RARE Drug Development SYMPOSIUM

Advocates Powering Research

Global Genes® Allies in Rare Disease

Joseph B. Martin Conference Center
Harvard Medical School
Boston, MA

Wednesday, September 3, 2025

8:00 AM - 9:00 AM ET — Amphitheater Lobby, Ground Floor

Registration & Continental Breakfast | *Sponsored by* biocryst

9:00 AM - 9:15 AM ET — Amphitheater, Ground Floor

Welcome: Charlene Son Rigby, CEO, Global Genes

9:15 AM - 10:15 AM ET — Amphitheater, Ground Floor

Session 1: Patient Advocates Transforming the Landscape of their Disease

Kicking off RDDS, this opening session will explore the powerful impact of patient advocacy in driving research forward. Charlene Son Rigby will present from the perspective of a parent advocate, highlighting successful models across various pediatric diseases. Joining her will be Sonia Vallabh, sharing parallel successes in adult-onset conditions, demonstrating what is possible when patients and families lead the charge in shaping the research agenda.

- Charlene Son Rigby, CEO, Global Genes
- Sonia Vallabh, Ph.D., Adult-Onset Disease Advocate, Director, Prion Therapeutic Science, Broad Institute

10:15 AM - 10:30 AM ET — Amphitheater Lobby, Ground Floor

Morning Break | *Sponsored by* ACADIA

10:30 AM - 11:30 AM ET — Amphitheater, Ground Floor

Session 2: Navigating the Drug Development Roadmap - An Overview

This engaging session will guide attendees through the Drug Development Roadmap, introducing advocates to key stages and highlighting critical areas for engagement. By leveraging practical tools and real-world case studies, this session will equip participants for deeper content explorations and help lay the groundwork for future research strategies tailored to their disease.

- ***Moderator*: Suki Bagal, M.D., M.P.H., Rare Disease Drug Development Expert; Founder, RSVGT Consultancy**
- Elizabeth Buttermore, Ph.D., Director, Translational In Vitro Models, Boston Children's Hospital
- Ebony Dashiell-Aje, Ph.D., Executive Director and Head, Patient-Centered Outcomes Science, BioMarin Pharmaceutical

Wednesday, September 3, 2025

11:30 AM - 11:45 AM ET — Amphitheater Lobby, Ground Floor

Mid-Morning Break | *Sponsored by* Bio

11:45 AM - 12:45 PM ET — Amphitheater, Ground Floor

Session 3: Therapeutic Modalities & Their Relevance to Your Disease

A solid understanding of your disease biology is essential for identifying the most effective therapeutic approaches. In this deep dive session, we will explore different therapeutic modalities and how they align with disease biology, prevalence and other factors. You will gain insight into how this knowledge can shape your research strategy and help prioritize the most promising areas for development.

- ***Moderator*: Danny Levine, Global Genes**
- Daniel Fischer, President & CEO, Tevard Biosciences
- Sarah Gladstone, M.D., Chief Scientific Officer, Chief Medical Officer, The Snow Foundation for Wolfram Syndrome Research
- Tim Yu, M.D., Ph.D., Principal Investigator, Boston Children's Hospital

1:00 PM - 2:00 PM ET — Rotunda, Third Floor

Networking Lunch & Table Topic Discussions | *Sponsored by* Chiesi global rare diseases

2:15 PM - 3:15 PM ET — Amphitheater, Ground Floor

Session 4: Pitch Perfect: De-Risking Your Disease to Drive Investment & Collaboration

This strategic session will help advocates understand what is needed in their research portfolio to make their disease area more compelling to potential research partners, biopharma and investors. You will learn how to develop the right assets and position your disease in a way that highlights its scientific opportunity, unmet need, and potential for impact - key elements to drive future collaboration and funding. Pitch presentations by advocates driving research, will provide real world experience and examples to consider.

- ***Moderator*: Walt Kowtoniuk, Ph.D., Venture Partner, Third Rock Ventures**
- Maya Chopra, MBBS, FRACP, Clinical Geneticist, Boston Children's Hospital
- Craig Lipset, Co-Founder & Co-Chair, Decentralized Trials & Research Alliance
- Mustafa Sahin, M.D., Ph.D., Neurologist-in-Chief, Department of Neurology, Boston Children's Hospital
- Yael Weiss, M.D., Ph.D., CEO, Mahzi Therapeutics

Pitches by:

curehht — ***Cassi Friday, Ph.D.***, *Director, Research*

CURE VCP DISEASE, INC. — ***Zollie Yavarow, Ph.D.***, *Director, Scientific Engagement & Collaborations*

Agenda subject to change

Wednesday, September 3, 2025

3:15 PM - 3:30 PM ET — Amphitheater Lobby, Ground Floor

Afternoon Break | *Sponsored by* bridgebio

3:30 PM - 4:30 PM ET

Interactive Groups on De-Risking Your Disease

Join small group discussions to ask questions, share ideas, and get practical insights to strengthen your strategy and spark future partnerships.

