RARE Drug Development SYMPOSIUM

Advocates Powering Research

Global Genes® Allies in Rare Disease

Wednesday, September 9, 2026

8:00 AM - 9:30 AM ET — Atrium | 3rd Floor

Registration & Continental Breakfast

Roundtable Discussions | *Sponsored by* Catalyst pharmaceuticals — Canopy Hall | 3rd Floor

8:30 AM - 9:30 AM ET — Branch View, Cedar Grove Lane's Edge | 2nd Floor

Expert Office Hours - *By Appointment Only* | *Sponsored by* astellas GENE THERAPIES

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

9:30 AM - 9:45 AM ET — Canopy Hall | 3rd Floor

Welcome: **Charlene Son Rigby**, CEO, Global Genes

9:45 AM - 10:45 AM ET — Canopy Hall | 3rd Floor

Opening Fireside Chat | *Sponsored by* ALEXION AstraZeneca Rare Disease

Parent-Led Drug Development: From Cell Lines to Dosing Patients, The FOXG1 Story

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.

- **Charlene Son Rigby,** CEO, Global Genes
- **Nasha Fitter**, CEO, FOXG1 Research Foundation; CBO, Citizen Health

11:00 AM - 12:00 PM ET | *Sponsored by* TRAVERE THERAPEUTICS — Canopy Hall | 3rd Floor

Session 1: Build It and They Will Come:
Datasets that De-risk Therapeutic Development & Attract Investment

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.

- ***Moderator*: Ashley Winslow, Ph.D.,** CEO & CSO, Odylia Therapeutics
- Megan Abbott, M.D., Pediatric Epileptologist, University of Colorado
- Lindsay Jesteadt, Ph.D., CEO, Sleep Consortium, Inc.
- Joe Katakowski, Ph.D., Director of Research, RTW Foundation

Wednesday, September 9, 2026

12:15 PM - 1:30 PM ET — Canopy Hall | 3rd Floor
Lunch with Interactive Breakouts | *Sponsored by* ALEXION AstraZeneca Rare Disease — Cedar Grove | 2nd Floor
**Refer to information in folder for locations* — Lane's Edge | Riverbend

- **Natural History Studies & Real World Data** with Megan Abbott, University of Colorado and Ashley Winslow, Odylia Therapeutics
- **Patient Advocacy & Multi-Industry Consortia** with Lindsay Jesteadt, Sleep Consortium, Inc.
- **IP & Ownership** with Joe Katakowski and Nicole Litt, RTW Foundation
- **From Community Insight to Scientific Impact: Publishing & Amplifying Rare Disease** with Rohita Sharma, Global Head - Strategic Alliances & Patient Advocacy, Alexion and Hélène Dassule, Head of Excellence, Scientific Communications, Alexion

1:45 PM - 2:45 PM ET — Canopy Hall | 3rd Floor

Session 2: Therapeutic Timeline Deep Dive: Synergies and Handoffs that Matter Most
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, academic and industry partnerships, Angelman syndrome now has multiple "Breakthrough Therapy"- designated candidates in active clinical trials, while Niemann-Pick Type C (NPC) 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.

- **Moderator**: **Jill Weimer, Ph.D.,** Senior Director, Therapeutic Development, Sanford Research
- Sean Kassen, Ph.D., Director, Ara Parseghian Medical Research Fund, University of Notre Dame
- Jennifer Panagoulias, R.A.C., Head of Regulatory & Policy, Foundation for Angelman Syndrome Therapeutics (FAST)
- Marc Patterson, M.D., Chief Medical Officer, IntraBio

2:45 PM - 3:15PM ET — Atrium | 3rd Floor
Afternoon Break | *Sponsored by* biocryst

Wednesday, September 9, 2026

3:15 PM - 4:30 PM ET — Canopy Hall | 3rd Floor
Session 2 Interactive Breakout Sessions | *Sponsored by* TRAVERE THERAPEUTICS — Cedar Grove | 2nd Floor
**Refer to information in folder for locations* — Lane's Edge | Riverbend

