2ND

PRIORITY REVIEW VOUCHER SUMMIT

Understand the Regulatory Landscape, Valuation Approaches and How to Successfully Develop a Drug to Secure a Priority Review Voucher

OCTOBER 26-27, 2017 | HYATT REGENCY BOSTON HARBOR | BOSTON, MA

FEATURED SPEAKERS

CONFERENCE CHAIR

David Ridley, *Faculty Director, Health Sector Management, Fuqua School of Business,* **DUKE UNIVERSITY**

Beatrice M. Biebuyck, J.D., MBA, RAC, *Head, Global Regulatory Policy and Intelligence,* **ALEXION PHARMACEUTICALS**

Phillip L. Gomez, Ph.D., *CEO,* **SIGA TECHNOLOGIES, INC.**

Scott Requadt, J.D., MBA, *Managing Director,* **CLARUS**

Khyati Roberts, *Senior Director, Regulatory and Policy Intelligence,* **ABBVIE**

Andrew Robertson, Ph.D., J.D., AVP, *Head of Regulatory Science and Policy, North America,* **SANOFI U.S.**

NEGLECTED TROPICAL DISEASES | RARE PEDIATRIC DISEASES | MEDICAL COUNTER-MEASURES

TOP FIVE REASONS TO ATTEND

- **Review** the various priority review voucher programs and their respective eligibility requirements
- **Hear** from investors and various stakeholders on the strategic and financial value of a PRV based on the current market
- **Gain** a better understanding of the regulatory/legislative landscape and the future of the program
- **Discuss** the calls for change to the "access" and "novelty" requirements in the NTD program and whether PRVs reward true innovators
- **Network** and engage with key stakeholders in the neglected tropical disease, rare pediatric disease, and medical countermeasures space

Dear Colleague,

There are a number of neglected diseases in the world that lack sufficient treatment options due to the affected patient population. Neglected Tropical Diseases (NTDs) usually affect populations in developing countries and are especially common in tropical areas where people have limited access to clean water or hygienic sanitation facilities. Additionally, Rare Pediatric Diseases (RPDs) are classified as diseases that affect less than 200,000 people in the U.S. under the age of 18. Since NTDs rarely affect populations in wealthy nations, and the number of patients with RPDs in the U.S. is so small, there is a lack of investment in the research and development of treatments for these neglected diseases.

The FDA's Priority Review Voucher (PRV) programs are incentives meant to encourage the development of new treatments for diseases that lack commercial viability and do not typically garner interest from companies. These incentives come in the form of special vouchers, which allow a company to have any one of their future drugs reviewed under the FDA's priority review system. Additionally, there is the option to sell a PRV, which can help offset development costs. A number of priority review vouchers have been sold to other drug sponsors for prices ranging from $67.5 million to $350 million. These mechanisms can be extremely beneficial for both companies and patients, leading to a quicker review process and allowing patients with serious conditions to gain access to potentially life-saving or -changing treatments. It also allows companies to market their product sooner and begin recouping their considerable development costs.

Since the 21st Century Cures Act was signed into law, the rare pediatric disease program has been extended to 2020, bringing stability to an otherwise uncertain landscape. The medical countermeasures program is also a new vehicle to incentivize R&D innovation, which can potentially impact the value of current and future vouchers. With added data to reflect on, stakeholders can determine whether forecasted buying and selling prices for PRVs have met market predictions, and whether the program is truly incentivizing research and development in new treatments.

The 2nd Priority Review Voucher Summit is the leading event to understand the benefits and challenges of developing a drug that would be eligible for a PRV. It will provide attendees with the opportunity to network and engage with leaders from pharmaceutical companies, social enterprises, patient groups, government agencies and the investment community to create the best development strategy for your organization/portfolio.

