AGENDA

Membership Meetings – Wednesday, September 25, 2024

1:00 PM – 5:00 PM
Registration
West Foyer - Ballroom Level

12:00 PM – 3:00 PM
Corporate Alliance Meeting (Members Only)
Lunch and Transportation included thanks to our friends at Children's Mercy Hospital
Children's Mercy Hospital

4:00 PM – 5:30 PM
Freshman Orientation
Sponsored by ALEXION AstraZeneca Rare Disease
City Beautiful B/ C

5:30 PM – 6:00 PM
Global Advocacy Alliance Networking (GAA Members Only)
City Beautiful A

RARE Advocacy Summit – Thursday, September 26, 2024

7:00 AM - 5:00 PM
Registration
West Foyer - Ballroom Level

8:00 AM – 5:00 PM
Exhibitors
City Beautiful Foyers

7:30 AM - 8:45 AM
Breakfast
Sponsored by TRAVERE THERAPEUTICS
Neptune Ballroom

8:00 AM - 9:00 AM
Global Advocacy Alliance Breakfast (GAA Members Only)
Sponsored by ucb Inspired by patients. Driven by science.
Muse

9:00 AM - 10:00 AM
A Father and Leader Who Transformed How We Think About Rare Disease
John Crowley's quest to save his children, resulting in a breakthrough therapy for Pompe disease, has inspired hope among rare disease patients and advocates for decades. Hear how John is now helping rare disease families blaze a path forward in his new role as President & CEO of the Biotechnology Innovation Organization (BIO), the world's largest biotechnology advocacy organization.

- John Crowley, J.D., President and CEO, Biotechnology Innovation Organization
- Charlene Son Rigby, CEO, Global Genes

City Beautiful D/E

Agenda subject to change

10:00 AM

RARElу Told Stories film screening: *"Rarely Have You Seen a Fox Like This"*

by Claudia Parker, Author, FOXP1 Parent and Advocate

City Beautiful D/E

10:15 AM – 11:15 AM

Track Sessions and Expert Office Hours

10:15 AM – EMPOWERING THE RARE INDIVIDUAL

Sponsored by ALEXION AstraZeneca Rare Disease

City Beautiful A

Living with Visible and Invisible Disabilities

Explore how to take back your power with tips for allies, what to do when people miss the mark, and understanding "disabilities as dynamic".

- **Moderator:** Mackenzie Abramson, Senior Manager, Research Program Communications, Global Genes
- Kendra Gottsleben, Marketing Communication Specialist, Center for Disabilities, Sanford School of Medicine, University of South Dakota
- Richie Kahn, Clinical Researcher, Co-founder and COO, Canary Advisors
- Soniya Vadher-Patel, Social Media Influencer, Entrepreneur and Mental Health Advocate

10:15 AM – COMMUNITY AND CAPACITY BUILDING

Sponsored by Chiesi global rare diseases

City Beautiful D/E

Think Like a Start-Up: Grow your Patient Advocacy Group

Discover principles of fast organization building: how to create scalable and sustainable models, collect metrics, learn, adapt and adopt legal structures to protect your IP.

- **Moderator:** Charlene Son Rigby, CEO, Global Genes
- Michael Hund, CEO, EB Research Partners
- Sunitha Malepati, Founder, Buffalo Initiative and Vice President, CACNA1A Foundation
- Mark Wilhelm, Partner, Troutman Pepper

10:15 AM – GROWING YOUR ORGANIZATION'S RESEARCH READINESS

Sponsored by ucb Inspired by patients. Driven by science.

City Beautiful B/C

Know (and Build) Your Strength as a Research Partner

Join a hands-on workshop featuring the FasterCures Research Partnership Maturity Model. Learn about the four domains of research capacity, and identify next steps to increase your organization's partnership value.

