US Conference on Rare Diseases & Orphan Products: The New Era in Health Care
October 7-9 | North Bethesda, MD
The One Conference for all Stakeholders in the Rare Disease/Orphan Product Community

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:
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 (CEUs). Type of Activity: Knowledge.

ACPE Credit Requests:
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As an IACET Authorized Provider, Drug Information Association offers CEUs for its programs that qualify under the ANS/IACET Standard. Drug Information Association is authorized by IACET to offer up to 2.0 CEUs for this program. Participants must attend the entire program in order to be able to receive an IACET statement of credit. No partial credit will be awarded.

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 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 to the educational activity, and (2) discussions of unlabeled or unapproved uses of drugs or medical devices. Faculty disclosures will be disclosed to the program audience (1) any real or apparent conflict(s) of interest related to the content of their presentation and/or to 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.

Unless otherwise disclosed, DIA acknowledges that the statements made by speakers are their own opinion and not necessarily that of the organization they represent, or that of the Drug Information Association. Speakers, agenda, and CE information are subject to change without notice. Recording of any DIA educational material in any type of media, is prohibited without prior written consent from DIA.

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

View DIA’s grievance policy at diahome.org/CE.

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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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.

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## 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-François 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

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**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)

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

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**A Model of Patient, Payer, and Product Developer Collaboration to Support Innovating for Value**

October 30-31 | Washington, DC

This conference will be an important step toward ensuring that patients, payers, and product developers are each contributing to the creation of cost-effective, quality-producing therapies.

Register Now at  
diahome.org/Collaboration

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Co-sponsored by:
**TUESDAY, OCTOBER 8**

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<td><strong>Pamela Gavin, MBA</strong></td>
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<td><strong>Jurgen Venitz, MD, PhD</strong></td>
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<td>Professor, Pharmaceutics</td>
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<td>Virginia Commonwealth University</td>
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<td><strong>Kim Hollander, BS</strong></td>
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<td>The Oxalosis &amp; Hyperoxaluria Foundation</td>
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<td><strong>J. Russell Teagarden</strong></td>
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<td>Senior Vice President, Medical &amp; Scientific Affairs</td>
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<td><strong>Larry Newfeld</strong></td>
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<td><strong>Lorelei M. Birk, RPh, MBA</strong></td>
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<td><strong>Lynn Rossetto</strong></td>
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<td>WellPoint Inc.</td>
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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