INSIDER UPDATE

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

October 7-9, 2013 | North Bethesda, MD

National Center for Advancing Translational Sciences

Insider Update

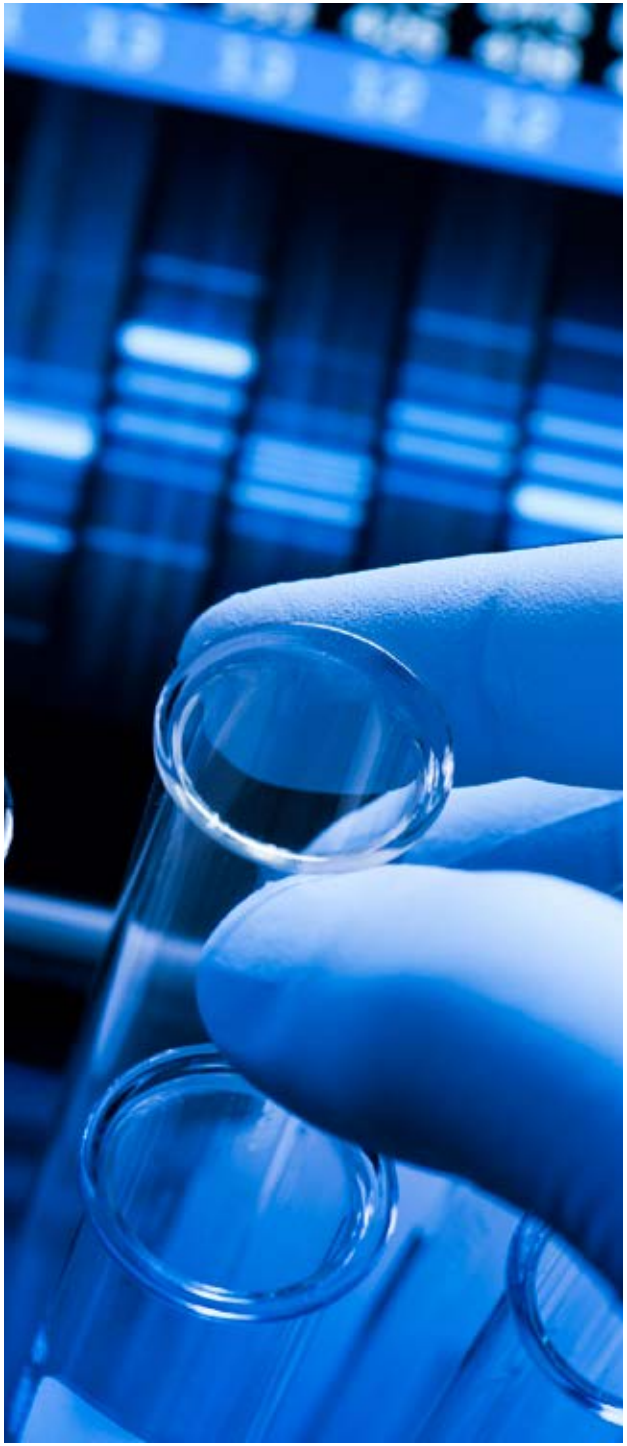
This edition of *Insider Update* provides insights from key industry thought leaders about important topics facing all stakeholders in the Rare Disease/Orphan Product Community, including new initiatives, increased collaboration, and investment opportunities.

All of the experts interviewed will speak at this year's US Conference on Rare Diseases & Orphan Products: The New Era in Health Care.

"All of these initiatives will greatly benefit rare diseases research and eventual new therapies."

"FDA's initiative to collaborate with patient communities is crucial."

Are YOU Ready to Get the Insider Scoop?



#1

Stephen C. Groft, PharmD

Director, Office of Rare Diseases Research
National Center for Advancing Translational Research
National Institutes of Health
U.S. Department of Health and Human Services

Q: What initiatives does National Institutes of Health (NIH) have to advance research into rare diseases and new therapies?

A: "NIH provides significant emphasis on rare diseases research and the development of new treatments across its institutes, centers, and offices. NIH provides nearly $3.6 billion to support about 9,400 rare diseases and 1,600 orphan products research projects. Major projects include the Rare Diseases Clinical Research Network (RDCRN). The RDCRN provides support for the conduct of clinical research pilot projects, natural history studies, training of new investigators, and facilitates the close collaboration of patient advocate organizations with researchers at multiple sites throughout the world. Our office also supports the Global Rare Diseases Patient Registry Data Repository (GRDR), a patient registry and data repository and a publicly accessible database of human biospecimen repositories, the Rare Diseases Human Biospecimens/Biorepositories (RD-HUB).