Amphitheater - Walt Kowtoniuk, Cassi Friday and Zollie Yavarow
Classroom 214 - Maya Chopra
Classroom 216 - Craig Lipset
Classroom 217 - Yael Weiss

4:45 PM - 5:30 PM ET — Classrooms 214, 216, 217 & 2nd Floor Lounge

Expert Office Hours - By Appointment Only | *Sponsored by* ALEXION AstraZeneca Rare Disease

Get answers to your questions by scheduling a one-to-one session with one of our experts.

5:30 PM - 7:00 PM ET — Main Lobby, Ground Floor

Welcome Reception

Wi-Fi Instructions | *Sponsored by* sanofi

1. Go to Settings on your phone or laptop
2. Go to Wi-Fi
3. Click HMS Guest
4. You will then be prompted to register for the network.
5. You will be asked to provide the following: First Name, Last Name, Email Address and Cell Provider (Verizon, AT&T, etc.)
6. Once registered, you will then get your Username and Password (which will be emailed as well). This Username and Password will then give you access to the Guest Network.
7. With the given Username and Password, you can now login to the HMS Guest network.

*Access will be available both days with same Username/Password

Thursday, September 4, 2025

8:00 AM - 9:00 AM ET — Amphitheater Lobby, Ground Floor

Registration & Continental Breakfast | *Sponsored by* IONIS

9:00 AM - 9:15 AM ET — Amphitheater, Ground Floor

Day 1 Recap: Charlene Son Rigby, CEO, Global Genes

Welcome: R. Duane Clark, General Manager, Rare Diseases, Sanofi

9:15 AM - 10:15 AM ET — Amphitheater, Ground Floor

Session 5: Developing Data Assets to Support Therapeutic Development

High-quality, disease-specific data is foundational to advancing therapeutic research. In this session, we will explore what types of data are most valuable - such as natural history, biomarker, genomic, and patient-reported outcomes - and how to strategically generate, structure, and share these assets. You will learn how robust data sets can attract research collaborators, inform clinical trial design, and accelerate therapeutic development. Whether you are just starting or looking to strengthen your existing data strategy, this session will provide actionable guidance for building a data portfolio that drives impact.

- ***Moderator*: Wendy Chung. M.D., Ph.D., Chair, Pediatrics, Boston Children's Hospital**
- Susan Faja, Ph.D., Associate Professor, Pediatrics, Harvard Medical School
- Krista Vasi, M.P.A., Executive Director, Usher Syndrome Coalition

10:15 AM - 10:30 AM ET — Amphitheater Lobby, Ground Floor

Morning Break | *Sponsored by* ultragenyx

10:45 AM - 11:45 AM ET

Interactive Groups on Data Assets

Continue the conversation and discuss your current data efforts, share challenges, and get peer insights on building or strengthening your disease-specific data assets.

Amphitheater - Wendy Chung
Classroom 214 - Susan Faja
Classroom 216 - Krista Vasi
Classroom 217 - Astrid Rasmussen

12:00 PM - 1:00 PM ET — Classrooms 214, 216, 217 & 2nd Floor Lounge

Expert Office Hours - By Appointment Only | *Sponsored by* TRAVERE THERAPEUTICS

Get answers to your questions by scheduling a one-to-one session with one of our experts.

Thursday, September 4, 2025

1:15 PM - 2:00 PM ET — Rotunda, Third Floor

Networking Lunch | *Sponsored by* rtw Foundation

Presentation by Mimi Lee, M.D., Ph.D., *Innovative Funding in Rare Disease*

2:00 PM - 3:00 PM ET — Amphitheater, Ground Floor

Session 6: Clinical Trials: Early & Often Practical Framework

This session will review key recommendations from the *Early and Often* whitepaper and introduce a collaborative framework developed by biopharma and patient groups. Through a practical lens, we will map out the drug development timeline, with an end goal of successful, efficient clinical trials, and highlight where, how, and what advocacy groups can meaningfully contribute. By understanding these strategic touch points, you will be better equipped to accelerate and advance research efforts in your disease area.

