

Christine WAGGONER

topic

**CURE GM1 COMMUNITY
CONFERENCE - CURE
GM1 OVERVIEW,
INITIATIVES, AND
RESEARCH**



International GM1

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October 2, 2026
Virtual



Christine Waggoner and her husband Douglas Dooley founded the Cure GM1 Foundation in April 2015 in honor of their daughter Iris and all those affected by GM1 gangliosidosis. Christine received the Sanofi TORCH award in 2017 for outstanding patient advocacy and she also received the WORLD Symposium Patient Advocacy Leadership Award in 2023. Founding and running Cure GM1 is a true labor of love to help bring a treatment to all those affected by GM1 gangliosidosis.

Rafael ESCANDON

moderator

**BLOCK 1: GM1 FAMILY
STORIES AND MORE**



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Rafael “Rafa” Escandon has worked in global clinical research and development and patient advocacy in the biotechnology industry for three decades and is currently a consultant to the biotechnology industry. His approach is to be highly considerate of the ethical implications of conducting and participating in clinical research, especially with advanced therapies in rare conditions, as well as in vulnerable and developing-world populations. He has been a member of the NYU Grossman School of Medicine’s Department of Bioethics Pediatric Gene Therapy Medical Ethics Working Group (PGTME) since 2019, and is a contributing graduate faculty member in Pediatric Bioethics at the Children’s Mercy Bioethics Center in Kansas City Missouri. Rafa has also served as adjunct faculty at the University of California Berkeley’s Clinical Research Management Program and as an educator in human rights and justice. He holds graduate degrees from the University of Maryland at Baltimore, Walden University of Minnesota, and the Johns Hopkins Bloomberg School of Public Health, and is a certified healthcare ethics consultant.

GM1 FAMILY SERIES



Sarah
WELLS



Amber
LEE

Lesa BRACKBILL

topic

**IT DOESN'T HAVE TO
BE THIS WAY: A
BRIGHTER BLUEPRINT
FOR ADVOCACY**



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KEYNOTE SPEAKER

Lesa Brackbill is a wife, mother, patient advocate, author, and Director of Advocacy for Patient Advocacy Strategies. After her daughter Victoria was diagnosed with Krabbe Disease in 2015, Lesa channeled her experience into reforming Pennsylvania's Newborn Screening program, helping pass Act 133 of 2020 which added Krabbe Disease to the state's screening panel. She went on to guide the passage of Act 29 of 2022 for cCMV screening and has since built coalitions across state and national platforms including the EveryLife Foundation, the Institute for Gene Therapies Patient Advocacy Advisory Council, and Life Sciences PA.

Lesa is the author of *Even So, Joy*, which chronicles her family's journey with Krabbe Disease, and *A Brighter Blueprint: The Twelve Threads of Effective Advocacy*, the framework behind her presentation at this year's conference. She holds a Bachelor of Arts in Political Science and a Master of Arts in Strategic Communication from Azusa Pacific University and lives in Hershey, PA with her husband, Brennan and their twin boys, Isaiah and Caleb.

Dawn BLESSING

moderator

**BLOCK 2: CLINICAL
TRIALS AND PRE-
CLINICAL RESEARCH**



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Dawn Blessing was the Chief Business Officer at Neucore Bio and was recently Vice President of Scientific Strategy and Corporate Development at Zogenix. Prior to Zogenix, she served as Senior Vice President Corporate Development at Modis Therapeutics and previously Vice President, Corporate Development at Audentes Therapeutics. She has 30 years of experience in biotechnology finance, business development, and alliance management. Over this period, Ms. Blessing has focused on programs for rare diseases and the application of genetic information to drug development. Before joining Audentes, Ms. Blessing served as Senior Director, Business Development and Alliance Management at 23andMe, and Director of Business Development at BioMarin Pharmaceutical.

Christian FREITAG

topic

**NIZUBAGLUSTAT
DEVELOPMENT IN
LYSOSOMAL DISEASES
– AN UPDATE**



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Dr Freitag obtained his medical degree from Kiel University, Germany. After several years in different hospital posts, he started his career in the pharmaceutical industry at Roche. After A few different roles in different clinical development, he started working fully in the rare disease area in 2018. He joined Azafaros in 2022 and has responsibility for the clinical development and medical management of the clinical programme.

Emma CANEPA

topic

**ICV ERT IN THE
REAL WORLD
ICV 101**



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Emma Canepa, MS, CCRP, is a Clinical Research Program Manager at the University of California, San Francisco, where she leads clinical research programs within the Center for Maternal-Fetal Precision Medicine and Center for Genome Surgery. Her work focuses on translating innovative therapies for rare fetal and pediatric genetic diseases from scientific discovery into early-stage clinical research.