- **Built to Partner: Developing Ready-Made Toolkits for Drug Developers** with Jennfier Panagoulias, FAST and Jill Weimer, Sanford Research
- **The Partnership Playbook: Academic, Advocacy and Industry Collaboration from Day One** with Sean Kassen, University of Notre Dame and Marc Patterson, IntraBio
- **Do I have the Right Models to Advance my Development** with Dana Layo-Carris, Ph.D., Senior Study Director, The Jackson Laboratory
- **Using AI in Early-Stage Drug Discovery** with Casey McPherson, CEO, AlphaRose Therapeutics and Zohreh Talebizadeh, Ph.D., Senior Director, RARE-X Research Program, Global Genes

4:30 PM - 7:00 PM ET — Atrium | 3rd Floor
Welcome Reception – Catalyst Zone — East & West Branch
**Refer to information in folder for exhibitors*

Join fellow attendees, speakers, and sponsors - explore our revamped expo hall - The Catalyst Zone. Specifically designed for rare disease community leaders to discover and partner with essential enablers of patient-advocate-driven research. Connect directly with experts in laboratory services, cell and gene therapies, cell line development, manufacturing, and contract research organizations (CROs) during our evening reception.

NOTES:

Thursday, September 10, 2026

8:00 AM - 8:30 AM ET — Atrium | 3rd Floor

Registration & Continental Breakfast | *Sponsored by* Citizen Health

8:30 AM - 8:45 AM ET — Canopy Hall | 3rd Floor

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

8:45 AM - 9:45 AM ET — Canopy Hall | 3rd Floor

Session 3: Successes & Lessons Learned from Recent Rare Disease Drug Approvals & Clinical Trials
Sponsored By BRIDGEBIO

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.

- **Moderator**: **Steve Rodems, Ph.D.**, VP, Research & Nonclinical Development, Travere Therapeutics
- Erica Cox, Ph.D., Executive Director, Denali Therapeutics
- Lindsay Marjoram, Ph.D., Chief Scientific Officer, Barth Syndrome Foundation
- Reenie McCarthy, J.D., CEO, Mighty Therapeutics
- Kim Stephens, D.B.A., Executive Director, Muenzer MPS Research & Treatment Center

9:45 AM - 10:15 AM ET — Canopy Hall | 3rd Floor

Session 4: Regulatory Update: Innovative Paths to Therapy Development & Scalability
This focused session offers a direct window into the FDA's evolving approach to rare disease drug development.

- **Amy Comstock Rick, J.D.**, Director, Rare Disease Innovation Hub, FDA
- **Christina Hartman**, Head, Government Affairs, Orchard Therapeutics; Board Member, Global Genes

10:15 AM - 10:45 AM ET — Atrium | 3rd Floor

Morning Break | *Sponsored by* dyno

Thursday, September 10, 2026

10:45 AM - 11:45 AM ET — Canopy Hall | 3rd Floor

Session 5: Market Access for Rare & Ultrarare Communities: We Can't Wait Until the Approval Comes
Sponsored by ALEXION AstraZeneca Rare Disease

FDA approval is only the first step; securing affordable patient access requires early planning. This panel brings together experts from private insurance, HTA organizations, and biopharma to explore how evidence drives access and reimbursement. Attendees will gain strategies for patient advocacy groups to generate high-quality evidence that sways key healthcare stakeholders and accelerates access to innovative treatments.

- **Moderator**: **Leora Schiff, M.S., M.B.A**, Principal & Founder, *Retired*, Altius Strategy Consulting
- Dan Ollendorf, Ph.D., M.P.H., Chief Scientific Officer & Director of HTA Methods & Engagement, ICER
- Clark Paramore, M.S.P.H., Head of Global Value Evidence Strategy, Biogen
- Michael Sherman, M.D., M.B.A., M.S., Chief Strategy Officer, GeneSprout

12:00 PM - 1:30 PM ET — Canopy Hall | 3rd Floor
Sessions 3 – 5 Interactive Breakouts | *Sponsored by* Jazz Pharmaceuticals — Branch View | 2nd Floor
**Refer to information in folder for location* — Cedar Grove | Lane's Edge | Riverbend

