Accelerating Rare Disease Progress: Aligning Advocates, Science & Industry

SEPTEMBER 9, 2026 | DAY 1

Registration | Continental Breakfast | Roundtables | *8:00 - 9:30 AM*

Expert Office Hours | *8:30 – 9:30 AM*

Attendees will have the opportunity to sign up for one-on-one, 20-minute sessions with leading experts across drug development, regulatory affairs, data strategy, and market access - a signature feature of RDDS designed to give participants direct access to personalized guidance.

Welcome | *9:30 – 9:45 AM*

Charlene Son Rigby, CEO, Global Genes

Opening Keynote / Fireside Chat | *9:45 – 10:45 AM*

This fireside chat will highlight what it takes to move rare disease science from discovery to approval - and what today's advocates can do to position their programs for success.

Session 1: Build It and They Will Come: Datasets that De-risk Therapeutic Development and Attract Investment |*11:00 AM – 12:00 PM*

High-quality, strategically designed datasets are among the most powerful tools a patient advocacy group can develop - but building them requires foresight about what industry and regulators need. This panel will explore how natural history studies, biomarker data, patient-reported outcomes, and genomic assets can be structured to attract industry partners, satisfy FDA expectations, and de-risk therapeutic development for potential investors. Panelists will also address the practical considerations that advocates must navigate to ensure their research investments can be fully utilized - including data governance, IP ownership, and publication strategy.

Lunch with Session 1 Interactive Breakouts | *12:00 – 1:30 PM*

Attendees will continue the conversation from Session 1 in facilitated small-group breakouts over lunch, diving deeper into focused topics: natural history study design and data collection, the value and process of scientific publication, or IP and data ownership considerations for patient advocacy organizations.

**agenda subject to change*

Session 2: Therapeutic Timeline Deep Dive: Synergies and Handoffs that Matter Most | *1:45 – 2:45 PM*

Understanding the full arc of drug development - from gene discovery through preclinical work, clinical trials, and commercialization - is essential for patient advocates who want to accelerate therapeutic research. This session will bring each stage to life utilizing two in-depth disease case studies. Through close advocacy/ industry partnerships, Angelman syndrome now has multiple "Breakthrough Therapy"-designated candidates in active clinical trials, while Niemann-Pick Type C disease recently celebrated the FDA approval of two treatments for NPC, with a third anticipated this year. Together, they will share the real-world handoffs, setbacks, and synergies that shaped their journeys.

Session 2 Interactive Group Breakouts | *3:00 – 4:30 PM*

Building on the case studies presented in Session 2, attendees will break into smaller facilitated groups anchored by the session's panelists, allowing for deeper dialogue on the specific challenges and opportunities at each stage of the therapeutic development timeline.

Welcome Reception – Expo, Networking & Success Stories | *4:45 – 7:00 PM*

Join fellow attendees, speakers, and sponsors for an evening of networking in the expo hall, featuring poster presentations highlighting rare disease research milestones and patient community success stories.

SEPTEMBER 10, 2026 | DAY 2

Day 1 Recap | *8:30 – 8:45 AM*

Charlene Son Rigby, CEO, Global Genes

Session 3: Successes and Lessons Learned from Recent Rare Disease Drug Approvals and Clinical Trials | *8:45 – 9:45 AM*

The rare disease field has seen a wave of hard-won approvals in recent years - alongside pivotal trials that did not succeed but reshaped our understanding of what it takes to get there. Through a series of case studies, this panel will examine the clinical trial designs, outcome measures, and advocacy strategies that made the difference in recent programs. Attendees will walk away with honest, actionable lessons from both breakthroughs and disappointments that can be directly applied to their own research efforts.

Session 4: Regulatory Update: Innovative Paths to Therapy Development & Scalability | *9:45–10:15 AM*

This focused session offers a direct window into the FDA's evolving approach to rare disease drug development.

Session 5: Market Access for Rare and Ultrarare Communities: We Can't Wait Until the Approval Comes | *10:45 AM – 11:45 AM*

For rare disease communities, FDA approval is only one milestone. Ensuring that patients can access and afford therapy is a challenge that must be anticipated long before the approval comes. This panel will bring together state Medicaid leadership, biopharma access experts, payer strategists, and a patient advocate voice to examine the full market access landscape. Panelists will offer concrete perspectives on what advocacy groups can do now to generate real-world evidence and navigate the policy levers that determine whether an approved therapy reaches the patients who need it.

**agenda subject to change*

Sessions 3 – 5 Interactive Breakouts | *11:45 AM – 1:00 PM*

Attendees will choose from facilitated breakout sessions on focused tracks: outcome measures and clinical trial design, market access strategy, and regulatory innovation - connecting the morning's main stage themes to their own disease areas and program needs.

Expert Office Hours | *2:00 – 3:00 PM*

Attendees will have the opportunity to sign up for one-on-one, 20-minute sessions with leading experts across drug development, regulatory affairs, data strategy, and market access - a signature feature of RDDS designed to give participants direct access to personalized guidance.

Session 6: Reimagining Industry–Advocacy Collaboration in Drug Development | *3:00 – 4:00 PM*

Rare disease drug development moves faster and smarter when shaped by the people who live with these conditions every day. Yet, patient engagement is still too often treated as a consultation rather than a strategic partnership. This panel explores how advocates can drive earlier, deeper collaboration across the development lifecycle—from discovery through trial design. Discover concrete strategies to elevate your voice, build trial readiness, and ensure patient insight is built directly into industry decisions from day one.

Closing Keynote: Scaling N of 1 - A Collective Effort| *4:30 – 5:30 PM*

Precision medicine has moved us from treating common rare diseases like cystic fibrosis to therapies for ultra-rare conditions affecting a few thousand patients, and more recently N-of-1 treatments for patients with unique mutations. These aren't separate stories: they're a spectrum. In this keynote, we will discuss scaling N of 1, how the regulatory and access frameworks we build will impact who gets access to treatments, and the criticality of the strength and cohesion of disease communities.

Networking Reception & Expo | *5:30 – 7:30 PM*

Join fellow attendees and sponsors for a networking reception in the expo hall.

SEPTEMBER 11, 2026 | DAY 3

Day 3 Kickoff: De-Risking Your Disease to Attract and Sustain Investment | *9:00 – 9:30 AM*

Attracting investment for a rare disease program is never easy, but some disease communities have cracked the code by systematically building and promoting the assets that make investors and biopharma companies take notice.

Pitch Session – Main Stage | *9:30 – 10:30 AM*

Two exceptional patient advocates will take the main stage to present their disease programs, making the case for partnership, investment, and collaboration to a panel of expert judges. Presentations will demonstrate how communities have built the scientific, clinical, and organizational assets needed to attract and sustain interest from biopharma and investors.

Pitch Sessions by Disease Area | *11:00 AM – 12:30 PM*

Following the main stage pitches, attendees will break into disease-specific tracks to pitch and receive feedback from a panel of clinical and industry representatives with expertise in specific disease areas. These smaller group sessions will allow for deeper, community-specific feedback from judges and peers, and create space for the kinds of conversations that lead to lasting research collaborations.

**agenda subject to change*