

RARE ADVOCACY SUMMIT

LEVEL UP YOUR ADVOCACY



Global Genes®
Allies in Rare Disease

Agenda brought to you by

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2023 RARE Advocacy Summit Agenda

**agenda subject to change
Times in PST*

TUESDAY, SEPTEMBER 19TH, 2023

11:00am – 12:00pm PT | **Freshman Orientation**

Location: Silver Pearl 3 & 2

12:15pm – 12:45pm PT | **Global Advocacy Alliance Leadership Council Meeting**

Attendance for Members

Location: Silver Pearl 1

1:00pm – 1:15pm PT | **Welcome**

Speaker: Charlene Son Rigby, CEO, Global Genes

Location: Pacific Jewel C & B

1:15pm – 2:00pm PT | **Opening Keynote: We the Scientists: How Patients Are Driving Research and Why It's Necessary for Finding Cures**

Hear how a daring team of doctors and parents of children diagnosed with the fatal genetic lysosomal storage disorder Niemann-Pick disease type C forged a new path for medicine.

Speaker: Amy Dockser Marcus, Pulitzer Prize-winning Wall Street Journal Health and Science Reporter

Location: Pacific Jewel C & B

2:15pm – 3:00pm – **TRACK 1 – EMPOWERING THE RARE INDIVIDUAL | Coordinating Complex Care**

Learn to navigate the challenges of RARE care, including the essential role of finding a physician who can serve as quarterback, how to improve communication, work across institutions, navigate insurance and sort through the pros and cons of centers of excellence.

Moderator: Effie Parks, Coordinator, Community Engagement, Global Genes

Panelists:

- Nicole Glenn, MD, Pediatrician and International Delegate, Yellow Brick Road Project
- Parvathy Krishnan, CEO, Krishnan Family Foundation

Location: Pacific Jewel C & B

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2:15pm – 3:00pm PT – **TRACK 2 – COMMUNITY AND CAPACITY BUILDING | Beginner's Guide to Community Activation**

Understand how to engage your community, welcome the newly diagnosed, bond through local and online meetups, communicate effectively, and activate people to participate in research and data collection.

Moderator: Wendy Erler, Global Head, Patient Experience, Patient Advocacy and Patient and Caregiver Insights, AstraZeneca

Panelists:

- Kasey Woleben, Founder, Rare Village
- Kim Stephens, Executive Director, Joseph Muenzer's MPS Research and Treatment Center of UNC; President, Project Alive

Location: Silver Pearl 3

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Catalyst
pharmaceuticals

2:15pm – 3:00pm PT – **TRACK 3 – BECOMING A RESEARCH READY ORGANIZATION** | **Create Your Strategy: The Rare Research Roadmap**

Learn how organizations can create a research strategy and build a toolbox of what researchers will need to progress research, and discover and advance potential therapies.

Moderator: Karmen Trzupek, MS, CGC, Senior Director, Scientific Programs, Global Genes

Panelists:

- Debbie Requesens, PhD, Jump Start Program Director, UPenn Orphan Disease Center
- Rachel Groth, PhD, VP Research, BridgeBio Pharma

Location: Silver Pearl 2

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THERAPEUTICS

2:15pm – 3:00pm PT – **TRACK 4 – RARE DISEASE MASTER CLASS: SCIENCE AND TECH INNOVATION** | **Thinking Like a Researcher: Disease Biology 101**

This session explains how genes work and what can go wrong - and how a growing toolkit of therapeutic approaches is enabling precision approaches to address the underlying molecular causes of diseases.

Presenter: Maya Chopra, MBBS, FRACP, Assistant Professor, Harvard Medical School

Location: Silver Pearl 1

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Therapeutics

3:00pm – 3:15pm PT | **Proceed to Next Session**

3:15pm – 4:00pm PT – **TRACK 1 – EMPOWERING THE RARE INDIVIDUAL** | **Pediatric to Adult Healthcare Transition: The Rare Disease Experience**

This presentation of recent research findings and personal rare disease stories highlights the unique experiences, perceptions, and support needs of patients and families during the transition-of-care process, as they confront challenges from lack of knowledge by providers and ill-equipped facilities.

