

# Christine WAGGONER

## topic

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



*August 7-8, 2025  
Irvine, CA*



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.

# David LAW

## topic

**CAREGIVING 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.



# Dawn BLESSING

## topic

**CLINICAL TRIALS,  
PRECLINICAL  
RESEARCH, AND  
NATURAL HISTORY**



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Dawn Blessing is 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.



# Kylie HARRISON

## topic

**ADVOCACY AND  
PATIENT-LED  
EFFORTS**



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Kylie lives in Colorado with her 4 daughters and husband, Kyle. In June of 2020 her two oldest daughters, Kinley and Kennedy, were diagnosed with Juvenile GM1. Since learning of her daughter's diagnosis, she and Kyle have dedicated much of their time to raising awareness and funding to advance treatments for all children with GM1. Kylie recently became a Certified Nursing Assistant and is now paid to take care of Kinley and Kennedy. The Harrison family has been fundraising to support research and development of treatment options for this horrific disease since the girls were diagnosed in 2020. To date they have raised over \$350,000 for the foundation.



# Mary MCDIRMID

## topic

**SUPPORTING YOUR  
FAMILY WITH A  
SPECIAL NEEDS  
MASTER PLAN**



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When Mary isn't helping families navigate the world of special needs financial planning, she is being a wife to Jay and a mom to Charlie and Ruth. Not to mention her leadership role as founder of All Needs Planning and a volunteer advocate for kids with rare diseases. She takes the title "Mom Boss" seriously, just ask her!



# Rafael ESCANDON

## topic

**THE ABC'S & 1-2-3'S  
OF INVESTIGATIONAL  
ACCESS**



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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