- **Facilitator**: Raymond Puerini, Associate Director, FasterCures

10:15 AM – EXPERT OFFICE HOURS

Sponsored by ACADIA

Muse

Expert Office Hours - *By Appointment Only*

Attendees can meet one-on-one with leaders, industry members, researchers, and healthcare professionals to gain insights on topics like building capacity, advancing research, supporting communities, and avoiding costly detours. These 15-minute sessions are reserved in advance through the Attendee Hub event app.

11:15 AM – 11:30 AM

Session Transition / Break

City Beautiful Foyers

Agenda subject to change

11:30 AM – 12:30 PM

Track Sessions and Expert Office Hours

City Beautiful A

11:30 AM – EMPOWERING THE RARE INDIVIDUAL

Sponsored by ALEXION AstraZeneca Rare Disease

Help for the Rare Caregiver

Helping the helper is essential. We'll look at supports that provide the most benefit as well as research that shows what works, and provide actionable advice to ease caregiver burdens.

- **Moderator:** Megan Nolan, Founder, Rare Parenting
- Daniel DeFabio, Director, Community Engagement, Global Genes
- Bridgette Kelleher, Ph.D., Associate Professor, Psychological Services, Purdue University
- Bret Koncak, Rare Dad, Co-founder, mejo

City Beautiful D/E

11:30 AM – COMMUNITY AND CAPACITY BUILDING

Sponsored by Chiesi global rare diseases

Activate Your Community

Get expert tips on building connections, attracting and managing volunteers, creating lieutenants and outreach through community and corporate partnerships.

- **Moderator:** Effie Parks, Rare Mom; Podcast Host, Once Upon a Gene
- Lara Bloom, President and CEO, Ehlers Danlos Society
- Samantha Charleston, Director, Programs and Community Engagement, Orphan Disease Center, University of Pennsylvania
- Rob Long, Executive Director, Uplifting Athletes
- Luke Rosen, Co-Founder, KIF1A.org

City Beautiful B/C

11:30 AM – GROWING YOUR ORGANIZATION'S RESEARCH READINESS

Sponsored by ucb Inspired by patients. Driven by science.

Unlocking the Future: The Role of Natural History Studies

Hear about best practice and case studies for improving patient outcomes, uncovering insights on disease and treatment, and identifying what is acceptable to the U.S. Food and Drug Administration (FDA).

- **Moderator:** Deena Chisholm, Ph.D., Research Engagement Director, TANGO2 Research Foundation
- Lea Ann Browning-McNee, Director of Communication and Engagement, Reagan-Udall Foundation for the U.S. FDA
- Elizabeth Cassidy, Research Project Manager, Tuberous Sclerosis Alliance
- Kasha Morris, Co-Founder, TANGO2 Research Foundation

Muse

11:30 AM – EXPERT OFFICE HOURS

Sponsored by Amicus Therapeutics

Expert Office Hours - *By Appointment Only*

Attendees can meet one-on-one with leaders, industry members, researchers, and healthcare professionals to gain insights on topics like building capacity, advancing research, supporting communities, and avoiding costly detours. These 15-minute sessions are reserved in advance through the Attendee Hub event app.

12:30 PM – 1:50 PM

Lunch and Poster Presentations

Sponsored by AVIDITY BIOSCIENCES

Neptune Ballroom

Agenda subject to change

2:00 PM – 3:00 PM
Track Sessions and Expert Office Hours

2:00 PM – EMPOWERING THE RARE INDIVIDUAL

Sponsored by ALEXION AstraZeneca Rare Disease

City Beautiful A

Medical Gaslighting and Post Traumatic Stress Disorder (PTSD)
Medical outliers are sometimes doubted or dismissed. Learn what gaslighting is, what to do about it, and the impact of PTSD.