Presenters:

- Kristin McKay, Executive Director, Project Alive
- Nathan Grant, Board Member, Project Alive

Location: Pacific Jewel C & B

Brought to you by
bridgebio

3:15pm – 4:00pm PT – **TRACK 2 – COMMUNITY AND CAPACITY BUILDING** | **Why Genetic and Genomic Testing is Key to Increased Diagnosis**

The costs of genetic tests are falling, and their utility is increasing. Learn about current genomic testing in clinical and research settings, find out about free programs offered by biopharma companies that help identify patients, and legislative efforts to add genetic testing to newborn screening panels.

Moderator: Maria Della Rocca, MS, PMP, Senior Director, Support & Education Programs, Global Genes

Panelists:

- Ryan Colburn, Principal, odimm inc.
- Wendy Benson, Chief Strategy and Innovations Officer, Rady Children's Hospital
- Paul Kruszka, MD, FACMG, MPH, Chief Medical Officer, GeneDx

Location: Silver Pearl 3

Brought to you by
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pharmaceutical

3:15pm – 4:00pm PT – **TRACK 3 – BECOMING A RESEARCH READY ORGANIZATION** | **Developing Scientific Expertise to Drive Research Strategy**

Learn how to find and engage scientific advisors and expert researchers, build a scientific advisory board, and recruit a full-time or shared scientific director.

Moderator: Luke Rosen, MS, Founder, KIF1A.ORG

Panelists:

- Ron Garber, Co-Founder and President, The Yaya Foundation for 4H Leukodystrophy
- Yssa DeWoody, Co-Founder, Treasurer, Director of Research, Ring 14 USA

Location: Silver Pearl 2

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3:15pm – 4:00pm PT – **TRACK 4 – RARE DISEASE MASTER CLASS: SCIENCE AND TECH INNOVATION | Small Molecules, Drug Repurposing and Biologics**

This session will explore how small molecule therapies work, what they can and can't do, and opportunities to repurpose approved therapies that already exist. We'll also consider how biologics (monoclonal antibodies and enzyme replacement therapies) work and when they might be combined with other therapies.

Session Host: Danny Levine, Life Sciences Writer; RARECast Host, Global Genes

Presenter: Alex Burgin, PhD, Director, Center for the Development of Therapeutics, Broad Institute

Location: Silver Pearl 1

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Therapeutics

4:00pm – 4:15pm PT | **Proceed to Next Session**

4:15pm – 5:00pm PT – **TRACK 1 – EMPOWERING THE RARE INDIVIDUAL | I'm an Expert, Too! Communicating with HCPs**

While people with rare diseases may be experts on their condition, doctors may not always recognize this fact. Learn how to talk to doctors, advocate as an expert in your disease, and what to do when health care providers fail to speak in understandable ways.

Moderator: Daniel DeFabio, Director, Community Engagement, Global Genes

Panelists:

- Nicole Glenn, MD, Pediatrician and International Delegate, Yellow Brick Road Project
- Kim Aldinger, PhD, Assistant Professor of Pediatrics, Seattle Children's Hospital
- Mike Porath, Founder and CEO, The Mighty

Location: Pacific Jewel C & B

Brought to you by
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4:15pm – 5:00pm PT – **TRACK 2 – COMMUNITY AND CAPACITY BUILDING | Developing Sustainable Funding Models for Your Organization**

There's no one-size-fits all solution for finding the funds that will allow your advocacy organization to survive and grow. The key is to identify a strategy that will work for you. We'll consider various models of success including events, crowdsourcing, donor relations and grants.

