Agenda

May 1–3, 2023

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**

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

8:00 am **Breakfast**

9:00 – 9:45 am **Welcome:** Charlene Son Rigby, CEO, Global Genes
Day 1 Keynote: *Emil Kakkis – CEO, Ultragenyx and President, EveryLife Foundation (invited)*

9:45 –10:15 am **The RARE Research Roadmap** – Charting the Path to Treatments
Charlene Son Rigby, CEO, Global Genes
Deborah Requesens, Ph.D., Director, JumpStart, Orphan Disease Center, University of Pennsylvania

10:15 – 11:00 am **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: **Vanessa Vogel-Farley**, Research and Data Governance Lead, RARE-X, Global Genes

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 Centre, University of Pennsylvania

11:00 – 11:15 am **Coffee Break**

11:15 – 12:15 pm **Small Group Action Workshop**: Bridging basic research gaps

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**

More questions? Sign up for a 20-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**

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, University of Pennsylvania

Panelists:

Bruce Bloom, CSO, Kabuki Syndrome Foundation

Daniel Fisher, 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

3:00 – 4:00 pm **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 **Expert Office Hours**

More questions? Sign up for a 20-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 **Reception**

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

8:00 am **Breakfast**

9:00 – 9:30 am **Day 2 Keynote: *Intellectual Property: Balancing Stakeholders' Rights***
Jim Wilson, Director, Orphan Disease Center, University of Pennsylvania

9:30 – 10:15 am **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 Anne Browning-McNee, MS, Director of Communication and Stakeholder Engagement, Reagan-Udall Foundation

Neena Nizar, Founder and President, The Jansen's Foundation

Issac Rodriguez-Chavez, Ph.D., M.H.S., M.S.

10:15 – 10:30 am **Coffee Break**

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

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

More questions? Sign up for a 20-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**

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

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**, 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

2:00 – 3:00 pm **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 **Keynote: *How Can the FDA Improve Processes for Rare Diseases?***

Peter Marks, Director FDA's Center for Biologics Evaluation and Research

Closing Remarks: *What Have We Learned? What's Next?*
Charlene Son Rigby, CEO, Global Genes