Agenda

May 1–3, 2023

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Overview

The RARE Drug Development Symposium, hosted by Global Genes and the Orphan Disease Center of the University of Pennsylvania, equips advocates with the knowledge, skills and connections they need to advance therapy development for their communities.

Using case studies and real-world examples, advocates will discover how they can:

- ***Leverage the power of data to drive research***
- ***Expand research opportunities using new technologies.***
- ***Collaborate to advance research strategy and fill critical gaps***
- ***Give patients a voice through engagement with regulatory agencies***

The unique format of this two-day, live event allows participants *to* learn from rare disease leaders in ***panel discussions***, share experiences in ***workshops with peers*** and get personalized answers at **one-to-one *expert office hours*.**

Location and Times

- Monday, May 1, 2023 – Wednesday, May 3, 2023
- Sheraton Philadelphia Downtown, 201 N 17th St, Philadelphia, PA 19103

Who will attend

- Patient advocacy group leaders
- Rare disease patients
- Members of the rare disease community who want to learn more about the drug development process
- Clinicians and researchers focused on rare disease therapeutic targets

**Please note the following agenda is subject to change*

DAY 1	MONDAY May 1, 2023	6:30 pm - 8:00 pm

6:30 – 8 pm **Welcome Reception**
Horizon Ballroom

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DAY 2	TUESDAY May 2, 2023	8:00 am - 6:00 pm

8:00 am **Breakfast**
Liberty Ballroom C

9:00 – 10:00 am **Welcome:** Charlene Son Rigby, CEO, Global Genes
Liberty Ballroom D **Plenary Session**: Charting the Path to Treatments

Like rare diseases, each path towards discovery and development of rare disease therapeutics is different. To kick off this year's RARE Drug Development Symposium, our hosts will guide us through a number of current drug development journeys through conversations with a cohort of advocates each at a different stage of the process. Learn the multiplicity of pathways and approaches advocates are currently using to get closer, step by step, to approved treatments and therapeutics.

Moderator: **Charlene Son Rigby**, CEO, Global Genes

Panelists:

Simon Frost, CEO, Tiber Capital Group
Brett Kopelan, Executive Director, debra of America

Leah Schust Myers, Founder and Executive Director, FamilieSCN2A Foundation

Additional resources on this topic:
Understanding Pathways to Accelerate Therapeutics
Understanding Drug Development

10:00 –10:15 am
Liberty Ballroom D

The RARE Research Roadmap
Charlene Son Rigby, CEO, Global Genes
Deborah Requesens, Ph.D., Director, JumpStart, Orphan Disease Center, University of Pennsylvania
Additional resources on this topic:
Rare Research Roadmap Toolkit: The Road from Bench to Bedside

10:15 – 11:00 am
Liberty Ballroom D

Closer Look Panel 1: Understanding Data for Basic Research

Building a firm foundation for your research program helps avoid wasted time, money, and effort. Why is the data collected and produced by basic research important? What role does it play in driving research strategies? How can you evaluate the reliability and validity of basic research data? How can you identify the remaining research to be done and how can patient advocacy best support this part of the drug development process?

Moderator: **Yssa DeWoody**, Ph.D., Co-founder, Director of Research, and Treasurer, Ring14 USA

Panelists:

Maya Chopra, MBBS, FRACP, Director, Translational Genomic Medicine, Boston Children's Hospital

Eric Marsh, M.D., Ph.D., Clinical Director, Orphan Disease Center, University of Pennsylvania

Rodney Samaco, Ph.D., Assistant Professor, Baylor College of Medicine

Cara Weismann, Ph.D., Director of the MPS Program of Excellence, Orphan Disease Center, University of Pennsylvania

Additional resources on this topic:
Understanding the Biology of Rare Diseases
Preclinical Modeling: What Advocates Need to Know About Animal and Cell Models

11:00 – 11:15 am **Coffee Break**
Liberty Foyer
Mezzanine Foyer

11:15 – 12:15 pm **Small Group Action Workshop**: Bridging Basic Research Gaps
Freedom
Ballrooms E,F,G,H

This facilitated session will address questions around the basic research that remains to be done in different rare disease areas. It will provide tools to evaluate the research that already exists and the gaps that remain.

- After this session you will be to map the next steps you can take to strengthen your patient community's involvement and fill gaps in basic research in your disease area.

12:15 – 1:15 pm **Lunch**

1:15 – 2:15 pm **Expert Office Hours**
Freedom
Ballrooms E,F,G,H

More questions? Sign up for a 15-minute one-to-one conversation with an expert in the area you've identified as a pinch point in your drug development process.

2:15 – 3:00 pm **Closer Look Panel 2: Using New Technologies in Early Stage Research**
Liberty Ballroom D

Find out how emerging technologies may be used for drug discovery, design and repurposing, transforming an often complex, decades-long mission into a more efficient process, reducing the timeline and cost to bring therapies to patients.