*Deepika*  
**SRIRAM**



*Megan*  
**REINDL**



*Guadalupe*  
**CARINI**



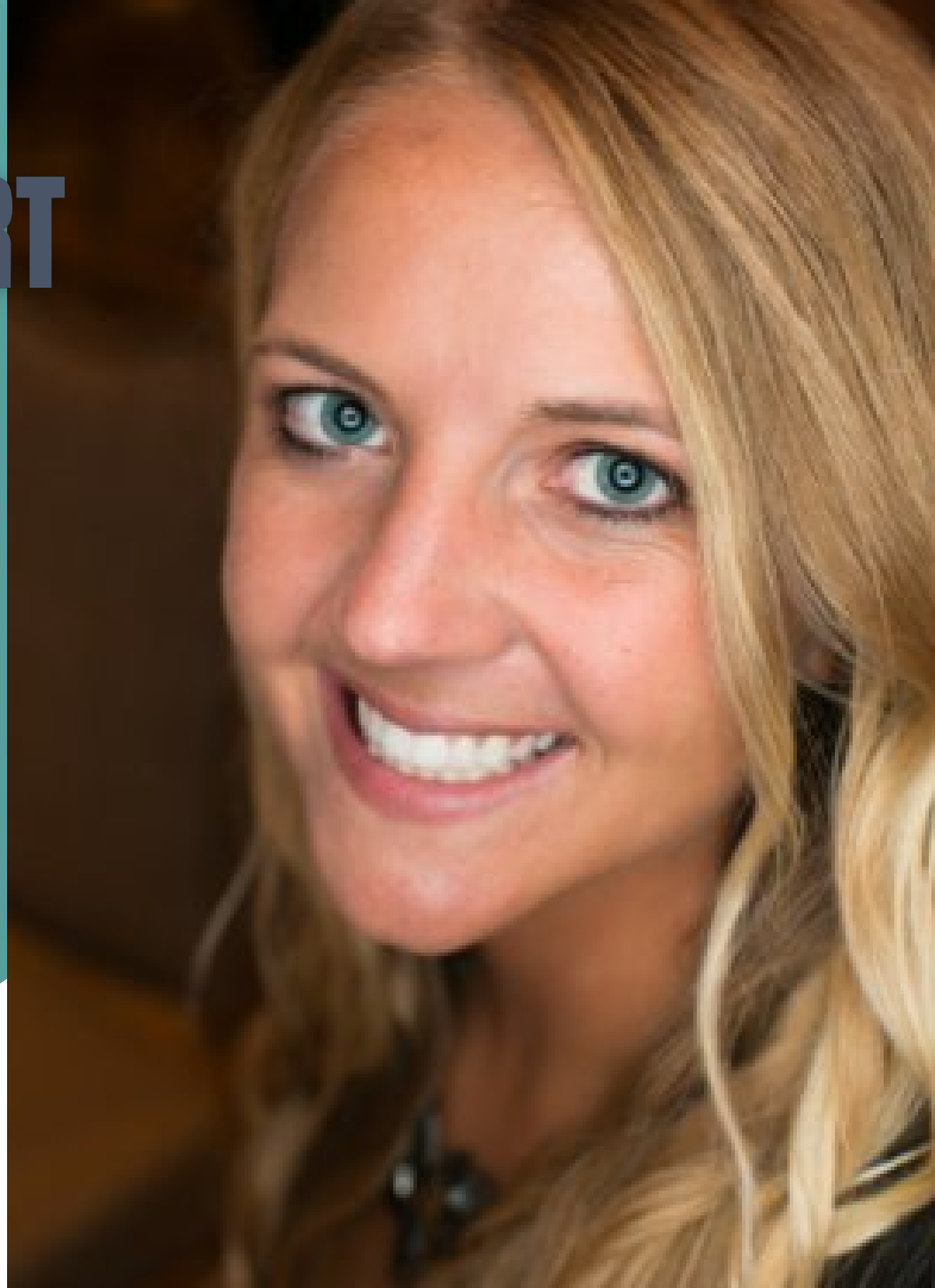
# Jillian STEWART

## topic

**WAVES, WHINNIES,  
AND WELLNESS:  
DELIVERING  
THERAPY BEYOND  
FOUR WALLS**



August 7-8, 2025  
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Dr. Jillian Stewart, PT, DPT, HPCS is a physical therapist, who graduated from Northwestern University with her Doctorate of Physical Therapy in 2014 and received her Hippotherapy Clinical Specialty in 2017. As a physical therapist she saw a need for community integration and inclusion for people with disabilities, which led her to start Surf & Turf Therapy, in 2018.

With 30 years of horsemanship experience, 15 years of surf experience, and over a decade as a physical therapist, Jillian is passionate about enhancing the relevance and reputation of the use of non-traditional therapeutic tools. She actively advocates for the profession, providing guest lectures at universities and therapy clinics, being featured in podcasts, participating in panels, and mentoring professional students. She is an active member of the American Hippotherapy Association, International Surf Therapy Organization, and San Juan Capistrano Equestrian Coalition, as well as a voting board member on the Doheny Longboard Surfing Association.



# Sylvia STOCKLER

## topic

**TOWARD A CORE  
OUTCOME SET FOR  
GM1: PATIENT AND  
CAREGIVER  
PERSPECTIVES**



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Sylvia Stockler-Ipsiroglu, MD PhD FRCPC, is a Professor of Pediatrics in the Department of Pediatrics, UBC and a distinguished clinical biochemical geneticist in the Division of Biochemical Genetics at BC Children's Hospital in Vancouver, BC, Canada. With her background in biochemical genetics and pediatric neurology, her main interest resides in finding treatments for children with neurometabolic conditions and rare diseases, including GM1 gangliosidosis and Morquio B syndrome. With a passion for improving the lives of young patients and their families, Dr. Stockler has devoted her career to advancing our understanding of rare metabolic disorders. She uses an integrated approach based on clinical, biochemical, molecular genetic, magnetic resonance spectroscopy (MRS), and imaging analyses. Her insights have shed light on the important biochemical pathways that underlie these conditions. She is the PI of several investigator initiated and industry sponsored clinical trials for rare disease treatments and is especially interested in practice and observation-informed evidence in rare diseases.

