additionally, this tutorial will give an overview of the different options to gather control data in rare/ultra rare disease settings. Case studies for requests for ODDs and marketing authorizations for OMPs will be presented and discussed.

LEARNING OBJECTIVES:

At the conclusion of the tutorial attendees will be able to:

- Identify the main features of the different Orphan legislations including opportunities and challenges
- Develop a regulatory strategy for development of OMPs and discuss the specificities of applying the general provision of the pharmaceutical legislative framework
- Discuss the ways that health authorities have exercised regulatory flexibility to approve OMPs

TARGET AUDIENCE

- Individuals involved in development and registration of OMPs, including regulatory intelligence .

MONDAY, OCTOBER 7

7:30 – 8:30 AM REGISTRATION; CONTINENTAL BREAKFAST AND NETWORKING

8:30 – 8:45 AM WELCOME AND BRIEF OPENING REMARKS

DIA:
Barbara Lopez Kunz
Global Chief Executive
DIA

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

8:45 – 9:15 AM KEYNOTE

Steven A. Grossman, JD
President
HPS Group

9:15 – 10:00 AM REFRESHMENT BREAK, NETWORKING & POSTER VIEWING

10:00 – 10:30 AM UPDATE ON ORPHAN DRUG APPROVALS

Frank J. Sasinowski, MS, MPH, JD
Director, Hyman, Phelps and McNamara, P.C.
Board of Directors of the National Organization for Rare Disorders (NORD)

10:30 – 11:45 AM PLENARY SESSION 1

The Affordable Care Act and the Rare Disease Community

Moderator:

Miriam O'Day
Senior Director Public Policy
Alpha-1 Foundation

John Michael O'Brien, MPH, PharmD
Vice President, Public Policy CareFirst
BlueCross BlueShield

11:45 AM – 1:00 PM LUNCH

1:00 – 2:30 PM THE INVESTMENT ENVIRONMENT FOR ORPHAN DRUGS/DEVICES

Session Chair:

David I. Scheer, MS
President, Scheer & Company, Inc.

Jean-Francois Formela, MD
Partner
Atlas Venture
Member Of The Massachusetts General Hospital Research Advisory Council

Rajiv Kaul
Portfolio Manager and Research Analyst
Fidelity Investments

David Mott
General Partner
New Enterprise Associates

Kris H. Jenner, MD, DPhil
Managing Director
Rock Springs Capital

Rogerio Vivaldi, MD, MBA
Senior Vice President,
Head of Rare Diseases
Genzyme Corporation

2:30 – 3:15 PM REFRESHMENT BREAK, NETWORKING & POSTER VIEWING

3:15 – 4:45 PM TRACKS / BREAKOUTS

TRACK 2

Hearing the Voice of the Patient

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

Ronald J. Bartek
President
Friedreich's Ataxia Research Alliance (FARA)

Kay Holcombe, MS
Vice President, Senior Policy Advisor
Genzyme – a Sanofi company

TRACK 3

The International Perspective on Orphan Drugs/Devices

Session Chair:

Geoffrey McDonough, MD
President & CEO
Sobi (Swedish Orphan Biovitrum AB)

Yann Le Cam, MBA
CEO
European Organisation for Rare Diseases EURORDIS

Hans GCP Schikan, PharmD
Chief Executive Officer
Prosensa

Vinciane Knappenberg
Pharmacist
File Manager & Coordinator of the
File Managers
Commission for Reimbursement
of Medicines (CRM)
Directorate
Pharmaceutical Policy
Health Care Department
National Institute for Health and
Disability Insurance (NIHDI)

4:45 – 6:00 PM NETWORKING RECEPTION & POSTER VIEWING

TUESDAY, OCTOBER 8

7:30 – 8:30 AM CONTINENTAL BREAKFAST AND NETWORKING

8:30 – 10:00 AM PLENARY SESSION 2

NORD Initiative on Natural History Studies

Pamela Gavin, MBA
Chief Operating Officer
NORD

Additional Speaker Invited

10:00 – 10:45 AM REFRESHMENT BREAK, NETWORKING & POSTER VIEWING

10:45 AM – 12:00 PM POSTER PRESENTATION SESSION

Session Chair:

Jurgen Venitz, MD, PhD
Professor, Pharmaceutics
Virginia Commonwealth University

12:00 – 1:30 PM LUNCH & POSTER VIEWING

1:30 – 3:00 PM TRACKS / BREAKOUTS

TRACK 2

Patients and Industry: Partnership and Collaboration in Research Funding and FDA Review

