eXL events

2nd

Innovative Regulatory Pathways SUMMIT

Regulatory Strategies to Expedite Patient Care

January 28-29, 2019

Sheraton Pentagon City Hotel | Arlington, VA

CHAIRPERSON

Dr. Brian Harvey
Executive Vice President Scientific and Regulatory Affairs
GLOBAL LIVER INSTITUTE

HARMONIZE MANUFACTURE READINESS PLANS WITH ACCELERATE DRUG DEVELOPMENT

Usha Ramesh
Executive Director, CMC Regulatory Affairs
PHARMACYCLICS

Dr. Long Wang
Director, Global Regulatory Team Leader, Vaccines/Infectious Disease
MERCK

EXPEDITED DEVELOPMENT OF VACCINES

A COMPARATIVE REVIEW OF THE GLOBAL OPTIONS FOR ACCELERATED DRUG DEVELOPMENT

Jim Wang, Ph.D., MBA
Head of Regulatory Affairs Strategy
SPARK THERAPEUTICS, INC.

Dr. Henrietta Ukwu
Senior Vice President, Head of Global Regulatory Affairs
OTSUKA

CREATE A GLOBAL REGULATORY STRATEGY FOR ACCELERATED DRUG DEVELOPMENT

CASE STUDY: EXPERIENCE WITH BREAKTHROUGH THERAPY DESIGNATION

Martine Zimmermann
Senior Vice President, Global Regulatory Affairs
ALEXION PHARMACEUTICALS, INC.

Mark Stewart
Senior Science Policy Analyst
FRIENDS OF CANCER RESEARCH

PATIENT-FOCUSED DRUG DEVELOPMENT FOR BREAKTHROUGH THERAPY APPLICATIONS

SAKIGAKE DESIGNATION SYSTEM OVERVIEW AND IMPACT ON PORTFOLIO STRATEGY

William K. Sietsema, Ph.D.
Executive Director, Global Regulatory Affairs
CALADRIUS BIOSCIENCES

2nd Innovative Regulatory Pathways SUMMIT

Regulatory Strategies to Expedite Patient Care

Dear Colleague,

Conducting regulatory reviews of novel therapies promptly is essential to improve public health concerns and ensure medical needs are met. Fortunately, there are regulatory mechanisms in place worldwide to accelerate the development of novel treatments. Not only do these pathways ensure that regulatory bodies allocate resources to review and approve therapies as soon as the benefits justify any risks, but they also offer sponsors an opportunity to collaborate with regulatory authorities to identify the most efficient clinical trial design.

Many regulatory authorities throughout the world have implemented pathways that aim to expedite the submission or the review of products that cater to these medical needs. Understanding how to utilize expedited regulatory paths like Breakthrough Therapy Designation, PRIME, and Sakigake Designation is imperative to make an informed decision about the best ways to accelerate drug development.

These regulatory options are available to ensure regulatory bodies allocated the resources to review and approve therapies as soon as the benefits justify any risks. ExL's 2nd Innovative Regulatory Pathways will serve as a platform to discuss and compare the current regulatory pathways that allow for earlier attention to drugs that have promise in treating severe or life-threatening conditions.

Sincerely,

Dario Cavaliere

Dario Cavaliere
Conference Production Director
ExL Events

WHO SHOULD ATTEND

This conference is designed for representatives from pharmaceutical, biotechnology, and medical device companies and advocacy groups with responsibilities in the following areas:

- Regulatory Affairs/Strategy/Operations
- Medical Director
- Clinical Operations/Development/Affairs
- Product Development
- R&D/Drug Development
- Patient Advocacy
- Federal Government Relations
- Strategic Planning
- Competitive Intelligence
- Oncology Program Management
- Rare Disease Program Management
- Pipeline/Portfolio Management
- Medical Information/Affairs

This conference is also of interest to:

- Regulatory Advisors/Service Providers/ Consultants
- Clinical/Contract Research Organizations
- Drug Development Service Providers
- Law Firms
- Preclinical/Analytical Research Organizations
- Strategic/Management Consultants

VENUE INFORMATION

Sheraton Pentagon City
900 S. Orme St. / Arlington, VA 22204

To make reservations, please call 1-800-325-3535 and request the negotiated rate for **ExL's January Meetings**. You may also make reservations online using the following weblink: https://bit.ly/2psJ6NT. The group rate is available until **January 7, 2019**. Please book your room early, as rooms available at this rate are limited.