- ***Moderator*: Charlene Son Rigby, CEO, Global Genes**
- Wendy Erler, Senior Vice President, Sarepta Therapeutics
- Steven Roberds, Ph.D., Chief Scientific Officer, TSC Alliance

The 2025 RARE Drug Development Symposium is grateful to our valued Collaborators:

Boston Children's Hospital
Where the world comes for answers

ROSAMUND STONE ZANDER TRANSLATIONAL NEUROSCIENCE CENTER

TERMEER INSTITUTE®

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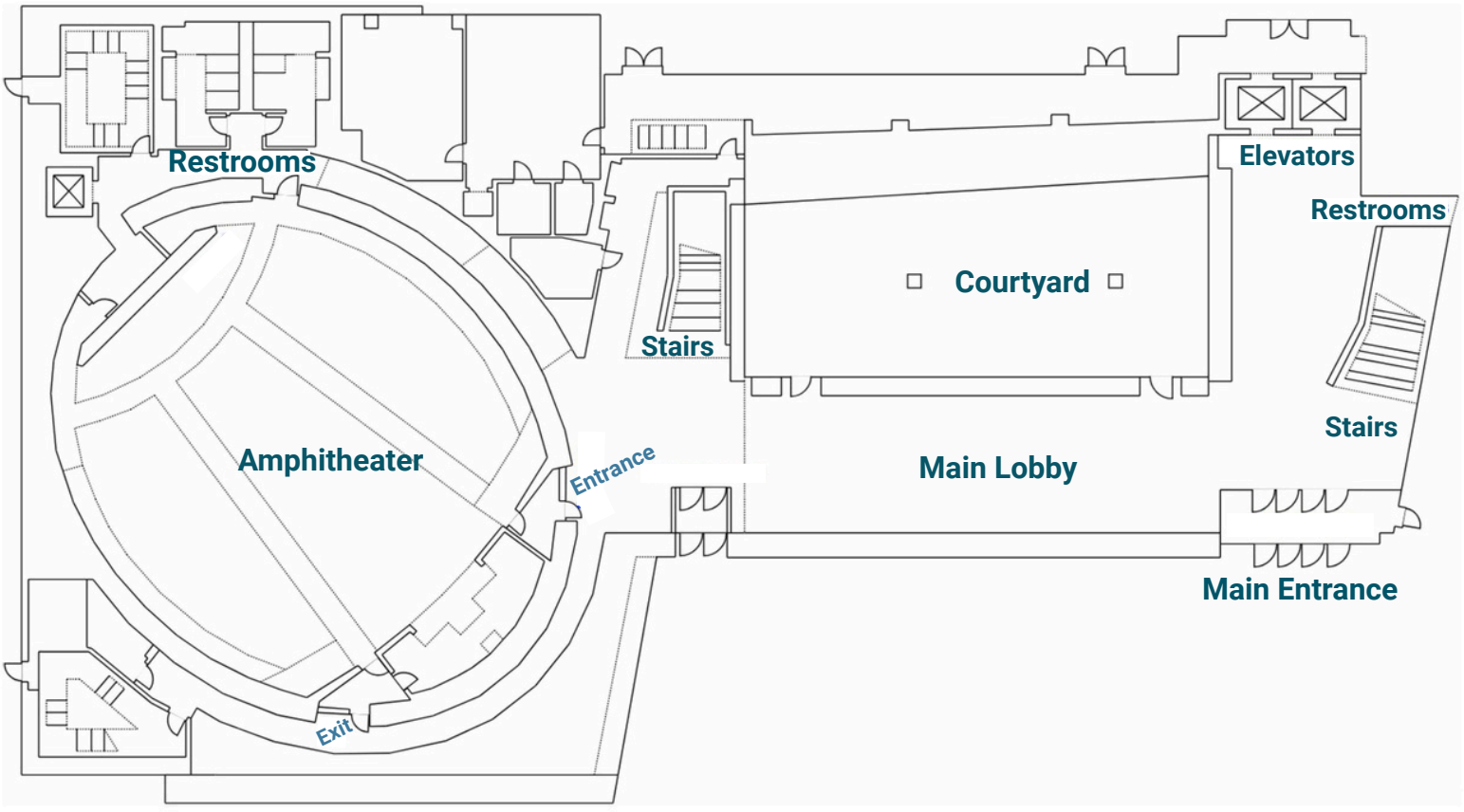
Ground Floor

Wednesday, September 3rd
Registration & Breakfast
AM & PM Breaks
Main Sessions
Interactive Groups
Welcome Reception

Thursday, September 4th
Registration & Breakfast
AM Breaks
Main Sessions
Interactive Groups