The Office of Rare Diseases Research (ORDR) supports an extensive scientific conferences program in response to barriers, needs, and scientific opportunities and work on developing collaborative solutions nationally and internationally to establish a research agenda for rare diseases research. There are also a number of major programs at our National Center for Advancing Translational Sciences to expedite the development of new treatments. The Therapeutics for Rare and Neglected Diseases (TRND) program bridges the gap that often exists between a basic research discovery and the testing of new drugs in humans. Bridging Interventional Development Gaps (BrIDGs) makes available certain critical resources needed for the development of new therapeutic agents. Another program employs drug "rescue" and "repurposing," a strategy to enable the investigation of new uses of approved or abandoned drug compounds to advance translational research. The Tissue Chip for Drug Screening initiative aims to develop 3-D human tissue chips that model the structure and function of human organs to assist in the screening of potential compounds for safety and efficacy. All of these initiatives will greatly benefit rare diseases research and eventual new therapies."

#2

Kari Luther Rosbeck

President and CEO
Tuberous Sclerosis Alliance

Q: What impact has the FDA's patient centric initiative had on enhancing collaboration between FDA and the patient community?

A: "From the Tuberous Sclerosis Alliance's perspective, the FDA's initiative to collaborate with patient communities is crucial. The initiative is particularly important for individuals with tuberous sclerosis complex (TSC), which affects around 50,000 people in the United States who really haven't had a seat at the table before. The FDA has clearly seen the importance of seeking direct input from families and individuals impacted by TSC about research and treatments that affect them directly, as well as the types of outcomes our constituents can expect. I certainly hope this new FDA approach will lead to quicker and better informed outcomes that directly benefit people with tuberous sclerosis complex."