Moderator: Lisa Greer, Author "Philanthropy Revolution: How to Inspire Donors, Build Relationships and Make a Difference"

Panelists:

- Kari Rosbeck, President and CEO, TSC Alliance
- Mike Graglia, Managing Director, SynGAP Research Fund

Location: Silver Pearl 3

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4:15pm – 5:00pm PT – **TRACK 3 – BECOMING A RESEARCH READY ORGANIZATION | Using Data Collection to Improve Outcomes**

Find out how a well-designed data collection program can help improve clinical care, create disease progression models, identify mechanistic changes and target therapies and windows of efficacy.

Moderator: Simon Frost, CEO, Tiber Capital Group

Panelists:

- Chandler Crews, Founder, The Chandler Project
- Luke Rosen, MS, Founder, KIF1A.ORG
- Nasha Fitter, VP Real World Evidence and Ciitizen Platform, Invitae

Location: Silver Pearl 2

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4:15pm – 5:00pm PT – **TRACK 4 – RARE DISEASE MASTER CLASS: SCIENCE AND TECH INNOVATION | RNA Therapies: ASOs, mRNAs, siRNAs**

RNA therapies offer an opportunity to either disrupt the translation of a faulty gene into a pathogenic protein or provide instructions for producing a needed protein that is missing. We'll consider how these therapies work, why delivery is a critical issue, and efforts to overcome some of the challenges.

Session Host: Danny Levine, Life Sciences Writer; RARECast Host, Global Genes

Presenters:

- Chris Hart, PhD, CEO and President, Creyon Bio
- Rachel Groppo, PhD, Director, Replicon Team Lead, Janssen

Location: Silver Pearl 1

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5:15pm - 6:00pm PT | **Closing Plenary: Compelling New Models for Patient Engagement and Trial Participation**

Empowering patients to generate quality data at home.

Moderator: Morrie Ruffin, Managing Partner, Adjuvant Partners

Speakers:

- Kelly McVearry, PhD, MA, EdM, Co-Founder and CEO, Bento Biology Platforms
- Kenneth Kalunian, MD, Co-Founder Project Bento, UCSD School Of Medicine, Wolfe Family Lupus Center of Excellence
- Gini Deshpande, PhD, Founder and Chair, NuMedii AI for Drug Discovery

Location: Pacific Jewel C & B



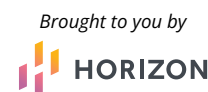
6:00pm - 6:30pm PT | **Champions of Hope - Winner Recognition and Celebration**

Join us in celebrating and honoring rare disease changemakers from advocacy, industry and research.

Speakers:

- Charlene Son Rigby, CEO, Global Genes
- Matt Flesch, Patient Advocacy, Horizon Therapeutics

Location: Pacific Jewel C & B



6:30pm - 8:30pm PT | **Evening Reception**

Location: Eventide Garden



WEDNESDAY, SEPTEMBER 20TH, 2023

6:00am - 7:00am PT | **Yoga with Mike Graglia**

***Bring Your Own Yoga Mat or Towel*

Location: Lanai Lawn

7:30am - 8:30am PT | **Corporate Alliance Leadership/Global Advocacy Alliance Leadership Networking Breakfast**

Attendance for Members Only

Location: Silver Pearl 2

7:30am - 8:30am PT | **Research Advisory Council Breakfast**

Attendance for Members Only

Location: Silver Pearl 3

7:30am - 8:30am PT | **Breakfast**

Location: Pacific Jewel A and Eventide Garden

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8:30am - 9:00am PT | **Welcome and Open Science Data Challenge**

Presentation of Winners

Speakers:

- Ravi Bhargava, MD, Roche Global Informatics, Global Medical Director, AI Digital Health
- Karmen Trzupek, MS, CGC, Senior Director, Scientific Programs, Global Genes

Location: Pacific Jewel C & B

9:00am – 9:45am PT | **Opening Plenary - Finding Your Flock: Emerging Opportunities to Accelerate Research Through Disease Community Collaborations**

Learn how rare disease communities have collaborated in ways big and small to support strategic planning, implement shared laboratory and clinical research studies, and identify and address critical challenges in therapeutic development. Concrete case examples highlight the benefits of identifying rare disease partners based on shared symptoms, shared disease biology, and organizational maturity.