Moderator: **David Fajgenbaum**, MD, MBA, MSc, Assistant Professor, Perelman School of Medicine and Associate

Director, Patient Impact, Orphan Disease Center, University of Pennsylvania

Panelists:

Bruce Bloom, CSO, Kabuki Syndrome Foundation

Daniel Fischer, MS, MBA, Co-Founder, President and CEO, Board Member, Tevard Biosciences

Maureen Hart, Ph.D., Director of Patient Advocacy, Policy, and External Engagement, Creyon Bio

Luke Rosen, MS, Founder, KIF1A

Additional resources on this topic:
From Molecules to Medicines: How are New Drugs and Therapies Developed
The Drug Rediscovery Pipeline: Finding New Indications for Approved Therapeutics
Early Drug Development: What Patients Bring
Artificial Intelligence and Machine Learning for Rare Disease

3:00 – 4:00 pm
Freedom Ballrooms E,F,G,H

Small Group Action Workshop: Collaborating with researchers

This facilitated workshop will explore identification of partners from leading areas of disease research, different types of research collaborations, and how collaborations using emerging technologies may differ.

- After this session, you will have greater confidence in how to approach and work with researchers and understand some of the alternative pathways to identifying drug targets.

4:00 – 5:00 pm
Freedom Ballrooms E,F,G,H

Expert Office Hours

More questions? Sign up for a 15-minute one-to-one conversation with an expert in the area you've identified as a pinch point in your drug development process.

5:00 – 6:30 pm
Liberty Foyer

Reception

DAY 3	WEDNESDAY May 3, 2023	8:00 am - 3:45 pm

8:00 am **Breakfast**

Liberty Ballroom C

9:00 – 9:30 am

Liberty Ballroom D

Day 2 Keynote: Intellectual Property: Balancing Stakeholders' Rights

Jim Wilson, Director, Orphan Disease Center, University of Pennsylvania

Additional resources on this topic:
Translational Steps: Academia to Industry

Response: **Alaa Hamed**, MD, MPH, MBA, Global Head of Medical Affairs, Rare Disease, Sanofi

9:30 – 10:15 am

Liberty Ballroom D

Closer Look 3: The Regulatory Landscape: Pathways, End Points, and Clinical Trials

In this session, you'll learn about the different pathways open for rare disease therapeutics, why the involvement of patients is essential to establishing meaningful outcome measures in these pathways, and what patient advocates can do to influence clinical trial design.

Moderator: **Katherine Maynard**, Communications and Alliance Development, PWR

Panelists:

Lea Ann Browning-McNee, MS, Director of Communication and Stakeholder Engagement, Reagan-Udall Foundation

Dan Lavery, Ph.D., CSO, Loulou Foundation

Neena Nizar, Ed.D., Founder and President, The Jansen's Foundation

Elizabeth Ottinger, Ph.D., Acting Director, Therapeutic Development Branch, National Center for Advancing Translational Sciences

Isaac Rodriguez-Chavez, Ph.D., M.H.S., M.S., Clinical Research and Digital Health Technology Consultant

Additional resources on this topic:
Strategies for Small Patient Populations
Mobilizing Your Community for Patient-Focused Drug Development

10:15 – 10:30 am **Coffee Break**

Liberty Foyer
Mezzanine Foyer

10:30 – 11:15 am **Small Group Action Workshop:** The Role of Patient Groups in the Regulatory Process

Freedom
Ballrooms E,F,G,H

This workshop will explore unique features of the rare regulatory landscape and information you need to facilitate partnerships. Find out what patients can do to influence clinical trial design and what's important to share with your community.

- After this session, you will have greater confidence in how to communicate in a meaningful way with regulatory bodies.

11:15 – 12:15 pm **Expert Office Hours**

Freedom
Ballrooms E,F,G,H

More questions? Sign up for a 15-minute one-to-one conversation with an expert in the area you've identified as a pinch point in your drug development process.

12:15 – 1:15 pm **Lunch**

Liberty Ballroom C

1:15 – 2:00 pm **Closer Look 4: Research Readiness**

Liberty Ballroom D

How do you know if your organization is “research ready?” This session will close the conference by bringing together the themes from two-days of conversations around patient-driven drug development, applying lessons learned to the collection and management of patient-collected data.

Moderator: **Karmen Trzupek**, M.S., C.G.C., Sr. Director, Scientific Programs, RARE-X, Global Genes

Panelists:

Sunitha Malepati, VP, CACNA1A Foundation

Ashley Winslow, Ph.D., President and CSO, Odylia Therapeutics

Sophia Zilber, Patient Registry Director, Cure Mito Foundation

Additional resources on this topic:

Data DIY: Your Involvement in Driving Understanding, Discovery and Treatments for Rare Disease
What Does It Take To Become a Research Ready Organization?
Chasing Cures: The Power of Patients

2:00 – 3:00 pm
Freedom Ballrooms E,F,G,H

Small Group Action Workshop: Creating Your Roadmap

Using the framework of the research readiness roadmap, what could your journey to a successful therapeutic look like? Learn how to create, implement and follow your research strategy utilizing the research readiness roadmap and data from your community and how you can leverage it within the research community.

- After this session, you will be able to better prioritize the next steps in your research strategy.

3:00 – 3:45 pm
Liberty Ballroom D

Keynote: How Can the FDA Improve Processes for Rare Diseases?

Peter Marks, M.D., Ph.D., Director FDA's Center for Biologics Evaluation and Research

Additional resources on this topic:
Cell and Gene Medicine

Closing Remarks:

Charlene Son Rigby, CEO, Global Genes