

RARE DRUG



DEVELOPMENT SYMPOSIUM



#RDDS

2024 AGENDA

Innovative Ideas from Next Generation Change-Makers

Global Genes &
University of Pennsylvania's Orphan Disease Center
Monday, April 29 – Wednesday, May 1, 2024
Sheraton Downtown Philadelphia

Monday, April 29, 2024

10:00 AM – 5:00 PM ET
Registration

Liberty Foyer CD

5:30 PM – 7:30 PM ET
Reception

Horizons Rooftop Ballroom

Tuesday, April 30, 2024

8:00 AM – 5:00 PM ET
Registration

Liberty Foyer CD

8:00 AM – 5:00 PM ET
Poster Viewing and Networking

Liberty Foyer CD

8:00 AM – 9:00 AM ET
Breakfast

Liberty C

9:00 AM – 9:45 AM ET

Welcome and Opening Keynote: Shifting the Paradigm to Push Past Limits

Advocates are dramatically altering the landscape of rare research and reducing the timeline for rare disease therapy development. What is it that allows some organizations to move faster, be more nimble, use resources effectively and blaze new paths? Is there a matrix that can help you determine what will work for you?

- Charlene Son Rigby, CEO, Global Genes
- Tania Simoncelli, Vice President, Science in Society, Chan Zuckerberg Initiative

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Liberty D

9:45 AM - 10:30 AM ET

Session 1: The Critical Need for Patient-Led Data Initiatives: Does Size Matter?

Researchers and regulators need data. But what kind? Who is the end user? What is "enough data?" Do you need certain types of data for specific research projects? Why? Most importantly how do you evaluate your data set to be certain it's useful?

- **Moderator: Zohreh Talebizadeh, Ph.D., Senior Director, RARE-X Research Program, Global Genes**
- Ramona Walls, Ph.D., Executive Director of Data Science, C-Path
- Ben Forred, Director, The CoRDS Registry, Sanford Health
- Dave Jacoby, M.D., Ph.D., BioMarin Fellow in Clinical Science & Vice President, Head of Discovery Medicine, Brineura/BioMarin

Liberty D

10:30 AM – 10:45 AM ET
Morning Break

Liberty Foyer CD

10:45 AM – 11:45 AM ET

Interactive Groups on Patient-Led Data Initiatives

Potential topics include patient identification, types of data (genotype/phenotype, biomarker, outcome measure, etc.); combining data sets; use of data for disease modeling, progression and insights on therapeutic target; and strategies for registries, natural history studies, biobanks and cell lines.

Salon 3/4, Salon 5/6,
Salon 10, Liberty D

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12:00 PM - 1:00 PM ET

Lunch with GAA Networking

Liberty C

1:00 PM - 2:00 PM ET

Expert Office Hours (By Appointment Only)

Get answers to your questions by scheduling a one-to-one session with one of our experts on topics such as data collection, research strategy, therapy development and engaging with the FDA. For available appointments, please check with the Global Genes staff at the registration desk.

Salon 3/4, Salon 5/6,
Salon 10, Liberty D

2:15 PM - 3:00 PM ET

Session 2: Rethinking Clinical Trials: What's Doable? What's Approvable?

Researchers are overcoming barriers posed by small populations using decentralized and basket trials, platform science, wearables, videos, natural history data and engaging diverse patients. But what does FDA guidance say? Are the regulatory constraints different? How can patient communities prepare by ensuring their data is well-structured?

- **Moderator: Katherine Maynard, Partner, PWR**
- Jennifer Farmer, CEO, Friedreich's Ataxia Research Alliance
- Manoj Malhotra, M.D., Chief Medical Officer, Ovid Therapeutics
- Cynthia Rothblum-Oviatt, Ph.D., External Engagement Lead for FDA's Rare Diseases Team, U.S. Food & Drug Administration (FDA)

Liberty D

3:00 PM – 3:15 PM ET

Afternoon Break

Liberty CD Foyer

3:15 PM – 4:15 PM ET

Interactive Groups on Rethinking Clinical Trials

Potential topics include remote clinical trials, perpetual clinical trial platforms, using natural history data in lieu of placebos, use of avatars, effective and equitable communication between patients and researchers, platform science, N of 1 and accrual of patients for gene therapy....

Salon 3/4, Salon 5/6,
Salon 10, Liberty D

4:30 PM – 5:30 PM ET

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Salon 3/4, Salon 5/6,
Salon 10, Liberty D

5:30 PM – 8:00 PM ET

Welcome Reception

Horizons Rooftop Ballroom



Network:
Sheraton_Meeting

Password:
RDDS2024

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Wednesday, May 1, 2024

8:00 AM – 4:00 PM ET

Registration

Liberty Foyer CD

8:00 AM – 3:00 PM ET

Poster Viewing and Networking

Liberty Foyer CD

8:00 AM – 9:00 AM ET

Breakfast

Liberty C

9:00 AM – 9:15 AM ET

Welcome and Opening Remarks

Wrap up from yesterday and preparing for today's sessions

- Charlene Son Rigby, CEO, Global Genes

Liberty D

9:15 AM - 10:15 AM ET

Session 3: What Will \$100K Buy You? Emerging Commercial and Non-Profit Financing Models

You've raised some money and have a strategy (or are about to develop one). The next question is, "What will your funds realistically buy for your organization?" Are there creative ways to make the most of what you have? How do you choose partners and negotiate?

- **Moderator: Karmen Trzupek, Senior Director, Scientific Programs, Global Genes**
- Betty Cabrera, Director of Research Engagement & Operations, UC San Diego Gene Therapy Initiative
- Casey McPherson, CEO, To Cure A Rose Foundation
- Ashley Winslow, Ph.D., CEO & CSO, Odylia Therapeutics

Liberty D

10:15 AM – 10:30 AM ET

Morning Break

Liberty CD Foyer

10:30 AM – 11:30 AM ET

Interactive Groups on Emerging Commercial and Non-Profit Financing Models

Potential topics include using your money to jumpstart a project or combine with other funding sources, non-profit biotechs, public benefit corporations, N of 1 and cost comparison for various tools.

Salon 3/4, Salon 5/6,
Salon 10, Liberty D

11:30 AM – 1:00 PM ET

Lunch with Table Topics

Liberty C

1:00 PM - 2:30 PM ET

Session 4: Rare Resource Fair

Whether you're creating a research strategy - or putting an existing one into action, this session will help you build knowledge, ask providers of products and services the questions you have about your specific situation, and make informed decisions on which companies you want to work with. If you're new to research, you can request a mentor at the registration desk to guide you through the fair.

Liberty D

2:30 PM – 2:45 PM ET

Afternoon Break

Liberty CD Foyer

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2:45 PM - 3:45 PM ET

Closing Keynote and Remarks

What new solutions are emerging to accelerate translational research in rare disease? What can patients, caregivers, researchers and organizations do to help move the needle? In this 'Fireside Chat', you'll learn how Dominique Pichard's unique experience as a physician, rare Mom and advocate has informed her approach to leading innovation in rare disease research at NCATS.

- Charlene Son Rigby, CEO, Global Genes
- Dominique Pichard, M.D., Director, Division of Rare Diseases Research Innovation, National Center for Advancing Translational Sciences (NCATS), National Institutes of Health (NIH)

Liberty C

SAVE THE DATE!



September 25 – 28, 2024

Registration opens in May

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