Moderator: Karmen Trzupek, MS, CGC, Senior Director, Scientific Programs, Global Genes

Speakers:

- Scott Demarest, MD, Associate Professor, Children's Hospital of Colorado
- Eric Sid, MD, Program Officer, National Center for Advancing Translational Sciences Division of Rare Diseases Research Innovation, National Institutes of Health
- Jillian Shaw, PhD, Scientific Advisor, Broad Institute

Location: Pacific Jewel C & B

10:00am – 10:45am PT – **TRACK 1 – EMPOWERING THE RARE INDIVIDUAL | Managing Life Without a Diagnosis**

This session addresses challenges, questions, and opportunities for those living without a specific diagnosis. Explore genetic testing basics, research studies, support services, and finding community. Gain strategies to empower yourself or a family member in navigating life without a diagnosis.

Moderator: Michele Herndon, Program Director, Patient Navigation Program, Undiagnosed Diseases Network Foundation (UDNF)

Panelists:

- Sarah Marshall, UDN/UDNF PEER
- Stephanie Tomlinson, UDN/UDNF PEER

Location: Pacific Jewel C & B

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10:00am – 10:45am PT – **TRACK 2 – COMMUNITY AND CAPACITY BUILDING | Develop a Long-Term Growth Strategy**

Gather insights into strategic planning, recruiting active leaders and tracking success. We'll also consider how to find and build relationships with other rare disease organizations and assemble health and medical review boards.

Moderator: Tom Hume, Co-Founder, CURE JM Foundation

Panelists:

- Jennifer Sills, President and Founder, CSNK2A1 Foundation
- Rob Long, Executive Director, Uplifting Athletes

Location: Silver Pearl 3

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10:00am – 10:45am PT – **TRACK 3 – BECOMING A RESEARCH READY ORGANIZATION | Becoming Clinical Trial Ready**


Make sure you have essential elements in place to allow experimental therapies to advance to clinical trials. We'll discuss conducting natural history studies, identifying and validating biomarkers, determining appropriate endpoints, disease concept models and regulatory strategies.

Moderator: Christina Saninocencio, PhD, CPH, CNP, Founder, LGS Foundation; Lecturer, Fairfield University

Panelists:

- Kendall Davis, MPH, Director of Advocacy and Engagement, ICON, plc
- Brian Pfister, VP, Global Medical Affairs Lead, Praxis Precision Medicines

Location: Silver Pearl 2

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10:00am – 10:45am PT – **TRACK 4 – RARE DISEASE MASTER CLASS: SCIENCE AND TECH INNOVATION**
| **Gene Replacement and Gene Editing Therapies**

Gene replacement and gene editing therapies have the potential to provide a one-and-done cure for genetic diseases. We'll explore how these therapies work, when these may be appropriate, and barriers that need to be overcome.

Session Host: Danny Levine, Life Sciences Writer; RARECast Host, Global Genes

Presenters:

- Amy Raymond, PhD, PMP, Therapeutic Strategy Lead, Rare Diseases & Cellular and Genetic Medicines, Worldwide Clinical Trials
- Matthew Fuller, PhD, PMP, Executive Director, Gene Therapy Research, Ultragenyx

Location: Silver Pearl 1

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10:45am – 11:00am PT | **Proceed to Next Session**

11:00am – 11:45am PT – **TRACK 1 – EMPOWERING THE RARE INDIVIDUAL** | **Coping with the Challenge of Rare Disease - With or Without a Diagnosis**

Discover practical strategies for managing symptoms, coordinating care across many specialties, prioritizing mental health, and navigating the diagnostic journey. Hear from our panel of rare patients and mental health professionals who will share their unique experiences, insights, and resources.