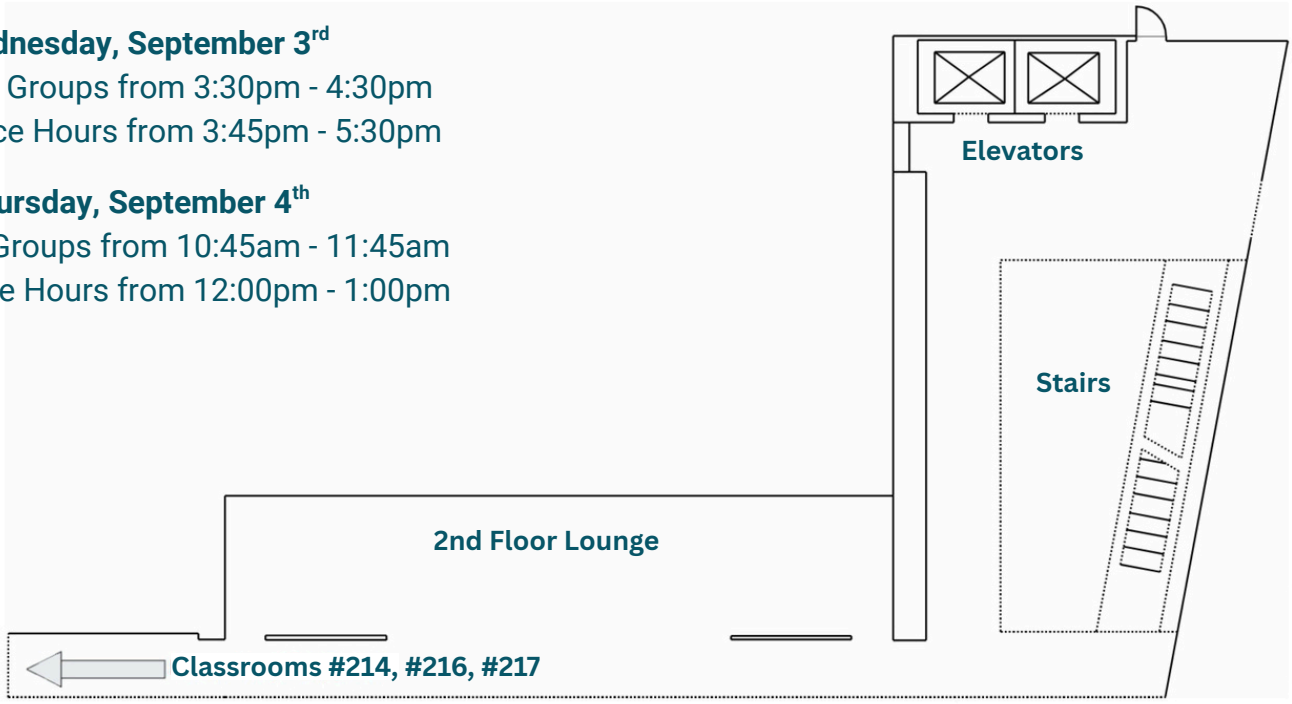


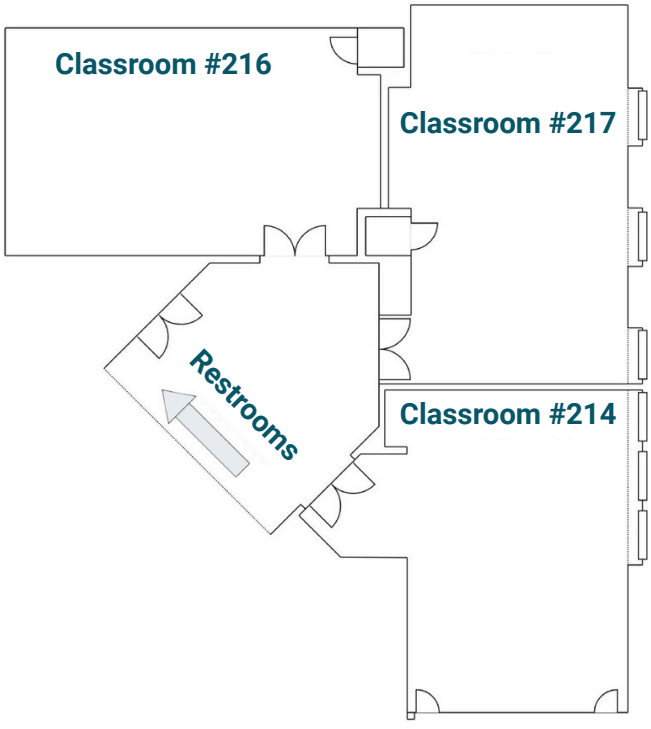
Agenda subject to change

Second Floor

Wednesday, September 3rd
Interactive Groups from 3:30pm - 4:30pm
Expert Office Hours from 3:45pm - 5:30pm

Thursday, September 4th
Interactive Groups from 10:45am - 11:45am
Expert Office Hours from 12:00pm - 1:00pm





Third Floor

Location of Networking Lunches

September 3 | 1:00pm - 2:00pm
September 4 | 1:15pm - 2:00pm