# Amanda GRIFFITH ATKINS

## topic

**HOW TO HANDLE  
MORE THAN YOU  
CAN HANDLE**



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## KEYNOTE SPEAKER

Amanda Griffith-Atkins is a licensed therapist and founder of the Amanda Atkins Counseling Group in Chicago. Amanda earned a Master's degree in Marriage and Family Therapy from Northwestern University, but it wasn't until her son Asher was born that she discovered her life's purpose: to help parents of disabled children find their stride. Amanda has become a leading expert in helping parents of disabled children navigate their emotions, relationships, and parenting. Her first book, *How to Handle More Than You Can Handle: Caring for Yourself While Raising a Disabled Child*, came out in June, 2025. She lives in Chicago with her husband and 3 sons.



# Kristen GRAY

*topic*

**CURE BATTEN STORY**



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Kristen Gray grew up in the suburban landscape of Chicago and later pursued a Bachelor of Science degree at the University of Southern California. Before dedicating herself to motherhood, she spent several years working in sales in both the radio and wine & spirits sectors. In 2010, she and her husband Gordon celebrated the arrival of their daughter Charlotte, followed by their second daughter Gwenyth in 2013. However, in 2015, their lives took a tragic turn when UCLA diagnosed their daughters with Batten Disease CLN6, a severe and terminal condition. Faced with the grim reality that their daughters' lives would be drastically altered, the Grays chose to take action. They established The Charlotte & Gwenyth Gray Foundation to fund research and seek treatments for this devastating illness. Driven by their mission, Kristen launched The Gray Academy in Santa Monica in 2018 to cater to the special needs of children with complex neurological disorders. Looking ahead, the foundation is preparing for a Phase II clinical trial at CHOC set for the fourth quarter of 2025, incorporating valuable lessons learned from previous trials. The Gray family welcomed two healthy boys, Callan in 2020 and Gavin in 2022. Tragically, Charlotte passed away in July 2024, but Kristen remains steadfast in her commitment to honor her daughter's legacy by ensuring that no other child endures the same fate.



# Caroline HASTINGS

## topic

**AZAFAROS UPDATE  
& GENOTYPIC  
HETEROGENEITY IN  
GM1**



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Dr. Caroline Hastings is a Professor of Pediatrics and is specialty boarded in Pediatric Hematology Oncology. She has been at Children's Hospital Oakland and now UCSF since completion of her training in 1992. Her longevity in the group has allowed for personal and professional growth and development of long-term relationships with her patients. She is currently focused in the NeuroOncology program as well as leading clinical trials and serving as an international consultant for rare lysosomal diseases including Neimann-Pick Type C, Gangliosidoses and Acid Sphingomyelinase deficiency. She also has several roles related to graduate medical education including serving as the Fellowship Director in Pediatric Hematology Oncology at Children's Hospital Oakland since 1996, as well as serving as the chair for the Graduate Medical Education committee in which she works with the program directors in ensuring regulatory compliance and quality training.

Dr. Hastings serves as the President of the American Society of Pediatric Hematology Oncology and in this role is very active in addressing workforce issues for faculty and trainees, advocacy, and issues related to the practice of the specialty and continuing education.



# Simon JONES

## topic

**THE THEORY OF  
APPLYING TANGANIL  
AND ACETYL-L-  
LEUCINE TO  
NEUROLOGICAL  
DISEASES**



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Dr Simon Jones is a consultant in paediatric inherited metabolic diseases at the Willink Unit at Saint Mary's Hospital. His major research interest is therapy for lysosomal storage diseases (LSD's). He received his medical training at the Edinburgh University Medical School, Edinburgh, UK, with a BSc in Neurosciences. He moved to London and trained in Paediatrics at Guy's and St. Thomas' Hospital, London, UK. He has been working at the Willink Biochemical Genetics Unit in Manchester since September 2005. Since 2008, he has been a consultant in paediatric inherited metabolic diseases at the Willink Unit and is now the clinical lead for the LSD service.

Dr. Jones has been actively involved in many phase I-IV international multicentre trials of novel therapies for LSD's. He is currently the principal investigator in a number of LSD trials, and a senior lecturer at the University of Manchester. He is an author of over 150 peer-reviewed papers and 3 book chapters.

When not working, he watches Liverpool Football club and spends time with his family.

# Billie LIANOGLOU

*topic*

**FETAL THERAPIES**



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Billie Lianoglou is a genetic counselor who specializes in prenatal diagnosis and fetal therapies for monogenic disease.