In her time at UCSF, Emma has operationalized and overseen complex investigator-initiated studies from protocol development and regulatory review through clinical implementation and long-term follow-up. At the heart of her work is serving as a “lighthouse” for patients and families facing devastating diagnoses—helping them navigate the uncertainty and complexity of clinical research while ensuring their experiences and perspectives remain central to the research process. She brings together multidisciplinary teams across science, medicine, ethics, regulatory affairs, and research operations to support the responsible translation of promising therapies into clinical care.

Through the Center for Maternal-Fetal Precision Medicine and the newly established Center for Genome Surgery, Emma helps build the clinical and organizational frameworks needed to responsibly advance novel genetic therapies for patients with serious genetic diseases.

Caroline HASTINGS

topic

**ACETYLLEUCINE IN
THE TREATMENT OF
GM1/GM2**



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Dr. Caroline Hastings is a Pediatric Hematologist Oncologist and Neuro-Oncologist, who specializes in the care of children and adolescents with blood disease or cancer. She focuses particularly on the care of patients with tumors of the spine and brain. In addition, she cares for children and adults with lysosomal storage disorders, metabolic diseases that result from defective proteins or enzymes that interfere with metabolic pathways.

Dr. Hastings conducts research in rare diseases, in particular brain tumors, Niemann-Pick Type C disease, and GM1 and GM2 Gangliosidosis. She works on clinical trials to develop new drugs and treatment protocols and serves on advisory boards and foundations dedicated to addressing unmet needs in rare diseases.

She earned her medical degree from the University of California, Davis School of Medicine. She completed her residency in pediatrics and fellowship in pediatric hematology oncology at Children's Hospital Oakland. Dr. Hastings believes that training young people who are passionate about medicine is critical to ensuring a healthy future for her patients, and finds that preparing the next generation of doctors in this specialty is one of the most rewarding aspects of her career in medicine.

Maria Ester BERNARDO

topic

**HEMATOPOIETIC STEM
CELL GENE THERAPY
FOR LYSOSOMAL
STORAGE DISORDERS**



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Dr Bernardo is Responsible for the Pediatric Transplant Unit and Pediatric Allogeneic Transplant Coordinator of the Pediatric Immunohematology Unit, San Raffaele Scientific Institute. At SR-TIGET, she is Group Leader and Clinical Coordinator of the Pediatric Clinical Research Unit. She is Associate Professor of Pediatrics at Vita-Salute San Raffaele University Medical School, Milan. She is PI of the Phase I/II clinical trial of ex-vivo gene therapy for the treatment of patients affected by Hurler Syndrome. She is Co-author of >100 peer-reviewed articles published in international journals; H index (Scopus) = 50.

Amber FREED

topic

**FIGHTING LIKE A
MOTHER**



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KEYNOTE SPEAKER



Amber Freed is a globally recognized rare disease advocate, strategist, and Founder CEO of SLC6A1 Connect, a leading patient-driven organization accelerating scientific research, therapeutic development, and community impact for SLC6A1-related neurodevelopmental disorders.

Her journey began in 2018 when her son Maxwell was diagnosed at 18 months old with SLC6A1, a debilitating neurodevelopmental condition marked by developmental delays, epilepsy, and cognitive and motor challenges. Faced with limited treatment options and uncertain outcomes, Amber left her career in equity research analysis to pursue a cure for her son and others like him.

After her son Maxwell was diagnosed at 18 months with SLC6A1, a rare genetic condition with profound neurological, developmental, and epileptic manifestations, Ms. Freed left her career in equity research analysis and dedicated herself to advancing treatments where none existed. Through her leadership, SLC6A1 Connect has raised critical philanthropic capital, built robust scientific and patient infrastructure, and forged strategic collaborations with academic, clinical, and industry partners to accelerate translational science and clinical readiness.

A historic milestone in her work came in 2025 when Maxwell became the first person in the world to receive an experimental gene therapy targeting SLC6A1, a testament to years of advocacy, fundraising, scientific partnerships, and clinical development.

Joshua HENDERSON

moderator

**BLOCK 3: PRECLINICAL
RESEARCH AND RESEARCH
LANDSCAPE**



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Joshua Henderson is driven by a singular mission: to accelerate the development of treatments for people living with rare diseases. As the Chief Cure Strategy Officer at Mission: Cure, he works at the intersection of clinical research, drug development strategy, and patient advocacy, building the infrastructure, partnerships, and evidence needed to move promising therapies from concept to clinic. Alongside this work, he co-founded the NW Rare Disease Coalition, bringing together patients, clinicians, researchers, and industry to strengthen the rare disease ecosystem in the Pacific Northwest and beyond.