- **Moderator:** Mackenzie Abramson, Senior Manager, Research Program Communications, Global Genes
- Amanda Griffith-Atkins, Marriage and Family Therapist, Founder, Amanda Atkins Counseling Group
- Carla D'Imperio, Family Support Specialist, Phelan-McDermid Syndrome Foundation
- Suzanne Edison, Mental Health Coordinator, CureJM

2:00 PM – COMMUNITY AND CAPACITY BUILDING

Sponsored by Chiesi global rare diseases

City Beautiful D/E

Understand Newborn Sequencing and Screening (NBS)
NBS can be a path to both diagnosis and new members. Learn why genetic diagnosis is key, how to create infrastructure to support diagnosis, and how to insure access to services and equity.

- **Moderator:** Annie Kennedy, Chief of Policy, Advocacy and Patient Engagement, EveryLife Foundation for Rare Diseases
- Wendy Chung, M.D., Ph.D., Chief of Pediatrics, Boston Children's Hospital
- Stacey Seeloff, Director, Strategic Alliances, Rady Children's Institute for Genomic Medicine
- Brooke Thomas, Chief Programs Officer, Alabama Rare

2:00 PM – GROWING YOUR ORGANIZATION'S RESEARCH READINESS

Sponsored by ucb Inspired by patients. Driven by science.

City Beautiful B/C

A Guide to Biomarkers and Outcome Measures
How are biomarkers and outcome measures different? How can biomarkers be used as surrogate endpoints? And what is the role of Patient Advocacy Groups in identifying them?

- **Moderator**: Terry Jo Bichell, Ph.D., Founder, COMBINEDBrain
- Pat Furlong, Founding President and CEO, Parent Project Muscular Dystrophy
- Celia Goeldner, Clinical Expert Team Leader, Clinical Scientist, Roche

2:00 PM – EXPERT OFFICE HOURS

Sponsored by biocryst

Muse

Expert Office Hours - *By Appointment Only*
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3:00 PM – 3:15 PM
Session Transition / Break

City Beautiful Foyers

Agenda subject to change

3:15 PM - 4:15 PM

Track Sessions and Expert Office Hours

3:15 PM – EMPOWERING THE RARE INDIVIDUAL

City Beautiful A

Sponsored by

ALEXION AstraZeneca Rare Disease

My Genetic Test Says VUS: What's Next?

What does "variants of unknown significance (VUS)" mean? When and how do you pursue reanalysis? Are these variants different for marginalized populations? Find out.

- **Moderator:** Abigail Sveden, Genetic Counselor, Boston Children's Hospital
- Dustin Baldridge, M.D., Ph.D., Assistant Professor, Washington University in St. Louis
- Christina Gurnett, M.D., Ph.D., Director, Division of Pediatric and Developmental Neurology, Washington University in St. Louis
- Michele Herndon, Program Director, Undiagnosed Diseases Network Foundation

3:15 PM – COMMUNITY AND CAPACITY BUILDING

City Beautiful D/E

Sponsored by

Chiesi global rare diseases

Disability and Access

This panel will focus on best practices for ADA website compliance, accessibility for research and clinical trials, and how best to support the needs of your community.

- **Moderator:** Kristin Smedley, Founder and CEO, Curing Retinal Blindness Foundation
- Rachelle Cook, Rare Disease Advocate
- Kendra Gottsleben, Marketing Communication Specialist, Center for Disabilities, Sanford School of Medicine, University of South Dakota
- Lori Renna Linton, Rare Disease Advocate and Filmmaker

3:15 PM – GROWING YOUR ORGANIZATION'S RESEARCH READINESS

City Beautiful B/C

Sponsored by

ucb Inspired by patients. Driven by science.

Quick Wins & Long-Term Investments in Data

Use the power of data to make a difference for patients and improve life now for your community members. Maximize users for broadest impact, move the needle with in-home collection and use data to create and maintain clinical guidelines.