- **Biomarker Development and Regulatory** with Erica Cox, Denali Therapeutics and Kim Stephens, Muenzer MPS Research & Treatment Center
- **Endpoint Development In Clinical Trial Prep** with Mustafa Sahin, M.D., Ph.D., Chair of Neurology & Audrey Thurm, Ph.D., Translational Neuroscience Center, Boston Children's Hospital (BCH)
- **Innovation in Study Design in Ultrarare Populations** with Lindsay Marjoram, Barth Syndrome Foundation and Reenie McCarthy, Mighty Therapeutics
- **Creating an Evidence Development Plan to Make the Best Case for Reimbursement and Access** with Leora Schiff, Dan Ollendorf, ICER, Clark Paramore, Biogen and Michael Sherman, GeneSprout
- **Can We Build a Repeatable Path to the Clinic? Lessons from the Accelerating Medicines Partnership® Bespoke Gene Therapy Consortium** with Kira Gillett, M.S., Program Manager, Rare Diseases Translational Science, Foundation for the National Institutes of Health (FNIH)

1:30 PM - 3:00 PM ET — Canopy Hall | 3rd Floor
Lunch with Roundtables & Catalyst Zone | *Sponsored by* astellas GENE THERAPIES

2:00 PM - 3:00 PM ET — Cedar Grove | 2nd Floor
Expert Office Hours - *By Appointment Only* | *Sponsored by* IONIS — Lane's Edge | Riverbend

Thursday, September 10, 2026

3:00 PM - 4:00 PM ET — Canopy Hall | 3rd Floor

Session 6: Reimagining Industry–Advocacy Collaboration in Drug Development

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.

- **Moderator**: **Daniel Levine, M.J.**, Life Sciences Writer, Global Genes
- Michelle Davis, Executive Director, International FOP Association (IFOPA)
- Wendy Erler, M.B.A., Senior Vice President, Patient Affairs, Sarepta; Boad Member, Global Genes
- Tai Pasquini, Ph.D., M.P.A., Chief Research Officer, Congenital Hyperinsulinism International

4:00 PM - 4:30 PM ET — Atrium | 3rd Floor

Afternoon Break | *Sponsored by* Catalyst pharmaceuticals

4:30 PM - 5:30 PM ET — Canopy Hall | 3rd Floor

Fireside Chat: Scaling N of 1 - A Collective Effort

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

- **Charlene Son Rigby,** CEO, Global Genes
- **Timothy Yu, M.D., Ph.D.**, Co-Founder, N=1 Collaborative and the Center for Therapeutic Genetics

5:30 PM - 7:00 PM ET — Atrium | 3rd Floor

Networking Reception & Catalyst Zone | *Sponsored by* Bial Keeping life in mind, biocryst — East & West Branch

**Refer to information in folder for exhibitors*

Connect with peers, industry leaders, and sponsors in The Zone. Explore the Catalyst Zone to discover innovative solutions, exchange ideas, and build valuable professional connections in a relaxed atmosphere.

Friday, September 11, 2026

8:00 AM - 8:45 AM ET — Atrium | 3rd Floor

Registration & Continental Breakfast

Roundtable Discussions | *Sponsored by* astellas GENE THERAPIES — Canopy Hall | 3rd Floor

8:45 AM - 9:15AM ET — Canopy Hall | 3rd Floor

Keynote: Venture Philanthropy Redefined: Building Scalable, Sustainable Models for Rare Diseases

Do you know what it takes to build rare disease funding models that are truly sustainable — ones that generate returns, reinvest them, and grow stronger with every therapy they help bring to patients? Under Michael Hund's leadership, EBRP pioneered a venture philanthropy approach that transformed the clinical landscape for epidermolysis bullosa from just two active trials to more than fifty and helped deliver three FDA-approved therapies where none had existed before — proof that the model works. Now, through Rare Ventures, Hund is scaling that blueprint across disease communities, uniting patient advocacy, academic and industry partners driving rare disease research, and next generation technologies in a coordinated platform designed to connect patient data, AI, translational science, and commercialization into one system capable of supporting hundreds of rare diseases over time.

- **Michael Hund, M.B.A.,** CEO, EB Research Partnership (EBRP); Co-Founder, Rare Ventures

9:15AM - 9:30 AM ET — Canopy Hall | 3rd Floor

Session 7: De-Risking Your Disease to Attract and Sustain Investment

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.

- **Yael Weiss, M.D., Ph.D.,** CEO, Mahzi Therapeutics

NOTES:

Friday, September 11, 2026

9:30 AM - 10:45 AM ET — Canopy Hall | 3rd Floor

Pitch Session – Main Stage | *Sponsored by* BRIDGEBIO

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.