Moderator: Albert Freedman, PhD, Psychologist, Rare Counseling

Panelists:

- Adam Johnson, Rare Disease DadVocate
- Caroline Cheung-Yiu, Founder, CURE
- Stephanie Yin, MS, Psychotherapist, RareMinds

Location: Pacific Jewel C & B

Brought to you by
 **bridgebio**

11:00am – 11:45am PT – **TRACK 2 – COMMUNITY AND CAPACITY BUILDING** | **Expanding Your Global Reach**

Rare diseases know no borders. To find larger patient populations and be more inclusive, it is necessary to look beyond your home country. This session will focus on how to increase your reach and navigate international research, regulatory agencies, and healthcare systems.

Moderator: James O'Brien, President/Chair, Director, CEO, International Prader-Willi Syndrome Organization

Panelists:

- Bojana Mirosavljevic, Director, Rare Disease Patient Advocacy Strategy, ICON plc
- Arik Johnson, PsyD, Chief Mission Officer, HDSA
- Michelle Davis, Executive Director, International FOP Association

Location: Silver Pearl 3

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11:00am – 11:45am PT – **TRACK 3 – BECOMING A RESEARCH READY ORGANIZATION** | **Pre-Clinical Research: Why and When to Invest**

One of the best ways for patient advocacy organizations to lay a foundation for the development of therapies is to invest in in-vivo and in-vitro models and tools. This session will explore essential tools for basic research including animal models, induced pluripotent stem cells, and next generation sequencing.

Moderator: Yael Weiss, MD, PhD, CEO, Mahzi Therapeutics

Panelists:

- Gene Yeo, PhD, MBA, Professor of Cellular and Molecular Medicine, University of California, San Diego
- Jeffrey Kaufman, Executive Director, Adenoid Cystic Carcinoma Research Foundation

Location: Silver Pearl 2

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11:00am – 11:45am PT – **TRACK 4 – RARE DISEASE MASTER CLASS: SCIENCE AND TECH INNOVATION**

| **Spinal Muscular Atrophy Case Study**

Spinal muscular atrophy (SMA) has benefitted from therapeutic innovations including an antisense therapy, a gene therapy, and a small molecule therapy. This case study will explore different therapeutic strategies and their pros and cons in the real world.

Session Host: Danny Levine, Life Sciences Writer; RARECast Host, Global Genes

Presenter: Ying Qian, MPH, Director, SMA Foundation

Location: Silver Pearl 1

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11:45am – 12:00pm PT | **Proceed to Next Session**

12:00pm – 12:45pm PT – **TRACK 1 – EMPOWERING THE RARE INDIVIDUAL | Grieving Diagnosis, Lack of Diagnosis and Loss**

When the life you've anticipated is forever changed by rare disease, there is often an overwhelming sense of loss. This session will discuss managing many forms of grief, learning to live well in your new reality and how to find organizations and resources that can help you cope.

Moderator: Daniel DeFabio, Director, Community Engagement, Global Genes

Panelists:

- Ronda Thorington, LPC, Clinician, Parent Coach
- Mindy Pace, Patient, Caregiver, Peer Support Leader, Give an Hour
- Kristin McKay, Executive Director, Project Alive
- Suzanne Edison, MA, MFA, Mental Health Coordinator, CURE JM Foundation

Location: Pacific Jewel C & B

Brought to you by
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12:00pm – 12:45pm PT – **TRACK 2 – COMMUNITY AND CAPACITY BUILDING | Working Groups on Building Your Foundation's Strategy**

Success comes with planning. In this hands-on session, working groups will help organizations consider ways to reach global audiences, build community, connect families to financial support, grow staff, attract volunteers, raise money to advance your mission, create mental health support programs and more.

Location: Silver Pearl 3

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12:00pm – 12:45pm PT – **TRACK 3 – BECOMING A RESEARCH READY ORGANIZATION | Crafting a Data Sharing Strategy**

Gathering data is not enough. To leverage that work requires tools and technology to share it safely and privately, as well as enable its use with other data sets. This session will discuss data sharing and data federation, how to evaluate options, and the critical responsibilities around data governance.