Session Chair:

Kari Luther Rosbeck
President and CEO
Tuberous Sclerosis Alliance

Kim Hollander, BS
Executive Director
The Oxalosis & Hyperoxaluria Foundation

Steven L. Roberds, PhD
Chief Scientific Officer
Tuberous Sclerosis Alliance

Judith Prestifilippo, MD
Executive Director
US CD MA Oncology-Rare Diseases
Novartis Pharmaceuticals Corporation

TRACK 3

Paying for Orphan Therapies

Session Chair:

J. Russell Teagarden
Senior Vice President, Medical & Scientific Affairs
National Organization for Rare Disorders

Larry Newfeld
Vice President, Health Operations
Foreign Service Benefit Plan

Scott McKibbin
Principal, McKibbin Group, Inc.
Board Member
University of Illinois at Chicago
Center For Employee Health Studies

Lorelei M. Birk, RPh, MBA
Director, Pharmacy Benefits
1199 SEIU Benefit and Pension Funds

Lynn Rossetto
Vice President
Pharmacy Clinical Accounts
WellPoint Inc.

3:00 – 3:30 PM REFRESHMENT BREAK AND NETWORKING

3:30 – 5:00 PM **ASSURING PATIENT ACCESS TO TREATMENTS**

MODERATOR:

Pamela Gavin, MBA
Chief Operating Officer
NORD

Ross Margulies, JD, MPH
Associate
Foley Hoag LLP

Ruth A. Suter
Senior Director, Market Access and Patient Services
BioMarin Pharmaceutical Inc.

Additional Speaker Invited

WEDNESDAY, OCTOBER 9

7:30 – 8:30 AM **CONTINENTAL BREAKFAST AND NETWORKING**

8:30 – 9:30 AM **PLENARY SESSION 3**

Health Care System of the Future

William Shrank, MD, MSHS
Division of Pharmacoepidemiology and Pharmacoeconomics
Brigham and Women's Hospital and Harvard Medical School

9:30 – 10:00 AM **REFRESHMENT BREAK AND NETWORKING**

10:00 – 11:30 AM **TRACKS / BREAKOUTS**

TRACK 2

Collaboration from Bench to Bedside: How Industry and Patients Can Partner in Rare Disease

MODERATOR:

Kevin Lee, PhD, MBA
Vice President and Chief Scientific Officer
Rare Disease Research Unit
Pfizer Inc

Robert J. Beall, PhD
President and Chief Executive Officer
Cystic Fibrosis Foundation

John E. Bournas
CEO/Executive Director
World Federation of Hemophilia

Robi Blumenstein, LLB, MBA
President
CHDI Management/CHDI Foundation

TRACK 3

Managing Orphan Drug Recalls and Shortages

Pamela M. Williamson, MBA
Senior Vice President, Global Head Regulatory Affairs and Compliance
Genzyme Corporation, A Sanofi Company

Naseem Kabir, MS, RAC
Director, Global Regulatory Affairs
Amgen Inc.

Tiffany House, JD
President, Acid Maltase Deficiency Association (AMDA)
Vice-Chair, International Pompe Association (IPA)

11:30 AM – 1:00 PM **LUNCH**

1:00 – 2:30 PM **PLENARY SESSION 4**

Research Frontiers in Rare Diseases: The Next Opportunities

MODERATOR:

Anne Marie Finley, MS RAC
President
Biotech Policy Group LLC

Bruce C. Trapnell, MD
F.R. Luther Professor of Medicine and Pediatrics
Cincinnati Children's Hospital Medical Center

Salvatore Alesci, MD, PhD
Vice President, Scientific Affairs
Pharmaceutical Research and Manufacturers of America (PhRMA)

Maureen Hoatlin, MBA, PhD
Founding Co-Chair
OHSU Rare Disorders Research Consortium

2:30 – 3:00 PM **REFRESHMENT BREAK**

3:00 – 4:00 PM **CLOSING PLENARY**

The Next 30 Years

SESSION CHAIR:

Wayne L. Pines
President
Regulatory Services and Health Care
APCO Worldwide Inc.

PANELISTS:

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

David I. Scheer, MS
President
Scheer & Company, Inc.

Stephen P. Spielberg, MD, PhD
Editor-in-Chief
Therapeutic Innovation & Regulatory Science
DIA

4:00 PM **CONFERENCE ADJOURNED**