Judges

- **Maya Chopra, M.B.B.S, F.R.A.C.P**, Division Chief, Neurogenetics and Neurodevelopment, Department of Neurology, Boston Children's Hospital
- **Michael Hund, M.B.A.,** CEO, EB Research Partnership (EBRP); Co-Founder, Rare Ventures
- **Walt Kowtoniuk, Ph.D**., Venture Partner, Third Rock Ventures; Board Chair, Global Genes
- **Ankit Malhotra, Ph.D**., Global Head of Genomics, Strategy & Solutions, Amazon Web Services
- **Yael Weiss, M.D., Ph.D**., CEO, Mahzi Therapeutics

Pitches by:

SOPHIE'S HOPE FOUNDATION

Building Industry Attraction
Jamas LaFreniere, Founder & President

dada2 FOUNDATION

Strategic Asset Partnerships
Lex Cowsert, Ph.D., Chief Scientific Officer

10:45 AM - 11:15 AM ET — Atrium | 3rd Floor

Morning Break | *Sponsored by* rtw Foundation

NOTES:

Friday, September 11, 2026

11:15 AM - 12:30 PM ET — Canopy Hall | 3rd Floor

Pitch Sessions by Disease Area — Lane's Edge | Riverbend 2nd Floor

**Refer to information in folder for locations*

Attendees will break into disease-specific tracks to watch disease area pitches and the feedback received from a panel of clinical and industry representatives with expertise in specific disease areas.

Track 1: Neurodevelopmental Disorders & Epilepsy | *Sponsored by* Jazz Pharmaceuticals

Judges

- Brett Abrahams, Ph.D., President & Founder, Heppinn Biosciences
- Wendy Chung, M.D., Ph.D., Physican in Chief, Boston Children's Hospital
- Joe Katakowski, Ph.D., Director, Research, RTW Foundation
- Rodney Bowling, Ph.D., Chief Scientific Officer, AlphaRose Therapeutics

Pitches by:

CTNNB1 FOUNDATION — Bringing hope to children
Špela Miroševič, Ph.D.
Co-Founder & President

International SCN8A ALLIANCE
Gabi Conecker
Co-Founder & Executive Director

Track 2: Adult Neurologic & Vision-Related Disorders

Judges

- Jeffrey Cehelsky, M.B.A., CEO, trialport
- PK Tandon, Ph.D., Statistical and Development Strategy, Ultragenyx Pharmaceuticals
- Ashley Winslow, Ph.D., CEO, CSO, Odylia Therapeutics

Pitches by:

HEREDITARY NEUROPATHY FOUNDATION
Matt Jarpe, Ph.D.
Scientific Consultant

USHER SYNDROME COALITION
Krista Vasi
Executive Director

Track 3: Immune & Connective Tissue Function

Moderator: Sumaira Ahmed, Founder & Executive Director, The Sumaira Foundation

Judges

- Michael Devlin, M.B.A, Board Member & Strategic Advisor, The Sumaira Foundation
- Barbara Henry, VP, Access Experiene Team, Precision AQ
- Alison Lawton, Chair, Dianthus Therapeutics

Pitches by:

The Ehlers Danlos Society
Briony Corbet
Partnerships Manager

MIHRA
Lesley Ann Saketkoo, M.D.
Board Member

The 2026 RARE Drug Development Symposium is grateful to our valued Collaborators:

ROSAMUND STONE ZANDER and HANSJOERG WYSS TRANSLATIONAL NEUROSCIENCE CENTER
at Boston Children's Hospital

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1. Go to Settings on your phone or laptop
2. Go to Wi-Fi settings
3. Select the network "**Harvard University**"
4. You will then be prompted to register for the network - Click "**I am a Guest**"
5. You will be asked to provide the following: First Name, Last Name, Email Address
6. Accept the terms of use and click **Login**.
7. Your temporary credentials will appear - click **Login** again to connect

Guest access is time-limited. If your session expires, simply repeat the registration process.

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Visit the Catalyst Zone!

ABOUT THE CATALYST ZONE

The Catalyst Zone puts rare disease community leaders face-to-face with the partners who can turn their research vision into reality. This year's Zone features laboratory services and genetic and cell therapy to cell line development, manufacturing, contract research organizations and more!