- **Moderator:** Zohreh Talebizadeh, Ph.D., Senior Director, RARE-X Research Program, Global Genes
- Wendy Chung, M.D., Ph.D., Chief of Pediatrics, Boston Children's Hospital
- Marianne Clancy, Executive Director, Cure HHT
- Jennifer Foss-Feig, Ph.D., Principal Investigator, Foss-Feig Lab, Icahn School of Medicine, Mount Sinai

3:15 PM – EXPERT OFFICE HOURS

Muse

Sponsored by

bridgebio

Expert Office Hours - *By Appointment Only*

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4:15 PM – 4:30 PM

Session Transition / Break

City Beautiful Foyers

4:30 PM – 5:30 PM

Expanding Momentum at NCATS and the FDA

Learn about innovations and collaborations designed to streamline therapy development and improve outcomes for patients.

- **Moderator:** Charlene Son Rigby, CEO, Global Genes
- Kerry Jo Lee, Ph.D., Associate Director for Rare Diseases, Center for Drug Evaluation and Research (CDER), U.S. Food and Drug Administration (FDA)
- Joni Rutter, Ph.D., Director, National Center for Advancing Translational Sciences (NCATS), National Institutes of Health (NIH)

City Beautiful D/E

5:30 PM

RAREly Told Stories film screening: *"Life Through a Lens"*

by Jeff Levenson, DDS, Co-President, Adult Polyglucosan Body Disease (APBD) Research Foundation and Ronete Levenson

City Beautiful D/E

5:30 PM – 8:30 PM

RARE KC Welcome Reception

Hosted by RareKC

Event Deck 7th Floor Outside

RARE Advocacy Summit – Friday, September 27, 2024

7:00 AM - 5:00 PM

Registration

West Foyer - Ballroom Level

8:00 AM – 8:00 PM

Exhibitors

City Beautiful Foyers

7:30 AM - 8:45 AM

Breakfast

Sponsored by Biogen

Neptune Ballroom

9:00 AM - 10:00 AM

Opening Plenary: More Than We Expected: Five Years with a Remarkable Child

This moving memoir, written by a rare dad, is a story of finding strength in unexpected places, reminding us that being human is a miraculous gift, however fleeting.

- James G. Robinson, Author and Director of Data Products, New York Times

City Beautiful D/E

10:00 AM

Rare Pediatric Legislative Activity

- **Facilitator:** Annie Kennedy, Chief of Policy, Advocacy and Patient Engagement, EveryLife Foundation for Rare Diseases

City Beautiful D/E

Agenda subject to change

10:15 AM – 11:15 AM
Track Sessions and Expert Office Hours

City Beautiful A

10:15 AM – EMPOWERING THE RARE INDIVIDUAL

Sponsored by

ALEXION
AstraZeneca Rare Disease

Rare Siblings
There are unique concerns around siblings to someone who has a rare disease. Parents often give them less attention or focus. The siblings feel they must do more than their typical peers due to the medical care needs in their home. We'll hear from rare siblings on how to manage the unique role.

- **Moderator:** Noah Seidman, Rare Sibling
- Matt Flesch, Vice-President, Communications & Patient Advocacy, Amgen
- Felix Townsin, Rare Sibling

City Beautiful D/E

10:15 AM – COMMUNITY AND CAPACITY BUILDING

Sponsored by

Chiesi
global rare diseases

Empowering Advocacy: Federal, State and Patient Insights
Join the EveryLife Foundation for Rare Diseases for an update on advocacy opportunities at the state and federal levels. Hear directly from advocate panelists for tips and tools to take home.

- James Griffin, Advocate
- Paloma Juarez, Advocate
- Stephanie Riordan, Director of Patient Programs, EveryLife Foundation for Rare Diseases
- Shannon von Felden, Senior Director of Advocacy, EveryLife Foundation for Rare Diseases
- Anneliese Williams, Advocate

City Beautiful B/C

10:15 AM – GROWING YOUR ORGANIZATION'S RESEARCH READINESS

Sponsored by

ucb Inspired by patients.
Driven by science.

Overcoming the "Valley of Death" in Research
Navigating from bench to bedside is one of the greatest challenges rare disease leaders face. What do PAGs need to do to de-risk drug development for partners? What internal and external expertise can you tap? Is venture philanthropy an option?

