



International GM1

COMMUNITY CONFERENCE

August 7-8, 2025
Irvine, CA

August 7th: Welcome Dinner

**6:00 PM - DINNER AT EL TORITO
(MUST REGISTER IN ADVANCE FOR THE DINNER)**

August 8th: Full Event Program

**CHECK-IN AND BREAKFAST BEGINS AT 8:00 AM
CHILDCARE 8:30 AM - 5:30 PM (HOSPITALITY SUITE, ROOM #1205)**

**CHECK-IN: THEATER FOYER (MAIN FLOOR/1ST FLOOR)
SESSIONS: THEATER ROOM (MAIN FLOOR/1ST FLOOR)
LUNCH: THEATER TERRACE (MAIN FLOOR/1ST FLOOR)
ROUNDTABLES: THEATER TERRACE (MAIN FLOOR/1ST FLOOR)**

SEE FULL PROGRAM BELOW

August 8, 2025

Full Program Agenda

GM1 FAMILY STORIES, KEYNOTE, AND MORE

Time	Sessions	Speaker
8:45 - 9:00 am	Welcome and Introduction	Christine Waggoner President, Founder Cure GM1 Foundation
9:00 - 9:15 am	GM1 Family Speaker Series	GM1 Families: Deepika Sriram Megan Reindl Guadalupe Carini
9:15 - 10:00 am	Keynote: How to Handle More Than You Can Handle A guide to navigating the emotional experience of caring for and raising disabled children with high-support needs	Amanda Griffith Atkins LMFT, PMHC
10:00 - 10:10 am	Cure Batten Story	Kristin Gray (Virtual) CureBatten, Gray Academy
10:10 - 10:40 am	Supporting Your Family with a Special Needs Master Plan	Mary McDirmid All Needs Planning
10:40 - 10:55 am	Q&A	
10:55 - 11:05 am	Break	

CLINICAL TRIALS AND PRECLINICAL RESEARCH

Time	Sessions Continued	Speaker
11:05 - 11:10 am	Session Moderation	Dawn Blessing Cure GM1
11:10 - 11:20 am	Azafaros Clinical Trial: NAVIGATE Study	Caroline Hastings UCSF
11:20 - 11:30 am	Fetal Gene Therapy and ERT	Billie Lianoglou UCSF
11:30 - 11:35 am	GEMMA Biotherapeutics Update	Monique Molloy (Virtual) GEMMA Biotherapeutics
11:35 - 11:45 am	The Theory of Applying Tanganil and Acetyl-L-Leucine to Neurological Diseases	Simon Jones (Virtual) Manchester University
11:45 - 11:55 am	Intravenous AAV9 NIH Team Update	Cynthia Tifft (Virtual live) NHGRI
11:55 am - 12:05 pm	Q & A	
12:05 - 12:15 pm	Group Photo	
12:15 - 1:10 pm	LUNCH BREAK	
1:00 - 2:00 pm	Pet Partners Therapy Dogs	

PRECLINICAL RESEARCH, DATA, AND OUTCOMES

Time	Sessions Continued	Speaker
1:10 - 1:15 pm	Session Moderation	Dawn Blessing Cure GM1
1:15 - 1:30 pm	GM1 Research Landscape	Allisandra Rha GM1 Researcher
1:30 - 1:40 pm	Genotypic Heterogeneity in GM1 UCSF Data Presentation	Caroline Hastings UCSF
1:40 - 1:55 pm	Toward a Core Outcome Set for GM1: Patient and Caregiver Perspectives	Sylvia Stockler University of British Columbia
1:55 - 2:05 pm	ICV ERT in the Real World ICV 101	Jacqueline Madden UCSF
2:05 - 2:20 pm	Foundation-Led ICV Enzyme Replacement Therapy	Gouri Yogalingham Cure GM1
2:20 - 2:30 pm	Q&A	
2:30 - 2:40 pm	Break	

CAREGIVING AND COMMUNITY

Time	Sessions Continued	Speaker
2:40 - 2:45 pm	Session Moderation	David Law Cure GM1
2:45 - 2:55 pm	Caring for a Child with Rare Disease and Caring for Your Community	David Law Cure GM1
2:55 - 3:10 pm	The ABC's & 1-2-3's of Investigational Access	Rafael Escandon Cure GM1
3:10 - 3:25 pm	Surf and Turf Therapy: Surfing and Riding Therapy	Jillian Stewart Surf and Turf Therapy
3:25 - 3:35 pm	Aquatic Physical Therapy for GM1	Carrie Thorson Aquatic Rehab and Consulting
3:35 - 3:45 pm	How to Read Your Genetics Report	Vanessa Rangel Miller Cure GM1
3:45 - 3:55 pm	Neuromodulation in Medically Complex Patient	Maija Steenari, MD (Virtual) CHOC
3:55 - 4:05 pm	Act for Ultrarare	Matt Harutinian (Virtual live) Ultragenyx
4:05 - 4:20 pm	Q&A	
4:20 - 4:30 pm	Break	

ADVOCACY AND PATIENT-LED EFFORTS, ROUNDTABLES

Time	Sessions Continued	Speaker
3:00 - 5:30 pm	Cure GM1 Video Interviews (Invitation Only)	
4:30 - 4:35 pm	Cure GM1 Overview, Initiatives, and Research	Christine Waggoner Cure GM1
4:35 - 4:45 pm	Fundraising for ICV ERT and for Cure GM1	Christine Waggoner Kyle Harrison Cure GM1
4:45 - 4:55 pm	Q & A	Kyle Harrison Moderator
4:55 - 5:00 pm	Thank you and Wrap-up Virtual Session Ends	Christine Waggoner Cure GM1
5:00 - 5:30 pm	Interactive In-Person Roundtables: Caring for the Caregiver All Needs Planning Fundraising Care tips Reading Your Genetics Report	
5:00 - 5:30 pm	Sign of Hope Activity and Professional Photography	Christine Retzer Photography