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

Event Sponsors:

NovaBiotics

GLOBAL CARE CLINICAL TRIALS

onevoice

To Register Call 866-207-6528 or Visit www.exlevents.com/rare
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

“Excellent choice of speakers. Brought together different perspectives.”
— Senior Director, Business Development,
PREMIER RESEARCH

“Great introduction and discussion!”
— Business Development,
DURBIN

Venue Information
Hilton Philadelphia at Penn’s Landing
201 South Columbus Boulevard | Philadelphia, PA 19106

If you require overnight accommodations, please call 1-800-HILTONS and request the negotiated rate for ExL’s July Meetings. You must book your room by June 24, 2015 to be eligible for the discounted rate. Please book your room early, as rooms available at this rate are limited.

To Register Call 866-207-6528 or Visit www.exlevents.com/rare
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

10:30 Networking and Refreshment Break

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

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
Kyra Rosow, International Therapeutic Area Lead, Rare Diseases, PFIZER

3:15  Patient-Centric Clinical Trials: Taking Study Visits to the Patients
• Exploring the use of an innovative, patient-centric approach to reduce patient burden by conducting at-home study visits
• Learning from case studies using ambulant care services to promote patient participation
• Discovering win-win benefits for all stakeholders involved
Gail Adinamis, Founder and CEO, GLOBALCARE CLINICAL TRIALS, LTD.

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

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  Conclusion of Day One

Case Study

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

4:15  Conclusion of Day One

Case Study

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

To Register Call 866-207-6528 or Visit www.exlevents.com/rare

Day 1 | Wednesday, July 15, 2015

"Very informative and great insight!"
-- Pediatric Co-Chair,
THE MASTOCYTOSIS SOCIETY, INC.

"Inspiring and passionate presenters!"
-- Director, PREMIER RESEARCH
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

REGULATORY AND REIMBURSEMENT DEVELOPMENTS

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

9:30 Navigate the Transition from Big Pharma to Small Biotech in Medical Affairs: What to Expect When You’re Defecting
   • Uncovering key career considerations in ‘going small’
   • Managing expectations during the transition to smaller biotech
   • Assessing the newfound situation and planning for continued success
   W. Richey Neuman, MD, MPH, FACP, Vice President, Medical Affairs, HORIZON PHARMACEUTICALS

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 Case Study: A Rare Disease Support Program for Data Repository and Resource Development
   • Uncovering the development, engagement, demonstration and dissemination of tools and resources to establish patient registries
   • Integrating data from patient registries, Electronic Health Records (EHR) and other data sources in a standardized manner in a large database accessible for query
   • Engaging all stakeholders in the overall goal to support the patient advocacy groups to accelerate various biomedical studies in drug development
   Yaffa Rubinstein, 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

12:00 Luncheon

1:15 Case Study: 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

1:45 Genetic Screening, Trial Design and the Importance of Natural 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 Aldrighetti, CEO, PULMONARY HYPERTENSION ASSOCIATION

3:15 Case Study: 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

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
GROUP DISCOUNT PROGRAMS:
*Offers may not be combined. Early Bird rates do not apply.*

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

SAVE 15%
Can only send three? You can still save 15% off of every registration. To find out more on how you can take advantage of these group discounts, please call 212-400-6240.

Payment:
Make checks payable to ExL Events, Inc. and write code C614 on your check. You may also use Visa, MasterCard, Discover or American Express. Payments must be received in full by the conference date. Any discount applied cannot be combined with any other offers and must be paid in full at the time of order. Parties must be employed by the same organization and register simultaneously to realize group discount pricing options.

**Please Note: There will be an administrative charge of $300 to substitute, exchange and/or replace attendance badges with a colleague occurring within five business days of any ExL conference.**

Cancellation and Refund Policy:
If you need to cancel your registration for an upcoming ExL event, please note the following policies derived from the Start Date of the event:

- Four weeks or more: A full refund (minus a $295 processing fee) or a voucher to another ExL event valid for 12 months from the voucher issue date.
- Less than four weeks: A voucher to another ExL event valid for 12 months from the voucher issue date.
- If you cancel within five business days or at any time after receiving the conference documentation, the voucher issued will be $395 less.
- To receive a refund or voucher, please fax your request to 888-221-6750.

Credit Vouchers:
Credit Vouchers are valid for 12 months from date of issue. Credit Vouchers are valid toward one (1) ExL Event of equal or lesser value. If the full amount of said voucher is not used at time of registration, any remaining balance is no longer applicable now or in the future. Once a Credit Voucher has been applied toward a future event, changes cannot be made. In the event of cancellation on the attendees’ behalf, the Credit Voucher will no longer be valid.

Substitution Charges:
There will be an administrative charge of $300 to substitute, exchange and/or replace attendee badges with a colleague occurring within five business days of the conference.

ExL Events reserves the right to cancel any conference it deems necessary and will not be responsible for airfare, hotel or any other costs incurred by registrants.

ExL Events’ liability is limited to the conference registration fee in the event of a cancellation and does not include changes in program date, content, speakers or venue.

*The opinions of ExL speakers do not necessarily reflect those of the companies they represent or ExL Events, Inc.

Please Note: Speakers and agenda are subject to change without notice. In the event of a speaker cancellation, significant effort to find a suitable replacement will be made.

Media Partners:

Sponsorship and Exhibiting Opportunities
Do you want to spread the word about your organization’s solutions and services to potential clients who attend this event? Take advantage of the opportunity to exhibit, present an educational session, host a networking event or distribute promotional items to attendees. ExL works closely with you to customize a package that suits all of your needs.

To learn more about these opportunities, contact Brendan Jordan, Business Development Manager, at 917-258-5154 or bjordan@exlevents.com.

Questions? Comments?
Do you have a question or comment that you would like to be addressed at this event? Would you like to get involved as a speaker or discussion leader?

Please email Conference Production Director Brendan Weiss at bweiss@exlevents.com.
Cultivate Partnerships with Patient Advocates to Leverage Rare Disease Therapy Processes and Developmental Success

To Register Call 866-207-6528 or Visit www.exlevents.com/rare


2nd Rare Disease Collaboration Summit

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

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

Five Ways to Register:

ExL Events, Inc.
Mail: 494 8th Avenue, 4th Floor
New York, NY 10001
Phone: 866-207-6528
Online: www.exlevents.com/rare
Email: registration@exlevents.com
Fax: 888-221-6750

YES! Register me for this conference!
Name: ________________________________
Title: ________________________________
Company: ____________________________
Dept.: ________________________________
Address: ______________________________
City: ________________________________
State: __________________ Zip: ________
Email: ________________________________
Phone: ________________________________
Fax: __________________________________