- **Moderator:** Danny Levine, Life Sciences Writer, Global Genes
- Sharie Haugabook, Ph.D., Senior Project Manager and Drug Development Lead, Therapeutics for Rare and Neglected Disorders (TRND), National Institutes of Health (NIH)
- Christopher Hopkins, Ph.D., CEO, Devine Bio
- Michael Hund, CEO, EB Research Partnership
- Yael Weiss, Ph.D., CEO, Mahzi Therapeutics

Muse

10:15 AM – EXPERT OFFICE HOURS

Sponsored by

IPSEN
Innovation for patient care

Expert Office Hours - *By Appointment Only*
Attendees can meet one-on-one with leaders, industry members, researchers, and healthcare professionals to gain insights on topics like building capacity, advancing research, supporting communities, and avoiding costly detours. These 15-minute sessions are reserved in advance through the Attendee Hub event app.

11:15 AM – 11:30 AM
Session Transition / Break

City Beautiful Foyers

11:30 AM – 12:30 PM
Track Sessions and Expert Office Hours

11:30 AM – EMPOWERING THE RARE INDIVIDUAL

Sponsored by ALEXION AstraZeneca Rare Disease

City Beautiful A

Your Child's Rights: IEPs and 504s
The battle to get the best Individualized Education Program agreed to and adhered to is a top complaint for rare parents. Our panel will help you understand your child's rights and make sure your child isn't offered "less than."

- **Moderator:** Shannon Bennett, Patient Navigator, Global Genes
- Elle Cole, Advocate, Sickle Cell Disease
- Piper Paul, Attorney, Piper Paul Law

11:30 AM – COMMUNITY AND CAPACITY BUILDING

Sponsored by Chiesi global rare diseases

City Beautiful D/E

Master Rare Disease Conference Planning
Our panel of experts will help you develop impactful family conferences and scientific meetings, including in-person and virtual events. Find out how to identify key funding sources, create inclusive and accessible events, and maximize outreach with effective marketing. Based on a new toolkit developed by Simons Searchlight and Global Genes.

- **Moderator:** Erica Jones, Outreach Manager, Simons Searchlight
- Jessica Fein, MitoAction Advocate and Author, *Breath Taking: A Memoir of Family, Dreams and Broken Genes*
- Ashley Point, President, Koolen-De Vries Syndrome Foundation
- Michele Whiteside, Director, Programs and Events, Global Genes

11:30 AM – GROWING YOUR ORGANIZATION'S RESEARCH READINESS

Sponsored by ucb Inspired by patients. Driven by science.

City Beautiful B/C

Looking for the Common in Rare
Hear case studies from industry and advocacy. Our panelists will be discussing how each collaboration evolved, what the outcomes were, and factors that led to success.

- **Moderator**: Danny Levine, Life Sciences Writer, Global Genes
- Simon Frost, CEO and Co-founder, Tiber Capital Group
- Karmen Trzupek, Senior Director, Scientific Programs, Global Genes
- Joni Rutter, Ph.D., Director, National Center for Advancing Translational Sciences (NCATS), National Institutes of Health (NIH)

11:30 AM – EXPERT OFFICE HOURS

Sponsored by Spark THERAPEUTICS

Muse

Expert Office Hours - *By Appointment Only*
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12:30 PM – 1:50 PM
Lunch and Poster Presentations

Sponsored by Pharming

Neptune Ballroom

2:00 PM – 3:00 PM
Track Sessions and Expert Office Hours

2:00 PM – EMPOWERING THE RARE INDIVIDUAL

City Beautiful A

Sponsored by

ALEXION
AstraZeneca Rare Disease

Career Considerations for Rare Adults
Overcome common challenges faced by rare adults in the workforce such as dealing with prejudice, asking for medical accommodations, and when/how to disclose a health condition.

