

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 29th, 2024

10:00 AM – 5:00 PM ET Registration	Liberty Foyer CD
3:00 PM – 5:00 PM ET RARE-X Exchange (Additional Fee) A dynamic exchange of insights, innovation, and introductions to support patient advocates and researchers partnering with RARE-X. Case studies and outcomes will be presented to celebrate the successes of current research studies and identify opportunities for future collaborations.	Liberty D
5:30 PM – 7:30 PM ET Reception	Horizons Rooftop Ballroom

Tuesday, April 30th, 2024

8:00 AM – 5:00 PM ET Registration	Liberty Foyer CD
8:00 AM – 5:00 PM ET Exhibitors	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 orgs 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? <ul style="list-style-type: none"> Charlene Son Rigby, CEO Global Genes Tanya Simoncelli, MS, Vice President, Science in Society, Chan Zuckerberg Initiative) 	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?	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.	

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<p>12:00 PM - 1:00 PM ET Lunch with GAA Networking</p>	<p><i>Liberty C</i></p>
<p>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. Appointments will open in March for registered attendees.</p>	
<p>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?</p>	<p><i>Liberty D</i></p>
<p>3:00 PM – 3:15 PM ET Afternoon Break</p>	<p><i>Liberty CD Foyer</i></p>
<p>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, and N of 1. Accrual of patients for gene therapy....</p>	
<p>4:30 PM – 5:30 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. Appointments will open in March for registered attendees.</p>	
<p>5:30 PM – 8:00 PM ET Reception</p>	<p><i>Horizons Rooftop Ballroom</i></p>

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Wednesday, May 1st, 2024

8:00 AM – 4:00 PM ET Registration	Liberty Foyer CD
8:00 AM – 3:00 PM ET Exhibitors	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 <ul style="list-style-type: none"> 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 Model 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?	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 using your money to jumpstart a project or combine with other funding sources, non-profit biotechs, public benefit corps, n of 1 and cost comparison for various tools.	
11:30 AM – 1:00 PM ET Lunch with Facilitated 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 to guide you through the fair.	Liberty C/D
2:30 PM – 2:45 PM ET Afternoon Break	Liberty CD Foyer
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 will inform her approach to leading innovation in rare disease research at NCATS.	Liberty D