Achieving Meaningful Regulatory and Clinical Outcomes for Patients: Strategies in Rare Disease Therapy Development

Tutorial: October 6 | Workshop: October 7

Hyatt Regency Bethesda | Bethesda, MD

As of October 2, 2015

PROGRAM CHAIR:

David Schubert
Vice President of Regulatory Affairs
and Quality Assurance
Stealth BioTherapeutics

PROGRAM COMMITTEE:

Jessica Foley, MS, RAC
Head of Regulatory, Rare Diseases
NovusLife Sciences, LLC

Shaghig Palanjian, RHIA, MBA
Head of Research and Development Quality
Assurance and Compliance
Shire Pharmaceuticals

Steven Roberds, PhD
Chief Scientific Officer
Tuberous Sclerosis Alliance

Scott Schliebner
Vice President, Scientific Affairs
Rare Diseases – Federal Programs
PRA Health Sciences

OVERVIEW:

This is a one day, intensive workshop on structuring rare disease therapeutic development programs that integrate patient, regulatory, and clinical perspectives to achieve the most meaningful outcomes for patients. Knowledge will be shared on establishing the foundation of a cohesive regulatory framework for rare disease therapeutic development through scientific planning. We will examine the unique aspects of clinical endpoint and study design for rare disease therapies, and how engagement of patient organizations and regulators can facilitate the development program. A case study on the development of a "mock" therapy will allow you to explore practical issues in the development of the clinical program and in integration of the patient and regulatory perspectives into a comprehensive strategy to meet patient needs.

FEATURED TOPICS:

- Rare Disease Protocol Development
- Addressing the Challenges of Early Patient Engagement
- Making the Most of What FDA Offers
- Regulatory Challenges of Orphan Drug Development

LEARNING OBJECTIVES:

At the conclusion of this workshop, participants should be able to:

- Explain how patient engagement can be successfully interwoven in regulatory and clinical programs
- Describe at least two resources at FDA that can be leveraged to assist companies developing rare disease therapies
- Discuss special considerations for study endpoint identification and development for rare disease therapies
- Discuss the differences in working with FDA on the development of rare disease therapies as compared to therapies for more common diseases
- Discuss the importance of collaboration between key stakeholders in the development of rare disease therapies
- Recognize issues related to the clinical development of new therapies for rare disease populations

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Tutorial:

- ACPE: 3.25 contact hours or .325 CEUs; 0286-0000-15-135-L01-P, Type of activity: Knowledge
- IACET.3 CEUs

Workshop:

- ACPE: 6.5 contact hours or .65 CEUs; 0286-0000-15-119-L01-P, Type of activity: Application
- IACET: .7 CEUs

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RDR Rare Disease Report

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TUESDAY, OCTOBER 6

12:45–1:00PM REGISTRATION

**Please note: Lunch is not provided by DIA for this tutorial.

1:00–4:30PM TUTORIAL: STUDY ENDPOINT DEVELOPMENT IN RARE DISEASES

Participants in this event will join the afternoon sessions of the meeting *"Advancing the Science of Study Endpoints: Seeking Practical Solutions"* for an in-depth view of the unique challenges of selecting and identifying study endpoints for rare disease clinical trials.

Experts will discuss strategies for demonstrating treatment benefit through the selection of optimal study endpoints and review key considerations for development and implementation of underlying assessments. Analysis and interpretation of endpoints will also be addressed. The session will explore adaptation of standard methods of study endpoint development to preserve scientific best practices while accommodating common issues facing rare disease development programs, such as limited knowledge of natural history of the disease, lack of treatment precedent, and absence of existing tools to evaluate treatment benefit. The perspectives of key stakeholder groups will be represented as we explore the many regulatory and clinical challenges, and address commercial considerations.

LEARNING OBJECTIVES:

At the conclusion of this tutorial, participants should be able to:

- Identify the unique issues and execution challenges in setting endpoint strategies and developing study endpoints for rare disease therapeutic products
- Describe scientific best practices for study endpoint development that increase the likelihood of demonstrating treatment benefits for rare disease therapies
- Discuss adaptations to standard methods for study endpoint development that accommodate special circumstances encountered with rare diseases

TARGET AUDIENCE:

This tutorial is for industry, academia, government, vendors, clinicians, and health technology agency professionals involved in setting, executing or evaluating endpoint strategy for drug approval, labeling, promotion, translational science and market access, especially those involved the development of therapies for rare diseases.

Full details can be found by visiting the online agenda.

Please note: registration for this tutorial is separate from the workshop. You may register online at DIAglobal.org

5:00-6:00PM NETWORKING RECEPTION

Reception open to attendees of *"Advancing the Science of Study Endpoints: Seeking Practical Solutions"* and *"Achieving Meaningful Regulatory and Clinical Outcomes for Patients: Strategies in Rare Disease Therapy Development."*

WEDNESDAY, OCTOBER 7

7:00AM-4:30PM **REGISTRATION**

7:00-8:00AM **CONTINENTAL BREAKFAST**

8:00-8:15AM WELCOME AND OPENING REMARKS

David Schubert
Vice President of Regulatory Affairs and Quality Assurance
Stealth BioTherapeutics

8:15–8:30AM CASE STUDY PRESENTATION

SESSION CHAIR:

David Schubert
Vice President of Regulatory Affairs and Quality Assurance
Stealth BioTherapeutics

In this "mock" case study for the treatment of a rare autoimmune disease, we will explore many of the very real and challenging issues in a rare disease development program. We will refer to and interactively discuss this case throughout the workshop as experts and participants address:

- Complex designs of rare disease clinical trials
- Recruitment challenges
- Ways to effectively engage with regulators
- Ways to involve patients and advocacy groups throughout the clinical development process
- Ways to engage "all" stakeholders to get drugs to patients faster
- Regulatory resources and programs available to facilitate development
- And more

8:30-9:30AM SESSION 1: ORPHAN DRUG DEVELOPMENT AND ITS REGULATORY CHALLENGES

SESSION CHAIR:

Jessica Foley, MS, RAC
Head of Regulatory, Rare Diseases
NovusLife Sciences, LLC

This session will focus on addressing the unique regulatory complexities and challenges specific to orphan drug development. Experts will examine the industry's perspective on many important issues, including, heterogeneity, natural histories and progression, and endpoints. FDA representatives will provide key information about programs available to expedite the development of orphan products. This session will close with an interactive roundtable discussion that will demonstrate the importance of cooperation and collaboration of all key stakeholders.