- **Moderator:** Mackenzie Abramson, MPH, Senior Manager, Research Programs Communications, Global Genes
- Dakota Fisher Vance, Global Patient Advocacy Associate Director, BioCryst Pharmaceuticals
- Kendra Gottsleben, Marketing Communication Specialist, Center for Disabilities, Sanford School of Medicine, University of South Dakota
- Lori Renna Linton, Rare Disease Advocate and Filmmaker

2:00 PM – COMMUNITY AND CAPACITY BUILDING

City Beautiful D/E

Sponsored by

Chiesi
global rare diseases

Working Together for Advocacy, Research and Therapies
Hear about a collaboration between GeneDx, Akouos and Hands & Voices that is breaking down silos for patients and families.

- **Moderator:** Gay Grossman, Patient Advocacy and Engagement Lead, GeneDx
- Alan Gilstrap, Senior Director, Advocacy and Engagement, Akouos
- Amanda Singleton, Clinical Lead, Biopharma Partnerships, GeneDx
- Janet DesGeorges, Executive Director, Hands & Voices

2:00 PM – GROWING YOUR ORGANIZATION'S RESEARCH READINESS

City Beautiful B/C

Sponsored by

ucb Inspired by patients. Driven by science.

Using AI to Improve Diagnosis
Learn how researchers are using AI and real world data to detect rare diseases earlier, clarify diagnoses and find solutions for the undiagnosed.

- **Moderator:** Danny Levine, Life Sciences Writer, Global Genes
- Erik A. Feingold, CEO and Co-founder, FDNA
- Melissa Haendel, Ph.D., Director of Precision Health and Translational Informatics, University of North Carolina, Chapel Hill
- Kat Schmolly, M.D., Founder, zebraMD

2:00 PM – EXPERT OFFICE HOURS

Muse

Sponsored by

ultragenyx
pharmaceutical

Expert Office Hours - *By Appointment Only*
Attendees can meet one-on-one with leaders, industry members, researchers, and healthcare professionals to gain insights on topics like building capacity, advancing research, supporting communities, and avoiding costly detours. These 15-minute sessions are reserved in advance through the Attendee Hub event app.

3:15 PM – 3:30 PM
Session Transition / Break

City Beautiful Foyers

3:15 PM - 4:15 PM

Track Sessions and Expert Office Hours

3:15 PM – EMPOWERING THE RARE INDIVIDUAL

Sponsored by ALEXION AstraZeneca Rare Disease

City Beautiful A

RARElу Told Stories Workshop

This hands-on workshop presented by Global Genes and The Disorder Channel covers the basics of filmmaking, editing, principles of storytelling, and what's unique to rare disease films.

- Daniel DeFabio, Director, Community Engagement, Global Genes
- Claudia Parker, Rare Mom, Entrepreneur, Filmmaker

3:15 PM – COMMUNITY AND CAPACITY BUILDING

Sponsored by Chiesi global rare diseases

City Beautiful D/E

Innovative Funding Strategies

We've gathered a stand-out panel of leaders to share their novel models of funding – and brainstorm revolutionary new approaches.

- **Moderator**: Megan Golden, Co-Founder and CEO, Mission: Cure
- Sunitha Malepati, Vice President, CACNA1A Foundation and Founder, Buffalo Initiative
- Bina Maniar Shah, Founder, CEO and President of the Board of Directors, Project 8p Foundation

3:15 PM – GROWING YOUR ORGANIZATION'S RESEARCH READINESS

Sponsored by ucb Inspired by patients. Driven by science.

City Beautiful B/C

Is Our Community Ready for Gene Therapy?

Find out how advocacy groups, researchers and industry use the GENE TARGET framework to determine readiness for gene therapy, identify gaps in the translational research pipeline and prioritize funding and research efforts.