EXHIBITORS

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CDG CARE

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Coriell Institute FOR MEDICAL RESEARCH

dyno

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Nome

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VIRALGEN

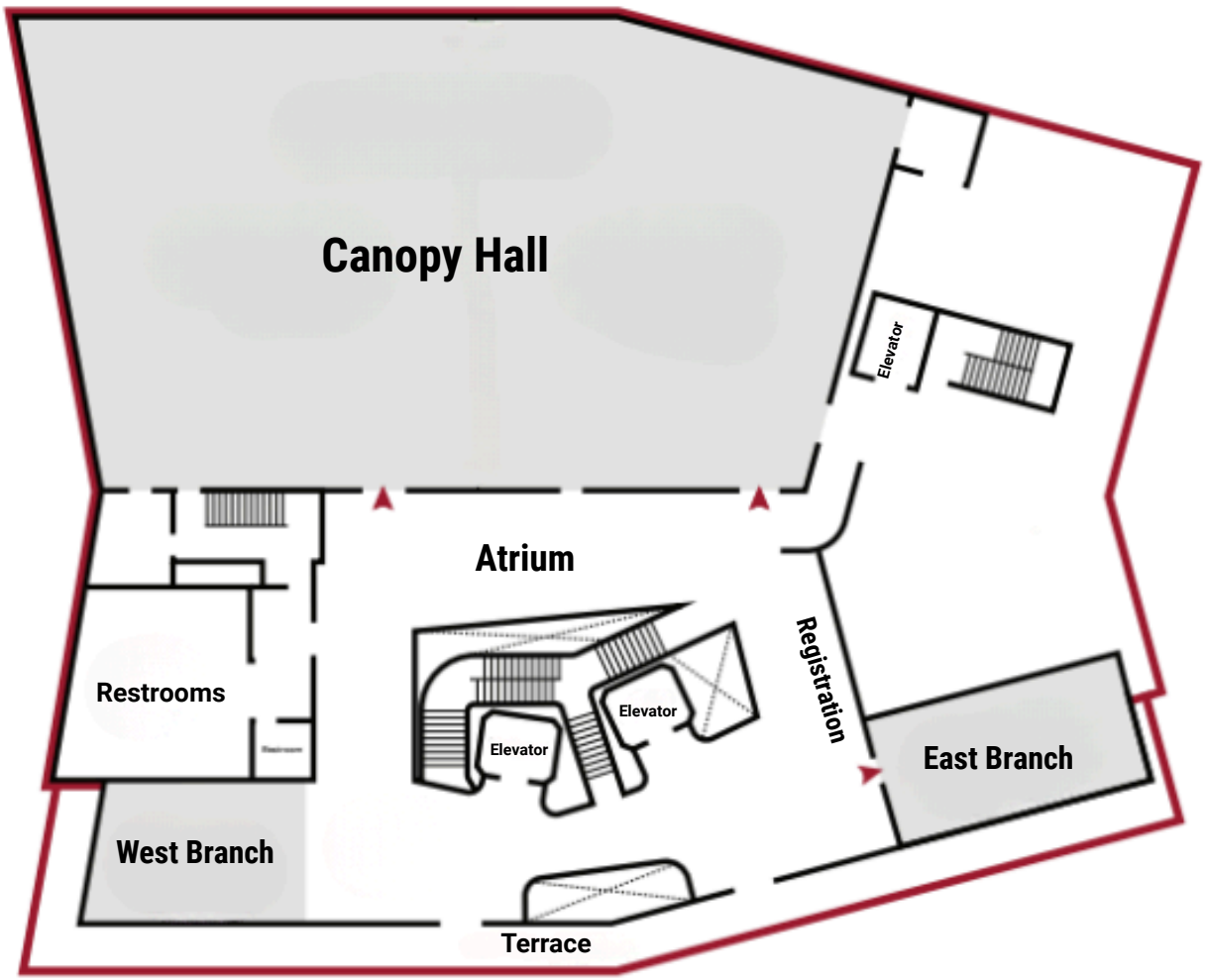
Third Floor

Canopy Hall
All General Sessions
Fireside Chats & Keynote
Pitch Session | Main Stage
Roundtable Sessions
Interactive Breakout Room