**ExL Events is not affiliated with Exhibition Housing Management (EHM)/Exhibitors Housing Services (EHS) or any third-party booking agencies, housing bureaus or travel companies. ExL Events is affiliated with event company Questex, LLC. In the event that an outside party contacts you for any type of hotel or travel arrangements, please disregard these solicitations and kindly email us. ExL has not authorized these companies to contact you and we do not verify the legitimacy of the services or rates offered. Please book your guest rooms through ExL's reserved guest room block using the details provided.*

SPONSORSHIP AND EXHIBITION OPPORTUNITIES

Do you want to spread the word about your organization's solutions and services to potential clients attending this event? Take advantage of the opportunity to exhibit, underwrite an educational session, host a networking event or distribute promotional items to attendees. ExL Events will work closely with you to customize a package that will suit all of your needs.

8:00 Open Registration and Continental Breakfast

8:50 **Conference Chair Opening Remarks**

Brian E. Harvey, M.D., Ph.D., *Executive Vice President, Scientific and Regulatory Affairs,* **GLOBAL LIVER INSTITUTE**

9:00 **Considerations about the Future of Accelerated Drug Development in the U.S.**

- Discuss the challenges of expediting drug reviews and approvals while taking the patient experience more into account
- Review the current success of expedited regulatory options established but the FDA and how these successes will lead to organizational changes moving forward
- Compare the eligibility requirements and expedited programs and define how these requirements go into developing organizational structure within regulatory bodies

Brian E. Harvey, M.D., Ph.D., *Executive Vice President, Scientific and Regulatory Affairs,* **GLOBAL LIVER INSTITUTE**

9:45 **A Global Policy Perspective on Harmonization of Regulatory Options for Expedited Drug Development**

- Review global policies to maximize market access in uncertain markets for infectious and/or rare disease products
- Discuss the global impact of utilizing Breakthrough Therapy Designation, PRIME, and Sakigake
- Consider PhRMA's perspective on clinical pathway programs for expedited development

Camille Jackson, *Associate Vice President, Science and Regulatory Advocacy,* **PHRMA**

10:30 Networking Break

11:00 **Create a Global Regulatory Strategy for Accelerated Drug Development**

- Compare type, quality, and quantity of preliminary clinical data required for submission for each of these pathways
- Define and differentiate the types disease states or medical needs that would likely be assisted by each pathway
- Discuss the challenges and benefits of utilizing FDA and EMA expedited review pathways
- Examine the eligibility requirements for breakthrough therapies and PRIME

Dr. Henrietta Ukwu, *Senior Vice President, Head of Global Regulatory Affairs,* **OTSUKA**

11:45 **Spark Therapeutics Case Study: Impact of Breakthrough Therapy Designation on Clinical Trial Design**

- Collaborate with the FDA to identify innovative endpoints to assess the efficacy of Luxturna
- Identify a pathway for generating evidence needed about safety and efficacy in a narrow population
- Hear about the first gene therapy approved by FDA to treat a genetic disease with a breakthrough therapy designation

Jim Wang, Ph.D., MBA, *Head of Regulatory Affairs Strategy,* **SPARK THERAPEUTICS, INC.**

12:30 Luncheon

1:30 **Alexion Case Study: Experience With Breakthrough Therapy Designation**

- Understand the type of data required for BTD eligibility
- Optimize the application process and communication with the FDA prior to receiving designation
- Utilize increased communication to effectively expedite drug development through direct guidance regarding relevant data

Martine Zimmermann, *Senior Vice President, Global Regulatory Affairs,* **ALEXION PHARMACEUTICALS, INC.**

2:15 **Alnylam Case Study: Breakthrough Therapy Designation and Expedited Review of First RNAi Therapeutic**

- Identify clinical endpoints based on disease progression
- Demonstrate non–progression of the disease to gain BTD eligibility
- Discuss regulatory strategies to earn conditional approval to expedite treatment availability
- Understand the options for expedited review for targeted amyloidosis therapies

Sara Nochur, *Senior Vice President Regulatory Affairs,* **ALNYLAM PHARMACEUTICALS**

3:00 Networking Break

3:30 **Harmonize Manufacture Readiness Plans With Accelerate Drug Development**

- Discuss the benefits and impacts of increased communication with the FDA on clinical trial design and CMC plans
- Implement and review CMC plans during early clinical development
- Accelerate manufacturing plans in pace with expedited clinical trial design
- Discuss safety and regulatory considerations for accelerated manufacturing

Usha Ramesh, *Executive Director, CMC Regulatory Affairs,* **PHARMACYCLICS**

Day One / January 28, 2019

4:15 **Breakthrough Therapy Designation: Promoting Efficient Trial Design and Addressing Unmet Clinical Need**

- Review the intent and purpose of breakthrough therapy designation
- Promote patient-focused drug development
- Discuss some of the recent opinion pieces regarding the validity of drugs that go through expedited approvals