- **Moderator**: Shruti Mitkus, Ph.D., Director, Patient Services, Global Genes
- Raymond Belanger-Deloge, Translational Genomic Medicine Specialist, Rosamund Stone Zander Translational Neuroscience Center, Boston Children's Hospital
- Matt Fuller, Ph.D., Executive Director, Gene Therapy Research, Ultragenyx
- Ashley Point, President, Koolen De-Vries Syndrome Foundation

3:15 PM – EXPERT OFFICE HOURS

Sponsored by VERTEX

Muse

Expert Office Hours - *By Appointment Only*

Attendees can meet one-on-one with leaders, industry members, researchers, and healthcare professionals to gain insights on topics like building capacity, advancing research, supporting communities, and avoiding costly detours. These 15-minute sessions are reserved in advance through the Attendee Hub event app.

4:15 PM – 4:30 PM

Session Transition / Break

City Beautiful Foyers

4:30 PM – 5:30 PM

Creating a Global Ecosystem for Rare Disease Knowledge

How are advancements in data science making this a hopeful time for the rare disease community? And how can we bring together the macro and micro to accomplish even more?

- **Moderator:** Charlene Son Rigby, CEO, Global Genes
- John Wilbanks, Senior Fellow, Datasphere Initiative
- Peter Goodhand, CEO, Global Alliance for Genomics and Health

City Beautiful D/E

5:30 PM

RARElу Told Stories film screening: *"Pass Me the Potatoes, Please"*

by Lori Renna Linton, IB English teacher, Patient Advocate for HSP

City Beautiful D/E

RARE Champions of Hope Celebration

#WEEKINRARE #COH

5:30 PM - 6:30 PM

RARE Champions of Hope Awards Ceremony

Open to all attendees

Sponsored by AMGEN Rare Disease

City Beautiful D/E

The RARE Champions of Hope Awards celebrate, honor and recognize those in the rare disease community who are raising the bar through their innovative approaches to research, programming and advocacy to create meaningful impact in the rare disease space. Awardees are beacons of hope in the rare disease community who lean in, take risks and lead collaborative efforts to positively impact the lives of rare disease patients, caregivers and families across the globe.

Awards will be presented in the following categories:

- RARE Champion in Advocacy (Organization)
- RARE Champion in Advocacy (Individual)
- RARE Champion in Industry
- RARE Champion in Research
- RARE Champion in Health Equity

6:30 PM - 8:30 PM

RARE Champions of Hope Reception

Open to all attendees

Sponsored by AMGEN Rare Disease

City Beautiful Foyers

RARE Health Equity Forum – Saturday, September 28, 2024

7:00 AM - 12:00 PM
Registration

West Foyer - Ballroom Level

8:00 AM - 5:00 PM
Exhibitors

City Beautiful Foyers

8:00 AM - 9:00 AM
Breakfast

Sponsored by Takeda

City Beautiful D

9:00 AM - 9:30 AM
Keynote: Living Rare Within Rare
Dr. Wright will discuss the historical contexts, complex issues, arduous challenges, and unconscious biases in the diagnosis and treatment of people of color who are "rare within rare," while sharing how her husband's unique rare disease journey inspired and led to their mission and commitment to advocating for underrepresented populations.

- Michele Wise Wright, Ph.D., Co-Founder, National Organization of African Americans with Cystic Fibrosis

City Beautiful E

9:30 AM - 10:15 AM
Lessons from the Field: Best Practices for Engaging Community Partners
This session will explore trust as a concept of something that is earned, and will identify the changes needed from healthcare institutions to do so. Pulling on success stories, panelists will discuss how to actively empower the community as a partner.

- **Moderator:** Teneasha Washington, Ph.D., Diversity, Equity & Inclusion, Global Genes
- Claudia Gonzaga-Jauregui, Ph.D., Assistant Professor, International Laboratory for Human Genome Research (LIIGH) of the National Autonomous University of Mexico (UNAM)
- Siwaar Abouhala, Clinical Research Coordinator, The Broad Institute
- Cecile Jones, Alabama Community Health Ambassador, All In. Rare - A Global Genes Program

City Beautiful E

10:15 AM - 10:35 AM
The Monarch Initiative: Informatics and Open Data Sharing
The Monarch Initiative is an international consortium focused on developing rare disease standards and methods for leveraging biomedical knowledge to connect genotypes with phenotypes to improve genetic diagnostics. The ultimate goal is to help clinicians diagnose patients and researchers to develop the best treatments faster.