Regulatory Challenges of Orphan Drug Development

Jessica Foley, MS, RAC
Head of Regulatory Services
NovusLife Sciences, LLC

Making the Most of What FDA Offers

Larry Bauer, RN, MA
Regulatory Scientist
CDER, FDA

Gayatri Rao, MD, JD
Director
Office of Orphan Products Development
FDA

Panel Discussion

Session Participants

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

10:00-11:30AM SESSION 2: CLINICAL DEVELOPMENT FOR RARE DISEASES

SESSION CO-CHAIRS:

Shaghig Palanjian, RHIA, MBA
Head of Research and Development Quality
Assurance and Compliance
Shire Pharmaceuticals

Scott Schliebner
Vice President, Scientific Affairs
Rare Diseases – Federal Programs
PRA Health Sciences

This session will focus on emerging issues related to the clinical development of new therapies for rare disease populations.

Rare Disease Protocol Development

David Whiteman, MD, FAAP, FACMG
Global Clinical Development Lead
Shire Pharmaceuticals

Leveraging Informatics to Accelerate Rare Disease Clinical Development

Scott Schliebner
Vice President, Scientific Affairs
Rare Diseases – Federal Programs
PRA Health Sciences

Clinical Logistics for Rare Disease Trials

Larry Blankstein, PhD
Independent Consultant
Former Senior Director, Clinical Development
Genzyme, a Sanofi Company

Overview of FDA Draft Guidance for Industry: Rare Diseases - Common Issues in Drug Development

Jonathan Goldsmith, MD, FACP
Associate Director, Rare Diseases Program
OND/CDER, FDA

Interactive Panel Discussion

Jonathan Goldsmith, MD, FACP
Associate Director, Rare Diseases Program
OND, CDER, FDA

David Whiteman, MD, FAAP, FACMG
Global Clinical Development Lead
Shire Pharmaceuticals

Scott Schliebner
Vice President, Scientific Affairs
Rare Diseases – Federal Programs
PRA Health Sciences

Larry Blankstein, PhD
Independent Consultant
Former Senior Director, Clinical Development
Genzyme, a Sanofi Company

11:30AM-12:30PM NETWORKING LUNCHEON

12:30-2:00PM SESSION 3: IMPACT OF EARLY PATIENT ENGAGEMENT

SESSION CHAIR:

Steven Roberds, PhD
Chief Scientific Officer
Tuberous Sclerosis Alliance

In this session we will address both the value of and the challenges in engaging patients early-on in the clinical development process. Experts will review the specific concepts and principles relating to overcoming barriers and demonstrate how patient engagement can be successfully interwoven in regulatory and clinical programs.

Addressing the Challenges of Early Patient Engagement

Tiffany House, JD
President
Acid Maltese Deficiency Association

Putting it All Together

Richard Klein
Director, Patient Network Program
OC, FDA

Interactive Panel Discussion

Tiffany House, JD
President
Acid Maltese Deficiency Association

Molly White
Executive Director
Myotonic Dystrophy Foundation

Joel Beetsch, PhD
Vice President, Patient Advocacy Group
Celgene Corporation

Richard Klein
Director, Patient Network Program
OC, FDA

John Campbell
Senior Director, Clinical Development and Regulatory Sciences
Stealth BioTherapeutics

2:00-2:30PM REFRESHMENT AND NETWORKING BREAK

2:30-4:00PM **SESSION 4: ROUNDTABLE DISCUSSION: CREATING SOLUTIONS FOR COMPLEX CHALLENGES IN RARE DISEASE DEVELOPMENT**

SESSION CHAIR:

David Schubert
Vice President of Regulatory Affairs & Quality Assurance
Stealth BioTherapeutics

This interactive session is centered on discussing the various challenges in developing a clinical program in rare disease therapy with specific reference to the case study presented in the beginning of the program. Under the direction of moderators, participants will provide their perspective, experience and possible solutions to overcome the challenges.

These are the topics that will be discussed during the roundtable session (1 topic per table).

Target Product Profile – Considerations in Development

MODERATOR:

Joel Beetsch, PhD
Vice President, Patient Advocacy Group
Celgene Corporation

Integrating Clinical Outcome Measures

MODERATOR:

Linda Deal, MS
Head of Patient Centered Outcomes Measurement
Pfizer Inc

It Takes a Village

MODERATOR:

David Schubert
Vice President of Regulatory Affairs & Quality Assurance
Stealth Biotherapeutics

Registries

MODERATOR:

David Whiteman, MD, FAAP, FACMG
Global Clinical Development Lead
Shire Pharmaceuticals

4:00-4:30PM **CONCLUDING WORKSHOP DISCUSSION**

MODERATORS:

David Schubert
Vice President of Regulatory Affairs & Quality Assurance
Stealth BioTherapeutics

Scott Schliebner
Vice President, Scientific Affairs
Rare Diseases – Federal Programs
PRA Health Sciences

During this concluding discussion, we will address key questions and discuss important takeaways.

4:30PM **WORKSHOP ADJOURNED**