2nd Rare Disease Collaboration Summit

Cultivate Partnerships with Patient Advocates to Leverage Rare Disease Therapy Processes and Developmental Success


SPOTLIGHT FACULTY MEMBERS

Rino Aldrighetti, CEO, PULMONARY HYPERTENSION ASSOCIATION

Diane Berry, Vice President, Global Health Policy and Government Affairs, SAREPTA THERAPEUTICS

Carrie Burke, Director, Alliance Development, SHIRE PHARMACEUTICALS

Pat Furlong, CEO, PARENT PROJECT MUSCULAR DYSTrophy

Kari Rosbeck, CEO, TUBEROUS SCLEROSIS ALLIANCE

Nora Yang, Portfolio and Project Management, Strategic Operations, NATIONAL CENTER FOR ADVANCING TRANSLATIONAL SCIENCES, NATIONAL INSTITUTES OF HEALTH

SPEAKERS AND CASE STUDIES WILL PROVIDE YOU WITH THE TOOLS TO:

• Create substantial relationships with healthcare and patient advocates to further facilitate clinical trials
• Uncover typical development timelines for orphan drugs prior to the commercialized stage
• Generate protocols surrounding patient reimbursement requirements and necessities
• Combine resources to successfully apply proposed phenotypes to niche patient groups
• Understand clinical study pipelines to accommodate for orphan drug timelines

SUMMIT FEATURES:

» 5 Keynotes
» 8 Rare Disease Advocacy Specialists
» 6 Orphan Drug Development Companies

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Dear Colleague,

In the United States, any disease that affects fewer than 200,000 people is considered a rare disease. In such a small patient pool, completing a clinical trial can become very troublesome — and costly. Orphan therapeutics have consequently been classified as the most expensive pharmaceutical products of all time. If you are struggling with timing pipelines, budgeting and trial efficiency, the 2nd Rare Disease Collaboration Summit, taking place July 15-16, 2015 in Philadelphia, PA, is the answer to all of your needs.

FDA regulatory approval is obviously the end goal for all stakeholders in the industry, but a number of bridges need to be crossed prior to this. During the developmental stage, the most important piece of the puzzle is the patient. Ensuring happiness through the cultivation of relationships between the patient and support group must be a top priority. Proper use of data registries and targeting of new indications for orphan drugs are also key steps for this process. Reimbursement for gene therapies is another hurdle to jump, and this is absolutely crucial for the continued efficacy of drugs.

This conference focuses on addressing the above obstacles through the discussion of genomics, drug development and enhancement, patient involvement, rare disease diagnosis, and product approval. With representatives from patient advocacy and support groups, pharmaceutical companies, nonprofit organizations, and many other entities, the 2nd Rare Disease Collaboration Summit is the best networking event of the year, and you absolutely cannot miss out. Join us to learn from the best and return to your organization with a playbook of strategies to ensure success.

We can’t wait to welcome you to Philadelphia in July!

Sincerely,

Brendan M. Weiss

Brendan M. Weiss
Conference Production Director
2nd Rare Disease Collaboration Summit
ExL Events
8:00 Registration Opens and Continental Breakfast
9:00 Co-Chairpersons’ Opening Remarks
Ron Bartek, President, FRIEDREICH’S ATAXIA RESEARCH ALLIANCE
Beth White, Assistant Vice President, Rare Disease, PFIZER

**PATIENT/PHARMA ALLIANCES**
9:15 The Cost of Innovation and Productive Conversation Across All Rare Disease Medicine Stakeholders
- Surfacing effective communications and understandings about pricing and transparency
- Building a value proposition of rare disease medicines and generating a sustainable healthcare system
- Creating a win-win-win environment for patients, payers and industry
Beth White, Assistant Vice President, Rare Disease, PFIZER

9:45 Enhance Community Knowledge and Initial Involvement in Orphan Development for the Tuberous Sclerosis Alliance
- Educating and engaging regional communities in early development clinical studies
- Discussing the natural history database for orphan drug developments
- Understanding the five key pillars to drive research forward
Kari Rosbeck, CEO, TUBEROUS SCLEROSIS ALLIANCE