WHO SHOULD ATTEND

This conference is designed for representatives from pharmaceutical and biotechnology companies with responsibilities in the following areas:

- Regulatory Intelligence/Affairs
- Policy/General Counsel
- Strategic Planning
- Strategic/Competitive Intelligence
- Business Development
- Strategic Alliance/Alliance Management
- Licensing
- Due Diligence
- Scientific Assessment
- Research and Development
- Drug Development
- Clinical Operations/ Development
- Rare/Orphan Program Management
- Pipeline/Portfolio Management
- Medical Information/Affairs

This conference is also of interest to:

- Nonprofits/Patient Advocacy Groups
- Social Enterprises
- Strategic/Management Consultants
- Investment Community Members
- Law Firms
- Regulatory Strategy Advisers
- Valuation Experts
- Drug Development Service Providers
- Clinical Research Organizations

VENUE INFORMATION

HYATT REGENCY BOSTON HARBOR

101 Harborside Drive
Boston, MA 02128

To make reservations, please call 888-421-1442 and request the negotiated rate for **ExL's October Meetings**. You may also make reservations online using the following weblink: http://bit.ly/2tR0y2x. The group rate is available until **October 4, 2017**. Please book your room early, as rooms available at this rate are limited.

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THURSDAY, OCTOBER 26, 2017 // MAIN CONFERENCE, DAY ONE

8:00 Registration and Continental Breakfast

8:45 **Chairperson's Opening Remarks**
David Ridley, Ph.D., *Faculty Director, Health Sector Management, Fuqua School of Business,* **DUKE UNIVERSITY**

9:00 **The Current Market for Priority Review Vouchers**
- Review different models for estimating the price of a PRV
- Hear updates on supply and demand effects
- Discuss the recent policy changes
- Forecast future prices for PRVs

David Ridley, Ph.D., *Faculty Director, Health Sector Management, Fuqua School of Business,* **DUKE UNIVERSITY**

9:45 **An Update on the Potential Impact of Legislative Expansion on the Value of Priority Review Vouchers**
- Identify how the PRV program has expanded through legislation and policy
- Describe current proposals for further expansion of the PRV program
- Recognize the potential impact these expansions could have on the resale value of the PRV

Andrew Robertson, Ph.D., JD, AVP, *Head of Regulatory Science and Policy, North America,* **SANOFI US**

10:30 Networking Break

11:00 **Panel: Investor Perspectives on Priority Review Vouchers**
- Describe how the priority review voucher program has changed the way investors look at NTD, RPD and MCM therapeutics
- Highlight key considerations relevant to structuring PRV-based financings
- Identify business issues to resolve and determine whether to opt for early collaboration, joint ventures or outside investment

Scott Requadt, J.D., MBA, *Managing Director,* **CLARUS**
Sandra Panem, Ph.D., *President,* **NEURONETWORKS FUND**

11:45 Luncheon

1:00 **Company Practices: Developing Access Plans for Low- and Middle-Income Countries**
- Explore how the industry can promote access by engaging in key activities across the product lifecycle
- Report on results of the 2016 Access to Medicine Index and 2017 Access to Vaccines Index
- Share company best practices in promoting access to pharmaceuticals in low- and middle-income countries

Clarke B. Cole, *Researcher,* **ACCESS TO MEDICINE FOUNDATION**

1:45 **Panel: Discuss the Calls for "Access" Changes to the PRV Program Including:**
- Examine the available measures of access for new NTD treatments
- Outline potential policy options for the PRV program to increase access
- Explore how the FDA can be burdened by new access requirements
- Discuss non-regulatory strategies to increase the accessibility of NTD treatments

Jeffrey Moe, Ph.D., *Professor of the Practice of Global Health,* **DUKE GLOBAL HEALTH INSTITUTE**
Clarke B. Cole, *Researcher,* **ACCESS TO MEDICINE FOUNDATION**

2:45 Networking Break

3:15 **Stakeholder Perspectives on PRVs: Policy Discussions and Legislative Changes Under 21st Century Cures and FDARA**
- Review history of PRV programs and vouchers awarded to date
- Describe perceived problems and key topics of discussion for reform of voucher programs
- Provide overview of stakeholder perspectives and concerns
- Discuss impact of changes on programs going forward

Scott V. McGoohan, J.D., *Director, U.S. Regulatory Policy and Intelligence,* **VERTEX PHARMACEUTICALS**