Mark Stewart, *Senior Science Policy Analyst,* **FRIENDS OF CANCER RESEARCH**

5:00 Day One Concludes

Day Two / January 29, 2019

8:15 Open Registration and Continental Breakfast

9:15 **Conference Chair Opening Remarks**

Brian E. Harvey, M.D., Ph.D., *Executive Vice President, Scientific and Regulatory Affairs,* **GLOBAL LIVER INSTITUTE**

9:30 **Sakigake Designation System: A Japanese Strategy for Expedited Drug Development**

- Review a history of why the Sakigake Designation System was derived
- Understand the benefits of receiving Sakigake designation
- Discuss global development timelines for Sakigake submission
- Implement strategies to obtain clinical data for Sakigake application
- Incorporate Sakigake/PMDA in global development — engagement, clinical studies, and submission
- Regulatory considerations for applying and maintaining Sakigake designation

William K. Sietsema, Ph.D., *Executive Director, Global Regulatory Affairs,* **CALADRIUS BIOSCIENCES**

10:15 **PRIME: A European Strategy for Priority Medicines and Expedited Drug Development**

- Review a history of why the PRIME System was derived
- Understand the benefits of receiving PRIME compared to the benefits of receiving BTD
- Implement strategies to obtain clinical data for PRIME application
- Incorporate PRIME/EMA in global development — engagement, clinical studies, and submission

11:00 Networking Break

11:30 **Accelerated Review and Development of Vaccines**

- Provide safe and effective vaccines to market quickly for life-threatening diseases and unmet needs
- Consider innovative business model proposals for emerging infectious disease and how they might interact with existing regulatory policies
- Understand concerns over the timely conduct of post-marketing study commitments

Dr. Long Wang, *Director, Global Regulatory Team Leader, Vaccines/Infectious Disease,* **MERCK**

12:15 Luncheon

1:15 **A Comparative Review Of The Global Options For Accelerated Drug Development**

- Discuss the challenges utilizing Breakthrough Therapy Designation and other global options for accelerated development especially when working in emerging markets
- Review the expedited regulatory options in ICH regions
- Compare the eligibility requirements for expedited program and define how these requirements go into developing a global strategy

Lawrence Liberti, *Executive Director,* **CENTRE FOR INNOVATION IN REGULATORY SCIENCE (CIRS)**

2:00 **Utilize Expedited Review Pathways in Precision Medicine**

- Identify clinical endpoints based on available treatments and disease progression
- Demonstrate non–progression of the disease to gain BTD eligibility
- Discuss regulatory strategies to earn conditional approval to expedite treatment availability
- Understand the options for expedited review for targeted oncology therapies

2:45 Conference Concludes

To Register, Click Here or

Phone: 201 871 0474

Fax: 253 663 7224

Email: register@pmaconference.com

Mail: PMA Conference Management POB 2303 Falls Church VA 22042

Registration Fees for Attending ExL's Innovative Regulatory Pathways Summit

EARLY BIRD PRICING *—Register by Friday, December 14, 2018*	$1,895
STANDARD PRICING *—Register After Friday, December 14, 2018*	$2,095
ONSITE PRICING	$2,295

Group Discount Program

Save 25%

Save 25% per person when registering four

For every three simultaneous registrations from your company, you will receive a fourth complimentary registration to the program (must register four at one time). This is a savings of 25% per person.

Save 15%

Save 15% per person when registering three

Can only send three? You can still save 15% off every registration.

Offers may not be combined. Early Bird rates do not apply. To find out more about how you can take advantage of these group discounts, please call 201 871 0474.

❑ Register for the Conference

Name: _____ Title: _____

Company: _____

Dept.: _____

Address: _____

City: _____ State: _____Zip: _____

Email: _____

Phone: _____ Fax: _____

Method of Payment: ❑ Check ❑ Credit Card

Make checks payable to PMA Conference Management.

Card Type: ❑ MasterCard ❑ Visa ❑ Discover ❑ AMEX

Card Number: _____

Exp. Date: _____ CVV:_____

Name on Card: _____

Signature: _____

Please contact me:

❑ I'm interested in marketing opportunities at this event.

❑ I wish to receive email updates on ExL Events' upcoming events.

CONFERENCE CODE: 777519

Media Partners

MEDtube sharing medical knowledge™

pharmaLEADERS News. Resources. Community.

www.PharmCast.com – Pharmaceutical Internet –

pharmaphorum bringing healthcare together

PharmaVOICE

PM360 THE FULL SPECTRUM OF PRODUCT MANAGEMENT

TECHNOLOGY NETWORKS