Moderator: Sanath Kumar Ramesh, Founder and CEO, Open Treatments Foundation

Panelists:

- Nasha Fitter, VP, Real World Evidence and Ciitizen Platform, Invitae
- Alex Betourne, PhD, PharmD, Scientific Director for Rare Diseases Cures Accelerator, C-path
- Vanessa Vogel-Farley, Senior Director, Research & Data Analytics, Global Genes

Location: Silver Pearl 2

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 **Sangamo**
THERAPEUTICS

12:00pm – 12:45pm PT – **TRACK 4 – RARE DISEASE MASTER CLASS: SCIENCE AND TECH INNOVATION | Session 1: Expert Office Hours – *Reservation 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 August for registered attendees.

Location: Silver Pearl 1

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12:45pm – 2:00pm PT | **Lunch and Exhibits**

Lunch Location: Pacific Jewel A and Eventide Gardens; Exhibits: Pacific Jewel and Silver Pearl Foyers

2:00pm – 2:45pm PT – **TRACK 1 – LEGISLATIVE ACTION | Emerging Rare Policy Issues**

Join the EveryLife Federal & State Policy Team and other leading community members for an overview on current policy issues that impact the rare disease community.

Moderator: Shannon von Felden, Senior Director of Advocacy, EveryLife Foundation

Panelist: Emily Stauffer, State Policy Manager, EveryLife Foundation for Rare Diseases

Location: Pacific Jewel C & B

Brought to you by



2:00pm – 2:45pm PT – **TRACK 2 – COMMUNITY AND CAPACITY BUILDING | Working Nicely in the Same Sandbox - How Patient Groups Can Work Together, Not Against Each Other**

Three organization leaders in the MPS community have developed a collaborative relationship by focusing on shared goals and passion for the patient community. Find out how foundations can work together and what you can do as a leader to produce this cohesive relationship in your community.

Moderator: Kim Stephens, Executive Director, Joseph Muenzer's MPS Research and Treatment Center of UNC; President, Project Alive

Panelists:

- Kristin McKay, Executive Director, Project Alive
- Mark Dant, Executive Director, The Ryan Foundation for Rare Disease Research

Location: Silver Pearl 3

Brought to you by



2:00pm – 2:45pm PT – **TRACK 3 – BECOMING A RESEARCH READY ORGANIZATION | Power of Partnering: Children's Hospital Colorado**

Learn about collaborative research models and case studies involving Children's Hospital Colorado, including its Neurogenetics Clinic in partnership with STXBP1 Foundation, SLC6A1 Connect, Ring14 USA and Project 8p.

Moderator: Scott Demarest, MD, Associate Professor, Pediatrics and Neurology, Children's Hospital Colorado

Panelists:

- Melissa Hioco, Development Director, STXBP1
- Yssa DeWoody, PhD, Co-Founder, Treasurer, Director of Research, Ring 14 USA

Location: Silver Pearl 2

Brought to you by



2:00pm – 2:45pm PT – **TRACK 4 – RARE DISEASE MASTER CLASS: SCIENCE AND TECH INNOVATION | How Companies Decide Which Therapies to Pursue**

Why do some therapies advance to clinical trials and others are discontinued? What attracts a company to pursue research on a particular project? Find out the decision factors that companies use to answer these questions.

Moderator: Walt Kowtoniuk, PhD, Venture Partner, Third Rock Ventures

Panelists:

- Steve Rodems, PhD, VP of Research and Nonclinical Development, Traveo Therapeutics
- Neil Kumar, PhD, Co-Founder and CEO, BridgeBio
- Alaa Hamed, MD, MPH, MBA, Global Head of Medical for Rare Diseases and Rare Blood Disorders, Sanofi

Location: Silver Pearl 1

Brought to you by



2:45pm – 3:00pm PT | **Proceed to Next Session**

3:00pm – 3:45pm PT – **TRACK 1 – LEGISLATIVE ACTION | The Cost of Delayed Diagnosis - What Came After the National Economic Burden of Rare Disease Study?**

EveryLife Foundation's National Economic Burden of Rare Disease Study released critical data on the economic cost of 379 rare diseases and estimated the total cost of rare disease in 2019 at nearly \$1 trillion in the US. This session includes a high-level overview of the data and provides the groundwork for what is coming next in the Cost of Delayed Diagnosis in Rare Disease: A Health Economics Study.