Billie splits her time supporting the research of genetic specialists Dr. Mary Norton and Dr. Teresa Sparks as well as fetal surgeon Dr. Tippi MacKenzie at the UCSF Center for Maternal-Fetal Precision Medicine. With Dr. Norton and Dr. Sparks the research explores the value of prenatal exome and genome sequencing, a technique for determining potentially useful genetic information.

Billie manages two UCSF registries, one of patients with ATM and the second of Lysosomal Diseases. Her work includes outreach and enrollment for two fetal therapy clinical trials, in utero stem cell transplantation for ATM and in utero enzyme replacement therapy for lysosomal diseases at the UCSF Fetal Treatment Center.

Lianoglou earned a master's degree in genetic counseling from Icahn School of Medicine at Mount Sinai.



# Allisandra RHA

## topic

**GM1 RESEARCH  
LANDSCAPE**



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Dr. Allisandra Rha is an innovative and driven molecular biologist with demonstrated experience in contemporary gene editing modalities and AAV gene therapies for lysosomal storage disorders. She is passionate about propelling pre-clinical research pipelines to get therapeutics to patients and innovating novel solutions to disease hurdles.

Dr. Rha is a STEM educator who strives to engage the next generation in inquiry-driven research, instilling a love for learning, and encouraging curiosity and discovery.

# Gouri YOGALINGAM

*topic*

**FOUNDATION-LED  
ICV ERT**



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Gouri Yogalingam earned her BSc in Pharmacology and Physiology from the University of Adelaide, South Australia, where she later completed her PhD focusing on the development of gene and enzyme replacement therapies for the lysosomal storage disease, mucopolysaccharidosis type VI (MPS VI). She went on to complete her postdoctoral training at St. Jude Children's Research Hospital in Memphis, Tennessee, understanding disease mechanisms and developing cell-based therapeutic interventions for the neurodegenerative lysosomal storage disease, sialidosis.

As an industry scientist, Gouri has worked on mAb, peptide, gene therapy and small molecule programs across all therapeutic areas. She has also contributed to the successful development of enzyme replacement therapies for multiple lysosomal storage diseases, including Brineura for CLN2, Naglazyme for MPS VI, and preclinical-stage ERT programs targeting rare neurodegenerative lysosomal storage diseases like GM1 gangliosidosis. Gouri serves as a patient advocate and scientific advisor for the Cure GM1 Foundation and is an Independent Consultant and Founder of Pyra Bio, Inc, a company committed to advancing effective treatments for rare neurodegenerative diseases with high unmet medical need.



# Maija STEENARI

## topic

**NEUROMODULATION  
IN MEDICALLY  
COMPLEX PATIENT**



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As a pediatric neurologist and epilepsy specialist, Dr. Steenari's clinical interests include neurometabolic diseases, genetic epilepsy syndromes and treatment of refractory epilepsy, including intracranial monitoring and epilepsy surgery. Her research interests include understanding epileptic disorders, identifying the disease process and optimizing targeted and disease modifying treatments for these medically complicated patients. She is working on characterizing pathological networks in a multi-modal fashion and studying biomarkers of the epileptogenic zone with the ultimate goal of improving the quality of life for patients with epilepsy.

Dr. Steenari is board certified in child neurology, clinical neurophysiology, and epilepsy. She attended medical school at University of Helsinki Medical Faculty in Helsinki Finland, and completed her pediatric residency at White Memorial Medical Center in Los Angeles California and child neurology training at CHOC and UC Irvine. She also completed a subspecialty fellowship in pediatric epilepsy at CHOC and UC Irvine.

# Carrie THORSON

*topic*

**SWIM THERAPY**



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Carrie Thorson graduated from the Physical Therapy program at California State University Long Beach in 1987. She has worked in acute care, acute rehab, cardiac rehab, wound care, skilled nursing facilities, home health, and outpatient clinics treating both orthopedic and neuro clients in both land and pool based settings. She began working in the pool in 1995. Carrie started her own business, Aquatic Rehab & Consulting, in 2004 with two locations in Southern California. She treats a diverse population from pediatrics to geriatrics, ortho, neuro, and chronic pain. She is an adjunct faculty member for Chapman University's Physical Therapy program, assisted with editing for the Aquatic Physical Therapy Section's certificate in clinical competency, and is the Section Program Chair for the Aquatic section of the American Physical Therapy Association where she reviews and selects research for national presentation. Her research in the treatment of Parkinson's Disease has been published in the Journal of Physical Therapy.



