2023 RARE Advocacy Summit Agenda

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 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, Community Engagement Coordinator, Global Genes
Panelists:
• Nicole Glenn, MD, Pediatrician and International Delegate, Yellow Brick Road Project
• Parvathy Krishnan, CEO, Krishnan Family Foundation
• Nathan Grant, Board Member, Project Alive
Location: Pacific Jewel C & B

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 and President, Project Alive
Location: Silver Pearl 3
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 of 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

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

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

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 of 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

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
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, RARECast Host, Global Genes

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

**Location:** Silver Pearl 1

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

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

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

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, RARECast Host, Global Genes

**Presenters:**
- Chris Hart, PhD, CEO and President, Creyon Bio
- Rachel Groppo, PhD, Director, Replicon Team Lead, Jannsen

**Location:** Silver Pearl 1
Closing Plenary: Compelling New Models for Patient Engagement and Trial Participation
Empowering patients to generate quality data at home.

Speakers:
- Kelly McVearry, PhD, MA, EdM, Co-Founder and CEO, Bento Biology Platforms
- Morrie Ruffin, Managing Partner, Adjuvant Partners

Location: Pacific Jewel C & B

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

Evening Reception
Location: Eventide Gardens

WEDNESDAY, SEPTEMBER 20TH, 2023

Yoga with Mike Graglia
**Bring Your Own Yoga Mat or Towel
Location: Lanai Lawn

Corporate Alliance Leadership/Global Advocacy Alliance Leadership Networking Breakfast
Attendance for Members
Location: Pacific Jewel C&B

Research Advisory Council Breakfast
Attendance for Members
Location: Silver Pearl 3

Breakfast
Location: Pacific Jewel A and Eventide Garden

Welcome and Open Science Data Challenge
Presentation of Winners

Speakers:
- Fanny Sie, MMI, AI and Digital Health, Roche
- Karmen Trzupek, MS, CGC, Senior Director of Scientific Programs, Global Genes

Location: Pacific Jewel C&B

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 of 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
- Suki Bagal, MD, MPH – Global Lead for Medical Affairs and Strategy, Vertex
- 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:
Panelists:
• Adaline Dunnberg, MS, Director of Operations, Undiagnosed Diseases Network Foundation (UDNF)
• Sarah Marshall, UDN/UDNF PEER
• Stephanie Tomlinson, UDN/UDNF PEER
• Michele Herndon, Parent Advocate
Location: Pacific Jewel C&B

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

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 of LGS Foundation and Lecturer, StonyBrook 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

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, RARECast Host, Global Genes
Presenters:
• Amy Raymond, PhD, PMP, Therapeutic Strategy Lead, Worldwide Clinical Trials
• Matthew Fuller, PhD, PMP, Executive Director, Gene Therapy Research, Ultragenyx
Location: Silver Pearl 1

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

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 Mirosvavljevic, 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

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, ED, Adenoid Cystic Carcinoma Research Foundation
Location: Silver Pearl 2

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, RARECast Host, Global Genes
Presenter: Ying Qian, MPH, Director, SMA Foundation
Location: Silver Pearl 1
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

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

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 of Research and Data Analytics, Global Genes
**Location:** Silver Pearl 2

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

12:45pm - 2:00pm PT | Lunch and Exhibits
**Location:** Pacific Jewel A and Eventide Gardens

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:** Dylan Simon, Director of Policy, EveryLife Foundation for Rare Diseases
**Panelist:** Emily Stauffer, State Policy Manager, EveryLife Foundation for Rare Diseases
**Location:** Pacific Jewel C&B
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, and 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

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

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 and what you can do to secure protect intellectual property rights.

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

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

**Location:** Silver Pearl 1

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:** Dylan Simon, Director of Policy, EveryLife Foundation for Rare Diseases

**Location:** Pacific Jewel C&B
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, 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

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

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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 for Huntington’s Disease Youth Organization (HDYO)
- Lisa Facciolla, Community Engagement Specialist for US Hereditary Angioedema Association
- Sati Cooper-McCann, Young Advocate

**Location:** Pacific Jewel C&B
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, Rare Concierge Patient Services Manager, Global Genes

**Panelists:**
- Brian Altounian, Financial Planner and Special Needs Dad, Just Two Dads Podcast
- Tiara Green, MSEd, Program Manager, Accessia Health
- Erin Bradshaw, Chief of Mission Delivery, Patient Advocate Foundation

**Location:** Silver Pearl 3

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

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

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 Members

**Location:** TBA

**THURSDAY, SEPTEMBER 21ST, 2023**

7:15am - 9:30am PT | RARE-X Users Forum
Attendance for Members

**Location:** TBA

7:30am - 9:30am PT | Corporate Alliance Breakfast Meeting
Attendance for Members

**Location:** TBA

7:30am - 9:30am PT | Scientific Advisory Board Meeting
Attendance for Members

**Location:** TBA

7:30am - 9:30am PT | Breakfast & Exhibits

**Location:** Pacific Jewel A and Eventide Gardens
10:00am - 11:00am PT | **Plenary Panel: It’s All of Us Against the Disease: The Future of Research Collaborations and Disease Community Consortions**

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 consortions 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, UCSF

**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