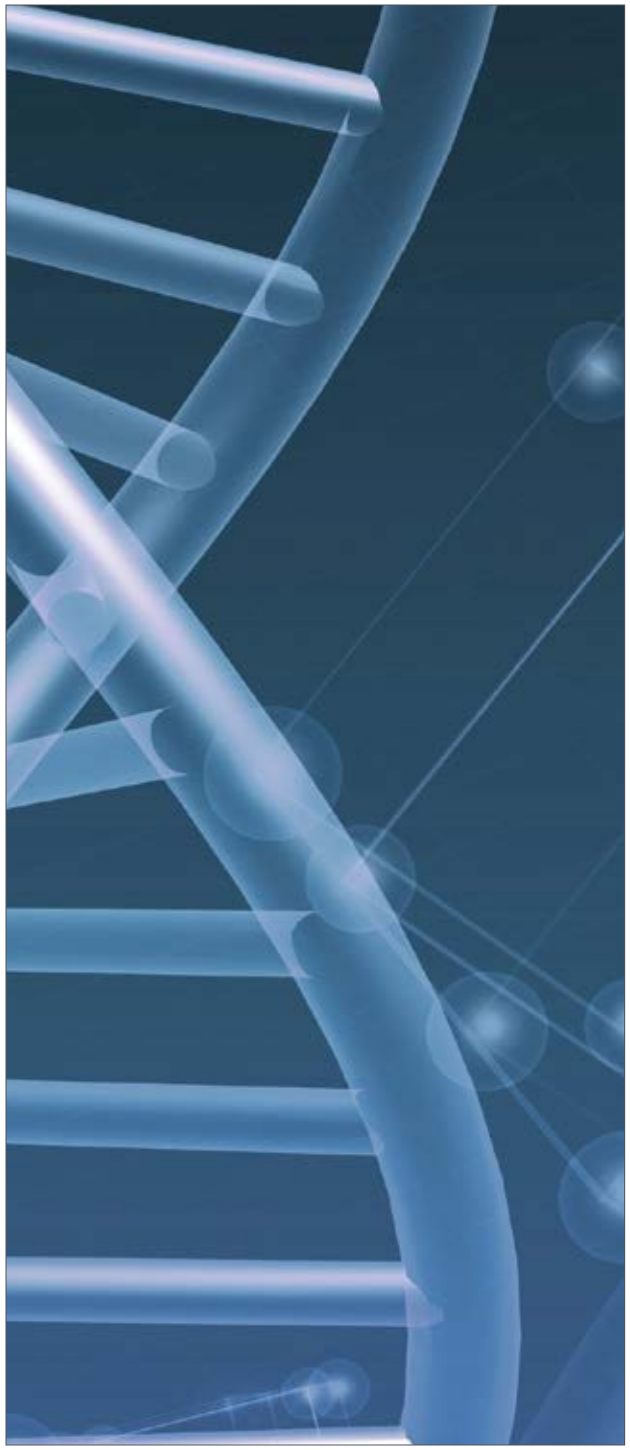
#3

David I. Scheer, MS

Chairman of the Board, Aegerion Pharmaceuticals
President
Scheer & Company, Inc

Q: Are you optimistic about the future for investment opportunities for Orphan Drugs and Devices?

A: "I am optimistic for the future in the Orphan Drug and Device space, although there are some challenges about which we need to be mindful. We have seen major advances over the past several decades in delivering therapeutics which can dramatically change quality of life, and/or extend life for patients afflicted with serious orphan conditions. During this period of time, as well, a business model has evolved which has facilitated access to the risk capital needed to address these indications. Moreover, there has been a sea change in the pharmaceutical industry to focus increasingly on more precise patient populations and what would have been classically regarded as less attractive. Moreover, investors who have provided capital to some of the innovator companies, have been rewarded with attractive rates of return, through share appreciation and ultimate liquidity as a consequence of strategic acquisitions. The question is whether the business model can be sustained, here and in other parts of the world, with a principal component of this question being continued access to these breakthrough agents with the appropriate support from payers."



Are YOU Eager to Know More?

- How will the evolving health care environment affect orphan product development and investment?
- How are the government and private sector addressing the special challenges faced by patients and companies in the new health care environment?
- What are the unique challenges faced by patients with rare diseases and the organizations that represent these patients?
- How do you develop better communication among the investigator, patient industry, investor and government influencers in the rare disease/orphan product community?
- What are the best ways for Rare Disease companies to dialogue with the FDA about important general issues?
- What are the major challenges facing companies that produce Orphan Drugs when a potential shortage of products exists?

There is only ONE place where you can get answers to these questions and more!

You're Invited to the One Conference for all Stakeholders in the Rare Disease/Orphan Product Community

We would like to invite you to this year's US Conference on Rare Diseases & Orphan Products: The New Era in Health Care, being held **October 7-9** in **North Bethesda, MD**, where you'll get the answers to all your questions.

Keynote Speaker:
Bill Corr
Deputy Secretary,
U.S. Department of Health and Human Services

Plenary Session Topics:
- The Affordable Care Act and the Rare Disease Community
- FDA Initiatives on Orphan Products
- NINDS NeuroNext Program
- NORD Initiative on Natural History Studies
- Healthcare System of the Future
- Research Frontiers in Rare Diseases: The Next Opportunities
- The Next 30 Years

Breakout Session Topics:
- The Investment Environment for Orphan Drugs/Devices
- FDA's Orphan Grants Program
- Managing Orphan Drug Recalls and Shortages
- Hearing the Voice of the Patient
- The International Perspective on Orphan Drugs/Devices
- Patients and Industry: Partnership and Collaboration in Research Funding and FDA Review
- Assuring Patient Access to Treatments
- Paying for Orphan Therapies
- Collaboration from Bench to Bedside: How Industry and Patients Can Partner in Rare Disease
- Repurposing: FDA and NIH Perspectives
- NIH Clinical Center-CDER Clinical Trials and Regulatory Training Collaborative
- Plus More

This Year'sThemes:
- Research & Regulation
- Access and Reimbursement
- The Role of the Patient in the Research and Regulatory Process
- The Implementation of the Affordable Care Act

NEW THIS YEAR:
Attendees will have the opportunity to schedule time to meet one on one with key leaders in the rare disease/orphan product community.

REGISTRATION FEES:
Charitable Nonprofit/Academia $800

Government $500

Industry Standard (As of 9/17/13) .....
..... $1865

Industry Early-bird $1665
Early-bird ends September 16!

Register 3, get the 4th FREE!

Patient Organizations & Patients are invited to attend the US Conference on Rare Diseases & Orphan Products at a reduced registration fee of $200. Online registration is not available for this rate. To register, please submit a copy of the registration form and submit the form by fax to +1.215.442.6199.