- Melissa Haendel, Ph.D., Director, Precision Health and Translational Informatics, University of North Carolina at Chapel Hill

City Beautiful E

10:35 AM - 10:45 AM - **Break**

Sponsored by HARMONY BIOSCIENCES

City Beautiful Foyers

10:45 AM - 12:30 PM
Dear Rare Disease, a Storytelling Workshop
Storytelling is a powerful form of advocacy. It can engage us to express our feelings and thoughts in ways that allow others to relate to, and understand the impact of our experience. In this session, attendees will participate in a combined writing and art workshop to capture their rare experience.

- Suzanne Edison, Mental Health Coordinator, CureJM

City Beautiful D/E

Agenda subject to change

12:30 PM - 2:30 PM
Lunch & Table Talks

Sponsored by GeneDx *Sponsored by* sanofi

City Beautiful D

2:30 PM - 2:50 PM
Data Driven Music: An Exploration of Rare Disease Harmony

Professor Taylor will discuss music driven advocacy through demonstration of his music composition based on a catalog of 230 rare diseases that was curated by Dr. Aditi Kantipuly. Twelve categories of disease correspond to the 12 Indian chakras, which provide a background harmony for each category.

- Stephen Taylor, Professor of Music, University of Illinois at Urbana-Champaign

City Beautiful E

2:50 PM - 3:25 PM
Film Screening: “*Holding Moses*”

Holding Moses chronicles the heartfelt story of a queer mother and Broadway performer who learns how to parent her profoundly disabled son. This documentary folds together several areas in the DEI space including assumptions about caregivers, shame narratives around disability, and LGBTQ parents.

- **Moderator:** Daniel DeFabio, Director, Community Engagement, Global Genes
- Randi Rader, Featured Subject, Frankly Speaking Films
- Rivkah Beth Medow, Director, Frankly Speaking Films

City Beautiful E

3:25 PM - 4:10 PM
Diversity in Rare Clinical Trials: What’s Working and What Isn’t

Historically marginalized racial groups, women and other minoritized communities are notably underrepresented in clinical trials, despite efforts and investment by advocates and health equity leaders. This panel will discuss effective strategies to address the lack of diversity in clinical trials.

- **Moderator:** Kim Moran, Ph.D, Senior Vice President and Head, U.S. Rare Diseases, UCB
- R’Kes Starling, Founder, Reveles Clinical Research
- Richie Kahn, Clinical Researcher, Co-founder and COO, Canary Advisors
- Tanvee Varma, M.D., Resident Physician, Brigham and Women’s Hospital

City Beautiful E

4:10 PM - 5:00 PM
Rural Rare Healthcare

Rural residents often encounter barriers to healthcare including workforce shortages, health insurance status, transportation issues, health literacy, and stigma in rural communities. These disparities are even greater among historically marginalized communities and rare disease patients. Panelists will discuss the work they are doing to combat these challenges.

- **Moderator:** Shobana Kubendran, Genetic Counselor, KU School of Medicine-Wichita
- Ana Cohen, Ph.D., Assistant Director, Molecular Genetics Laboratory, Children’s Mercy
- Nadia Falah, M.D., Clinical Geneticist, Johns Hopkins All Children’s Hospital
- Abdallah F. Elias, M.D., Chief Medical Officer, Shodair Children’s Hospital

City Beautiful E

5:00 PM - 5:15 PM
Closing Remarks

City Beautiful E

5:30 PM - 7:30 PM
Closing Reception

Sponsored by Genentech
A Member of the Roche Group

City Beautiful Foyers