10:30 Networking and Refreshment Break
11:00 Build Collaborations Between Patient Advocacy Groups and Healthcare Professionals
- Creating substantial relationships with healthcare and patient advocates to further facilitate clinical trials
- Building alliances with insurance companies to leverage help during clinical preparation
- Utilizing the experience of patient advocacy experts to properly collaborate with industry regulators
Ted Buckley, Ph.D., Head of US Government Relations and Public Affairs, SHIRE

11:30 Alternative Models to Discovering and Developing Orphan Therapeutics
- Uncovering the importance of cross-community engagement in streamlining orphan drug R&D (patients, physicians and advocacy groups)
- Leveraging the biotechnological advantage in the rare disease space to get appropriately priced drugs to market quicker
- Cultivating new uses for old drugs and understanding the wealth of under-exploited orphan potential in existing chemical entities
Deborah O’Neil, CEO and CSO, NOVABIOTICS

12:00 Luncheon
1:15 Partner with Patient Advocacy Groups to Collaborate and Achieve Success Over Orphan Therapies
- Creating disease-specific surveys to improve patient involvement
- Using innovative methodologies to identify potential candidates for orphan clinical studies
- Optimizing handling techniques for large rare disease patient registries
Catie Olson, Director, Coordination of Rare Diseases, SANFORD RESEARCH

“ Inspiring and passionate presenters!”
- Director, PREMIER RESEARCH

1:45 Patient Development Enhancement Techniques for Friedreich’s Ataxia Research Alliance
- Understanding actions advocacy groups and patients can take to accelerate development of orphan treatments
- Bringing patient viewpoints to the attention of pharmaceutical companies, the NIH and the FDA
- Developing instrumental partnerships in the rare disease industry
Ron Bartek, President, FRIEDREICH’S ATAXIA RESEARCH ALLIANCE

2:15 A New Framework for Patient and Academic Collaboration in Rare Diseases
- Expanding roles in rare disease medicine development, keeping the importance of collaboration in mind
- Partnering with patients to surface innovative programs supporting the patient journey from diagnosis to treatment
- Outlining new platforms in the rare disease space
Kya Rosow, International Therapeutic Area Lead, Rare Diseases, PFIZER

2:45 Networking and Refreshment Break
3:15 Optimize Patient Development, International Management and Advisory Boards
- Analyzing and applying patient input for clinical study design
- Understanding patient advocacy during all stages of a clinical trial
- Discussing regulatory submission and early access to investigational drugs
Jayne Gershihowitz, Vice President, Patient Advocacy and Public Policy, AMICUS THERAPEUTICS, INC.

3:45 Discuss Approaches to Personalized Rare Disease Therapeutics
- Using yeast, worm and fly models in highly scalable assays to study drug development
- Designing model assay studies to enhance therapeutic development
- Learning strategies to scale research and applying analytics to orphan therapeutics
Nina DiPrimio, Organism Engineer, PERLSTEIN LABS

4:15 Regulatory Strategies for Orphan Filing and Drug Indications
- Understanding the difficulties surrounding FDA submission and approval for orphan drugs
- Addressing approval challenges for rare diseases with the FDA and EMA
- Learning overall improvement strategies for driving efficiency in orphan drug study pipelines
Stefan Proniuk, Ph.D., Chief Development Officer, ARNO THERAPEUTICS

4:45 Conclusion of Day One

“Very informative and great insight!”
- Pediatric Co-Chair, THE MASTOCYTOSIS SOCIETY, INC.
8:00  Registration Opens and Continental Breakfast

8:15  Co-Chairpersons’ Recap of Day One
Ron Bartek, President, FRIEDREICH’S ATAXIA RESEARCH ALLIANCE
Beth White, Assistant Vice President, Rare Disease, PFIZER

8:30  A Paradigm of Cooperation Between a Patient Advocacy Group and a Biotechnology Firm in Setting the Stage for Drug Development
- Balancing the needs of a sponsor and a patient advocacy group in the development and ownership of a patient registry while avoiding conflict of interest
- Outlining communication expectations in populating a registry and in developing preclinical data
- Leveraging the change in landscape upon the acquisition of a sponsor
Brett Kopelan, M.A., CEO, debra of America, Chairman of the Board of Directors, NORD