View Rare Dieases 2012 Slideshow

Networking & Exhibits:
- Networking Reception: October 7 | 4:45-6:00PM
- Tabletop Exhibits: October 7-9

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 healthcare professionals
- Government officials responsible for rare disease research and orphan product oversight

Interested in Exhibiting?
Get the word out about your organization. Submit your application to exhibits@diahome.org today!

We look forward to seeing you in North Bethesda, MD!

REGISTER ONLINE

You Might Also be Interested in:

Meetings:

A Model of Patient, Payer, and Product Developer Collaboration to Support Innovating for Value
October 30-31 | Washington, DC | Co-sponsored with ENGELBERG CENTER for Health Care Reform at BROOKINGS

DIA and the Brookings Institution are collaborating to strengthening communication between health care stakeholder communities. "A Model of Patient, Payer, and Product Developer Collaboration to Support Innovating for Value" will focus on highlighting the perspectives and outcomes most relevant to patient communities, the reimbursement requirements, and innovative payment models of most interest for payer groups, and how both can help inform product developers as they drive toward novel value-based innovation. By exploring these issues and setting an actionable agenda for improving communication moving forward, this conference will be an important first step toward ensuring that patients, payers, and product developers are each contributing to the creation of cost-effective, quality-producing therapies. **Register Today!**

Beginning with the End in Mind – Study Endpoints: Targeting Patient-Centered Outcomes
October 21-23 | North Bethesda, MD

During this workshop, you will gain insight into the tradeoffs and various stakeholder perspectives for developing a study endpoint measurement strategy, including detailed and practical tips for ensuring that measurement tools are adequate to support the targeted objectives with a focus on establishing instrument content validity for the specified clinical trial context of use.

Build your Workshop to Suit your Needs! Come for One, Two, or Three Days! Register Today!

Training Courses:

- Basics of the IND | October 1-3 & 8-10 | 12:00-1:30PM ET | Online
- Basics of the NDA | October 22-24 & 29-31 | 12:00-1:30PM ET | Online
- Preparing for a US FDA Advisory Committee Meeting | November 10 | Philadelphia, PA
- Regulatory Affairs: The IND, NDA, and Postmarketing | November 11-14 | Philadelphia, PA

Registration Open

FDA/EMA Orphan Product Designation and Grant Workshop

Registration is now open for the **FDA/EMA Orphan Products Designation and Grant Workshop** at the FDA White Oak Campus in **Silver Spring, MD**, on **Friday, October 4, 8:30AM–4:00PM**. This one-day workshop is designed to provide valuable information about the EMA and FDA Orphan Drug Designation programs, the FDA Humanitarian Use Device (HUD) Designation program, the FDA Orphan Products Grant program to participants representing pharmaceutical, biotechnology, and device companies, as well as to academics. There will be no registration fee for the workshop, however registration is required.

All participants are welcome to register for the morning sessions which will provide an overview of the EMA and FDA Orphan Drug Designation programs, the FDA Humanitarian Use Device (HUD) Designation Program, the Orphan Products Grant programs. The morning sessions will also be available by webcast.

The afternoon session (no webcast), provides an opportunity for appropriately registered participants to have one-on-one meetings with FDA staff members onsite, to discuss the specifics on how to apply for an orphan product grant, a HUD designation, or orphan drug designation. It also provides for videoconference sessions with EMA staff representatives on EMA orphan drug designation. Participants requesting one-on-one meetings are expected to bring information for at least one candidate orphan drug or device that holds promise for the treatment of a rare disease or condition in order to discuss the processes for putting together an application. In addition, participants in the HUD or orphan drug designation one-one-one sessions are highly encouraged to come prepared with a working draft submission of their particular promising therapy in order to maximize the utility of the one-on-one meetings.

Registration will be limited to 240 participants for the morning session, of which approximately 50 teams (up to 150 participants) may register for the one-on-one sessions.

Questions about the FDA Orphan Product Designation and Grant Workshop?
Contact Eleanor Dixon-Terry Eleanor.Dixon-Terry@fda.hhs.gov.

The FDA/EMA Orphan Product Designation and Grant Workshop is conducted in partnership with the European Organisation for Rare Disease (EURORDIS), Genetic Alliance, and the National Organization for Rare Disorders (NORD).

Stay Connected with DIA

- DrugInfoAssn
- @DrugInfoAssn, #RareDiseases2013
- Drug Information Association (DIA)
- DrugInfoAssn DIA
- DrugInfoAssn

www.diahome.org