Previously, as Head of Rare Diseases at Pulse Inframe, Joshua partnered with patient advocacy organizations and biopharma companies globally to design and execute observational research studies and build patient registries that generate high-quality, regulatory-grade real-world evidence. Throughout his career he has cultivated deep bridges between the advocacy and industry communities, translating patient experience into research priorities and clinical strategy.

Joshua is also an investor focused on rare diseases, underserved patient populations, and underrepresented founders. He is the founder of StageNext, a venture fund that invested in early-stage life science and health technology companies, and an angel investor and Fellow at On Deck Angels (ODA4). Earlier in his career, he served as Vice President at non-profit Springboard Enterprises for over 12 years, helping build its life science and health technology programs and advise nearly 400 high-growth companies.

Allisandra RHA

topic

**ADDRESSING THE
PERSONALIZED
MEDICINE LANDSCAPE**



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A trained biochemist and molecular biologist, Dr. Rha has dedicated much of her professional career to basic and translational research that aims to propel therapeutic development for lysosomal storage diseases. Her work has centered on gene therapies, specifically, gene editing modalities, for GM1 gangliosidosis and other lysosomal disorders. She currently works at Chapman University where she serves as the principal investigator of a project evaluating the utility and efficacy of adenine base editing in GM1 cerebral organoids and believes that personalized medicine is our present and our future.

Mohsen HERJATI

topic

**BUILDING A SAFE AI
AGENT FOR THE GM1
COMMUNITY**



Mohsen Hejrati is co-founder and CEO of Basion, an AI platform for personalized medicine built for patients, caregivers, and clinicians facing rare diseases and other conditions where the standard of care has run out.

He was previously a Senior Director of AI at Genentech, where he led AI for clinical data and drug development. Earlier he worked on safety-critical machine learning at Google X, before that team became Waymo. He holds a PhD in machine learning and has had three prior startup exits.

He started Basion because nothing connects the science that could help rare-disease patients to the families who need it. His focus now is making medical AI available, safe, transparent, and accountable to the people who depend on it.

Jeanine JARNES

topic

COMPASSIONATE
USE VENGLUSTAT



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Dr. Jeanine Jarnes, PharmD, MSc, is an Associate Professor in the University of Minnesota Department of Pediatrics, Medical School, a Graduate Faculty of the University of Minnesota, and an Assistant Professor in the Department of Experimental and Clinical Pharmacology at the College of Pharmacy (adjunct). She is a clinical researcher and pharmacogenomicist for the Lysosomal Disease Network, and clinical care for patients with lysosomal diseases at the Advanced Therapies Clinic, University of Minnesota. She serves as a primary mentor for PhD students and physicians for lysosomal disease post-doctoral fellowship training programs. She is Associate Course Director for *WORLDSymposium*, an annual international meeting that brings together researchers from around the world for dissemination of lysosomal disease research. Her research focus includes projects in the following areas: natural history of gangliosidosis diseases (such as Tay-Sachs disease, Sandhoff disease, and GM1-gangliosidosis), adult and pediatric cystinosis, small molecule and gene therapies, and infusion therapies for lysosomal diseases. Dr. Jarnes is Principal Investigator of The Natural History of the Gangliosidoses, a study that has been active since 2009.

Dr. Jarnes obtained a Bachelor of Science in Pharmacy in 1990 and a Doctor in Pharmacy in 2004 from the University of Minnesota, a Master of Science in Pharmacogenomics from Manchester University, North Manchester, Indiana (2022). She holds and maintains Board Certifications through the Board of Pharmaceutical Specialties in the following areas: Board-Certified Pharmacotherapy Specialist (since 2007), and Board-Certified Oncology Pharmacist (since 2005). Dr. Jarnes completed graduate level training certificate program in Pharmacogenetics and Pharmacogenomics through Stanford University (2021), Pharmacogenomics Certificate from the Mayo Clinic (2022).

Valentina POLETTI

topic

**A HYPERACTIVE HUMAN
B-GALACTOSIDASE FOR
GENE THERAPY OF GM1
GANGLIOSIDOSIS AND
RELATED DISEASES**



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Dr. Valentina Poletti is an Assistant Professor of Genetics at the Department of Women's and Children's Health, University of Padova, Italy, and Principal Investigator of the Gene Therapy for Hemoglobinopathies and Neurometabolic Diseases lab at the Pediatric Research Institute in Padua. With over 15 years of experience at the intersection of basic and applied science, she has built her career across leading institutions in Italy, France, and the United States, including Harvard Medical School, Dana-Farber/Boston Children's Hospital, and Genethon. Her research focuses on the development of ex vivo gene therapies for rare and lethal pediatric diseases, with particular emphasis on GM1-gangliosidosis.