Moderator: Shannon von Felden, Senior Director of Advocacy, EveryLife Foundation

Speaker: Kim Stephens, Executive Director, Joseph Muenzer's MPS Research and Treatment Center of UNC

Location: Pacific Jewel C & B

Brought to you by
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3:00pm – 3:45pm PT – **TRACK 2 – COMMUNITY AND CAPACITY BUILDING | Supporting Your Rare Community's Mental Health**

Living with a rare disease brings unique mental health challenges. Patients, caregivers and advocates often experience high levels of stress, live with a great deal of uncertainty, and feel isolated. Our panel of mental health professionals, industry partners and rare parents will discuss mental health challenges faced by rare families and highlight support programs provided by rare disease advocacy organizations to meet the needs of their communities.

Moderator: Albert Freedman, PhD, Psychologist, Rare Counseling

Panelists:

- Maria Picone, Founder/CEO, TREND Community
- Tom D'Amato, Director of Patient Advocacy, Horizon Therapeutics
- Stephanie Yin, MS, Psychotherapist, RareMinds
- Wendy Erler, Global Head, Patient Experience, Patient Advocacy and Patient and Caregiver Insights, AstraZeneca

Location: Silver Pearl 3

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3:00pm – 3:45pm PT – **TRACK 3 – BECOMING A RESEARCH READY ORGANIZATION | Power of Partnering: Rosamund Stone Zander Translational Research Neuroscience Center**


Learn about collaborative research models and case studies involving advocacy groups and Rosamund Stone Zander Translational Research Neuroscience Center at Boston Children's Hospital.

Moderator: Kira Dies, ScM, CGC, Executive Director, Rosamund Stone Zander Translational Neuroscience Center, Boston Children's Hospital (BCH)

Panelists:

- Maya Chopra, MBBS, FRACP, Assistant Professor, Harvard Medical School
- Jamie Korin Capal, MD, Associate Professor of Pediatrics and Neurology, UNC Chapel Hill and Carolina Institute for Developmental Disabilities
- Tiina Urv, PhD, Program Director, National Center for Advancing Translational Sciences, Rare Diseases Clinical Research Network, National Institutes of Health

Location: Silver Pearl 2

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3:00pm – 3:45pm PT – **TRACK 4 – RARE DISEASE MASTER CLASS: SCIENCE AND TECH INNOVATION | 7 Ways to Engage the FDA**

Communicating with regulatory authorities can help avoid missteps in the research process that are costly in terms of both time and money. Learn when and how to bring the patient voice into the discussion.

Moderator: Lea Ann Browning-McNee, MS, Director of Communications and Stakeholder Engagement, Reagan-Udall Foundation

Panelists:

- Mark Dant, Founder, Ryan Foundation
- Kari Rosbeck, President and CEO, TSC Alliance
- Patti Engel, RN, BSN, President and CEO, Engage Health

Location: Silver Pearl 1

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Therapeutics

3:45pm – 4:00pm PT | **Proceed to Next Session**

4:00pm – 4:45pm PT – **TRACK 1 – EMPOWERING THE RARE INDIVIDUAL | Activating Advocacy for Kids, Teens and Youth**

Are you trying to activate your younger advocates? Do you dream of hosting a Hill Day just for kids, teens, or young adults? Members of the Young Adult Rare Coalition can help. Join a discussion on the value of engaging young people and hear tips on how to host a Hill Day or instill programming for a younger demographic.