4:00 **Panel: How Does the PRV Program Drive Innovation?**
- Discuss considerations and timelines for company investment in RPD and NTD programs
- Explore global regulatory filing strategies to improve access to NTD products
- Examine the influence of the PRV on portfolio design and prioritization

Andrew Robertson, Ph.D., J.D., AVP, *Head of Regulatory Science and Policy, North America,* **SANOFI U.S.**
Scott V. McGoohan, J.D., *Director, U.S. Regulatory Policy and Intelligence,* **VERTEX PHARMACEUTICALS**
Khyati Roberts, *Senior Director, Regulatory and Policy Intelligence,* **ABBVIE**

5:00 Day One Concludes

FRIDAY, OCTOBER 27, 2017 // MAIN CONFERENCE, DAY TWO

8:00 Continental Breakfast

9:00 **Chairperson's Recap of Day One**
David Ridley, Ph.D., *Faculty Director, Health Sector Management, Fuqua School of Business,* **DUKE UNIVERSITY**

9:15 **Case Study: Leverage Innovative Regulatory Pathways to Expedite Development of Drugs for Rare Pediatric Diseases**
- Outline regulatory programs available in the USA and EU and how these pathways can be leveraged across the development lifecycle
- Understand the opportunities, nuances and historical challenges encountered in leveraging the different innovative pathways
- Identify how recent legislative and regulatory changes have helped to address historical challenges in developing and designating drugs for pediatric rare diseases

Beatrice M. Biebuyck, J.D., MBA, RAC, *Head, Global Regulatory Policy and Intelligence,* **ALEXION PHARMACEUTICALS**

10:00 **Case Study: How Rare Disease Foundations and Parents Can Help Provide Information Needed for Achieving a PRV**
- Gain an understanding of the FDA's expectations for receiving rare pediatric disease designation
- Learn how to leverage alternative sources of data for your product
- Bring patients along with you – 21st Century Cures and the PRV
- Assess the value of a PRV for raising funds as a private or public company

Timothy J. Miller, Ph.D., *President and CEO,* **ABEONA THERAPEUTICS**
Michelle Berg, *Vice President, Patient Advocacy,* **ABEONA THERAPEUTICS**

10:45 Networking Break

11:15 **PDP and Non-Profit Models of Collaboration With Industry Partners**
- Gain an overview of how some PDPs and nonprofits work to eradicate and control the spread of rare pediatric and neglected tropical diseases
- Learn how to better collaborate with social enterprises and leverage these partnerships in the rare and NTD space
- Review case examples of successful collaborations with industry partners

If you are interested in leading this session, please contact Zohaib Sheikh at zsheikh@exlevents.com.

12:00 Luncheon

1:15 **Priority Review Vouchers for Medical Countermeasures: Ensuring National Preparedness Through Incentivization**
- Examine and discuss the current landscape for medical countermeasure development and procurement with the U.S. government
- Understand the implications of gaps in funding and preparedness
- Consider solutions for a more streamlined voucher process

Patrick Lucy, *Interim CEO, President and Secretary, and Chief Business Officer,* **PFENEX**

2:00 **TPOXX®: A Case Study in Medical Countermeasure Development and Stockpiling**
- Review the development of a novel antiviral drug targeting orthopoxvirus infections under the FDA animal-rule from pre-clinical through stockpiling sales
- Evaluate the opportunities and challenges of partnered development with the U.S. Government for a critical public health need
- Provide an overview of the business opportunities and challenges for Biodefense products

Phillip L. Gomez, Ph.D., *CEO,* **SIGA TECHNOLOGIES, INC.**

2:45 **Chairperson's Closing Remarks**

3:00 Conference Concludes

Registration Fees for Attending ExL's 2nd Priority Review Voucher Summit:

EARLY BIRD PRICING *Register by September 8, 2017*	**$1,895**
STANDARD PRICING *Register After September 8, 2017*	**$2,095**
ONSITE PRICING	**$2,195**

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To receive a refund or voucher, contact our offices at (201) 871-0474.

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