9:00  The Orphan Drug Financial Advantage in Translational Medicine
- Uncovering outcomes from NIH/NCATS rare disease therapeutic development programs
- Exploring recent business models for financing orphan disease research
- Identifying the challenges and advantages in orphan drug developmental processes
Nora Yang, Portfolio and Project Management, Strategic Operations, NATIONAL CENTER FOR ADVANCING TRANSLATIONAL SCIENCES, NATIONAL INSTITUTES OF HEALTH

9:30  Accelerate Approval for Development Timelines for True Orphan Drugs
- Enhancing protocols to gain approval behind skipping Phase III and fast-tracking Phase II in an oncology trial
- Identifying processes to cut trial pipelines in half with CMC obstacles
- Building solutions for product development and commercialization challenges
Stefan Proniuk, Ph.D., Chief Development Officer, ARNO THERAPEUTICS

10:00  Group Multiple Rare Therapeutics to Streamline Drug Delivery Processes
- Outlining innovative techniques to study the genetic makeup of rare diseases
- Utilizing analytics to determine rational strategies for grouping orphan therapeutics
- Understanding the importance of grouping rare developments in order to fast-track drug commercialization
Philip J. Brooks, Ph.D., Division of Clinical Innovation, NATIONAL CENTER FOR ADVANCING TRANSLATIONAL SCIENCES, NATIONAL INSTITUTES OF HEALTH

10:30  Networking and Refreshment Break

11:00  Newborn Screening Techniques for Orphan Drug Development
- Outlining the process of adding new screens on the state and federal levels
- Harnessing practical implementation and preparation for early development of rare therapeutics
Carrie Burke, Director, Alliance Development, SHIRE PHARMACEUTICALS

11:30  Co-Chairpersons’ Recap of Day Two

• Harnessing solutions
• Outlining innovative techniques
• Utilizing analytics to determine rational strategies
• Understanding the importance of grouping rare developments in order to fast-track drug commercialization

11:30  Analyze and Cultivate Data to Understand the Benefit Risk of Duchenne Clinical Development
- Examining stroke production and quantification to expand the benefit risk to disengaged patients
- Collaborating with the FDA to develop guidance and approach protocols
- Generating proactive and professional models on patient development interaction
Pat Furlong, CEO, PARENT PROJECT MUSCULAR DYSTROPHY

12:00  Luncheon

1:15  Catalyzed Development of Therapeutics for Rare Diseases with Public-Private Partnerships
- Connecting basic science and human medicine with translational science
- Exploring public-private collaborative models for NIH/NCATS rare disease therapeutics programs
- Outlining the preclinical development of therapeutics for rare diseases
Elizabeth Ottinger, Project Management, NATIONAL CENTER FOR ADVANCING TRANSLATIONAL SCIENCES, NATIONAL INSTITUTES OF HEALTH

1:45  Genetic Screening, Trial Design and the Importance of Genetic History Collection
- Evaluating receivable benefits from orphan therapies and the natural baseline of risks that patients can be exposed to
- Understanding disease progression and relative modifying treatments
- Discussing big data screening through tissue database analytics
Diane Berry, Vice President, Global Health Policy and Government Affairs, SAREPTA THERAPEUTICS

2:15  Networking and Refreshment Break

2:45  Changing the History of a Rare Disease for the Pulmonary Hypertension Association
- Utilizing fundamental topics to mature the field of pulmonary hypertension
- Growing related registries and accreditation programs for study centers
- Building a community to enhance and fast-track the treatment approval process
Rino Aldighetti, CEO, PULMONARY HYPERTENSION ASSOCIATION

3:15  Data Repository and Resource Development for Rare Diseases
- Integrating patient data from different registries and mapping them to the patient data repository program
- Involving all important stakeholders in an overall goal to support the patient at hand
- Working with patient advocacy groups and families to ensure engagement throughout drug development
Yaffa Rubenstein, Ph.D., Director of Patient Resources for Clinical and Translational Research at the Office of Rare Diseases Research, NATIONAL CENTER FOR ADVANCING TRANSLATIONAL SCIENCES, NATIONAL INSTITUTES OF HEALTH

3:45  Co-Chairpersons’ Closing Remarks
Ron Bartek, President, FRIEDREICH’S ATAXIA RESEARCH ALLIANCE
Beth White, Assistant Vice President, Rare Disease, PFIZER

4:15  Summit Concludes
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Patient Collaboration  Data Registries  New Treatment Indications  Reimbursement  Regulatory Approval