# Jacqueline MADDEN

## topic

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



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Jacqueline Madden, MS, RN, CPNP is a nurse practitioner at UCSF Benioff Children's Hospital in Oakland CA.

For the past 25 years, she has worked with Dr. Paul Harmatz doing clinical research looking for novel treatments for rare disorders. The research group was involved in the studies leading to FDA approval of therapies for achondroplasia, hypophosphatasia, Niemann-Pick Type C, MPS II, MPS IVA, MPS VI, and MPS VII.

Current protocols focus on different types of gene therapies as well as various approaches to crossing the blood brain barrier to treat the central nervous system.

She appreciates the commitment of the families participating in clinical trials who advance our understanding of rare disorders and make these new therapies possible. It is extremely rewarding to be a part of the process.

# Vanessa RANGEL MILLER

## topic

**HOW TO READ YOUR  
GENETICS REPORT**



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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.



# Matt HARUTUNIAN

*topic*

**ACT FOR ULTRARARE**



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Matt is an accomplished patient advocacy and public affairs executive with 18+ years of experience developing advocacy, policy, and engagement strategies across government and healthcare. He has a proven record of building award-winning teams, executing multi-stakeholder campaigns, and advancing public health, reimbursement, diagnostic, awareness, and drug development initiatives at the state, federal, and international levels.

Matt spent nearly nine years working in U.S. Congress in the House and Senate, where he focused on healthcare, appropriations, and economic development issues. During this time, Matt worked on FDA, Medicaid, and Medicare coverage legislation and regulatory policy. Since joining the healthcare industry, Matt has led patient-centric initiatives at multiple biopharma companies, where he has primarily focused on rare diseases and on collaborating with patient advocacy groups to removing regulatory barriers, improve access, diagnosis, and building strategic and impactful partnerships. He currently serves as Vice President, Patient Advocacy, at Ultragenyx, a biotech company founded to advance innovative medicines for rare and ultrarare diseases that have never been treated before.

# Cynthia TIFFT

## topic

**INTRAVENOUS AAV9  
NIH TEAM UPDATE**



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Dr. Tifft received her MD and PhD from the University of Texas Health Science Center and Graduate School of Biomedical Sciences at Houston. She completed pediatric residency training at Johns Hopkins Hospital and clinical genetics fellowship in the Inter-Institute Medical Genetics Training Program at the NIH. After 18 years at the Children's National Health System and Division Chair of Genetics & Metabolism, Dr. Tifft was recruited to the National Human Genome Research Institute to become Deputy Clinical Director and director of the pediatric NIH Undiagnosed Diseases Program. Her research interests include the natural history and pathogenesis of lysosomal storage disorders affecting the central nervous system, particularly Tay-Sachs and Sandhoff diseases, and GM1 gangliosidosis. In 2019 she and her collaborators initiated a "first-in-human" phase 1/2 trial of AAV9 intravenous gene therapy trial for patients with Type I and Type II GM1 gangliosidosis.



# Monique MOLLOY

## topic

**GEMMA  
BIOTHERAPEUTICS  
UPDATE**



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Monique Molloy is a veteran in the gene therapy industry, having worked in managerial and executive roles for more than 30 years.

Ms. Molloy manages GEMMABio's administrative, communications, community engagement, and human resources functions as well as supporting high priority projects and initiatives, such as fundraising, patient advocacy, relationship building with domestic and international stakeholders, and strategic planning.

Ms. Molloy started working in the field shortly after she graduated from the university, as Dr. Wilson initiated the University of Pennsylvania's major gene therapy research effort as a dedicated institute. With her deep expertise and dedication to rare disease research over the years, she assumed top leadership positions as the Executive Director of the Orphan Disease Center and Chief Administrative Officer of the Gene Therapy Program.