Moderator: Lindsey Cundiff, Associate Director of Patient Engagement, EveryLife Foundation for Rare Diseases

Panelists:

- Jenna Heilman, Executive Director, Huntington's Disease Youth Organization (HDYO)
- Lisa Facciolla, Community Engagement Specialist, US Hereditary Angioedema Association
- Sati Cooper-McCann, Young Advocate

Location: Pacific Jewel C & B

Brought to you by


4:00pm – 4:45pm PT – **TRACK 2 – COMMUNITY AND CAPACITY BUILDING | Financial Support for Your Community Members**


Rare disease has a big impact on your financial well-being. This session will highlight resources available to help patients and caregivers with healthcare costs, disability, and tax-advantaged savings accounts for individuals with disabilities known as ABLE accounts.

Moderator: Mary Morlino, Manager, RARE Concierge Patient Services, Global Genes

Panelists:

- Brian Altounian, Financial Planner and Special Needs Dad, Just Two Dads Podcast
- Tiara Green, MEd, Interim CEO, Accessia Health
- Erin Bradshaw, Chief of Mission Delivery, Patient Advocate Foundation

Location: Silver Pearl 3

Brought to you by


4:00pm – 4:45pm PT – **TRACK 3 – BECOMING A RESEARCH READY ORGANIZATION | Birds of a Feather Networking Session**

Increase your impact and make your funding dollars go further by collaborating with related disease communities and aligned organizations. Find your flock in this facilitated networking session.

Location: Silver Pearl 2

Brought to you by


4:00pm – 4:45pm PT – **TRACK 4 – RARE DISEASE MASTER CLASS: SCIENCE AND TECH INNOVATION | Session 2: Expert Office Hours – *Reservation 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 August for registered attendees.

Location: Silver Pearl 1

Brought to you by


5:00pm – 6:00pm PT | **Closing Plenary: Strengthened by Our Past, Working Together to Create Our Future**

Speakers:

- Joni L Rutter, PhD, Director, National Institutes of Health (NIH) National Center for Advancing Translational Sciences (NCATS)
- Charlene Son Rigby, CEO, Global Genes

Location: Pacific Jewel C & B

6:00pm – 7:00pm PT | **Global Advocacy Alliance Networking Reception**

Attendance for GAA Members

Location: Eventide Garden

7:15am – 9:30am PT | **RARE-X Users Forum**

Attendance for RARE-X Users Only

Location: Shorebreak

7:30am – 9:30am PT | **Corporate Alliance Breakfast Meeting**

Attendance for Members Only

Location: Pacific Jewel C & B

7:30am – 9:30am PT | **Scientific Advisory Board Meeting**

Attendance for SAB Members Only

Location: Tidepool 2

7:30am – 9:30am PT | **Breakfast**

Location: Pacific Jewel A and Eventide Garden

Brought to you by



10:00am – 11:00am PT | **Plenary Panel: It's All of Us Against the Disease: The Future of Research Collaborations and Disease Community Consortia**

Data pooling and aggregation of multi-sourced data can speed progress in research. Challenges to this approach may not stem not from technology or science, but from human nature. Hear directly from change agents driving these multi-stakeholder consortiums about their challenges and successes.

Moderator: Katherine Maynard, Partner, PWR

Panelists:

- Ryan Colburn, Principal, odimm, inc
- Carole Bakhos, Project Director, Jordan's Guardian Angels
- Adam Staffaroni, PhD, Assistant Professor, University of California, San Francisco

Location: Pacific Jewel C & B

11:00am – 12:00pm PT | **Closing Plenary Keynote: Rebel Health: How Rare Disease Communities Lead the Revolution**

Speaker: Susannah Fox, Author, Digital Health Expert, and Advisor for the Patient-Led Research Collaborative
"Health care needs to invite the rebels inside, to connect them with the resources they need to test and scale their ideas," says Susannah Fox, author of *Rebel Health*, a how-to guide that will help patients, survivors and caregivers learn new skills and deploy them for their loved ones.

Location: Pacific Jewel C & B

12:00pm – 12:15pm PT | **Closing Remarks**

Speaker: Charlene Son Rigby, CEO, Global Genes

Location: Pacific Jewel C & B