Dr. Poletti holds a PhD in Molecular Medicine from the University Vita-Salute San Raffaele in Milan and has contributed to the preclinical development of gene therapy strategies for a broad spectrum of genetic diseases. She is the recipient of competitive grants including a Marie Skłodowska Curie Individual Fellowship and the Fondazione Telethon ETS Multiround Call 2024 Grant, and holds two international patents in gene therapy vector design. Her work has produced over 23 peer-reviewed publications and more than 45 abstracts presented at major international conferences.

David LAW

moderator

**BLOCK 4: CAREGIVER
AND COMMUNITY**



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Dr. David Law is the father of Violet Aurora and Derek Sison Law. Violet was diagnosed with late-infantile GM1 gangliosidosis and passed away at age 4. He is an internal medicine physician who trained at the UC Davis Medical Center and Harbor-UCLA Medical Center, and currently practices hospital medicine and palliative care medicine in southern California. He has been married to his wife, Dr. Veena Sison, a developmental pediatrician, since 2014 and together they enjoy traveling, attending live music events, and are the co-founders of 3rd Call Productions, an award-winning independent film studio in Los Angeles. They use the majority of their time now advocating for rare disease awareness and research.

Chrissy SALLEY

topic

**SUPPORT YOUR
PARTNERSHIP WHILE
CARING FOR A SERIOUSLY
ILL CHILD**



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Dr. Chrissy Salley is a Pediatric Psychologist and Director of Clinician Education and Engagement at Courageous Parents network. She is an expert in delivering support and intervention to parents and caregivers of children with complex medical needs. In addition to co-authoring the Standards of Psychosocial Care for Parents of Children with Cancer, she has founded a professional group of pediatric psychologists who are committed to supporting parent caregivers through clinical intervention, research, education and outreach through her involvement in the Society of Pediatric Psychology. Dr. Salley is committed to engaging caregivers in research from development through implementation and oversees all research activities at CPN.

Andre SAM

topic

**DISABILITY & SPECIAL
NEEDS PLANNING
MADE EASY**



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Andre Sam is a Comprehensive Disability/Special Needs Planner who has been providing planning services and complimentary educational workshops to families and over 120 organizations around the country covering a span of more than 70 different disabilities, disorders, and diseases for over 14 years now to better educate the disability/special needs community on important planning topics such as Legal Planning documents, Guardianship & Alternatives to Guardianship, Government Benefits such as SSI, Social Security Disability and other Social Security benefit programs, as well as Medicaid, Medicaid Waivers, Medicare, ABLE Accounts, and Lifestyle/Transition Planning.

Kaitlyn OBER

topic

**GIVING VOICE TO EVERY
STATE: AAC STRATEGIES
FOR INDIVIDUALS WITH
GM1**



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Kaitlyn "Kait" Ober, M.S., CCC-SLP, is a speech-language pathologist serving public school students in Southern California. Her clinical background includes pediatric care at Rady Children's Hospital of Orange County, with specialized expertise in Augmentative and Alternative Communication (AAC) and eye gaze technology. She regularly conducts device trials and funding assessments to help individuals secure coverage through insurance and other funding sources and programs. Kait earned her B.A. in English Language and Literature from Gordon College and her M.S. in Communication Sciences and Disorders from the University of Redlands. Driven by the belief that every individual has the right to communicate, she is passionate about breaking down financial and systemic barriers to ensure each individual gains early access to the communication modalities that best honor their voice.

Vanessa RANGEL MILLER

topic

**SCREENING FOR
PREVENTION AND
GM1 FAMILY PANEL:
IVF**



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Vanessa Rangel Miller is certified genetic counselor, with over 15 years' experience in rare disease, patient engagement and genetic programs. Vanessa is currently in Medical Affairs at Ultragenyx. Previously at Invitae, Vanessa served in Biopharma/Advocacy Business Development, bringing together multiple partners in the biopharmaceutical industry to launch genetic diagnostic programs. As a co-founder of PatientCrossroads (d.b.a. AltaVoice), Vanessa brought together technology, genetics, and external partners, and was responsible for >50 patient-centered registry programs in rare and non-rare disease. Vanessa has previously worked for Emory University, and completed her master's in genetic counseling at the University of North Carolina-Greensboro and her MBA at Emory University.