Atrium
Receptions

West Branch
Catalyst Zone

East Branch
Additional Seating



Agenda subject to change

Second Floor

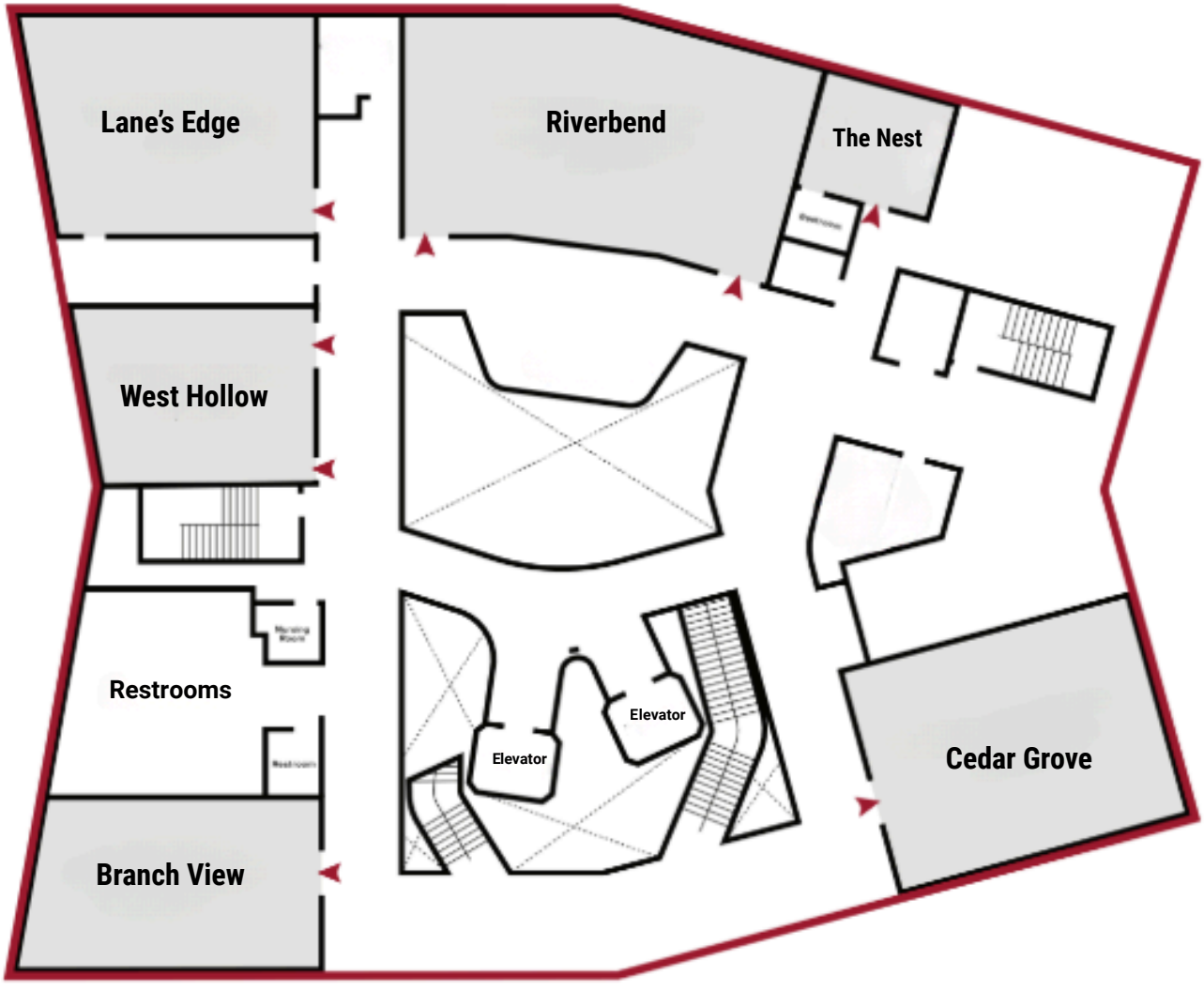
Riverbend
Interactive Breakouts
Expert Office Hours
Pitch Sessions

Lane's Edge
Interactive Breakouts
Expert Office Hours
Pitch Sessions

Cedar Grove
Interactive Breakouts
Expert Office Hours

West Hollow
Podcast Room

Branch View
Expert Office Hours



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