

AGENDA

2023 INTERNATIONAL GM1 HYBRID COMMUNITY CONFERENCE
HOSTED BY CURE GM1 FOUNDATION

FRIDAY, SEPTEMBER 22, 2023

9:00 AM - 5:00 PM

*Breakfast is included for all people staying at Hyatt House.

Lunch and dinner are included for all conference attendees.

**Times are listed in Pacific Time. Agenda is subject to change

PART ONE – INTRODUCTION & KEYNOTE

Moderated by Christine Waggoner

Time	Topic	Speaker
9:00 am - 9:10 am	Welcome and Introduction	Christine Waggoner President, Founder Cure GM1 Foundation
9:10 - 9:40am	Keynote: Is it Time for the Ultra-Orphan Drug Act?	Emil Kakkis, MD, PhD Ultragenyx
9:40 - 9:55 am	Gene Therapy: Unprecedented Opportunities and Unprecedented Challenges	Jim Wilson, MD, PhD [virtual] UPenn Orphan Disease Center
9:55am - 10:15 am	Sharing Insights from First Approved Treatment for Friedreich Ataxia: FARA's Approach to Advocacy and Engagement in Research	Jen Farmer, MS [virtual] CEO, FARA
10:15 - 10:30 am	Q & A	
10:30 - 11:00 am	BREAK	Auction items available to bid on!



PART TWO – RESEARCH, NATURAL HISTORY STUDIES AND CLINICAL TRIALS

Moderated by Dawn Blessing

Time	Topic	Speaker
11:00 am - 11:10 am	Improving Beta-galactosidase for GM1 Gangliosidosis Treatment	Casey Hallows, PhD Codexis
11:10 am - 11:20 am	Imagine-1 Study: Updated Interim Safety, Biomarker, and Efficacy Data from Phase 1/2 Open Label PBGM01 Trial	Samiah Al-Zaidy, MD VP Clinical, Clinical Lead GM1 Passage Bio
11:20 am - 11:35 am	LYS-GM01 Clinical Trial Update	Raymond Wang, MD CHOC
11:35 am - 11:45 am	Azafaros Clinical Trial and PRONTO Study	Cecile Paquet-Luzy [virtual] Azafaros
11:45 am - 11:55 am	Intravenous AAV9 Gene Therapy for GM1 Gangliosidosis: Updates and Next Steps	Cynthia Tifft, MD, PhD [virtual] NHGRI, NIH
11:55 am - 12:05 pm	Sanofi: AMETHIST Study and Venglustat	Isabela Batsu, MD [virtual] Sanofi
12:05 pm - 12:20 pm	Q & A	
12:20 pm - 12:30 pm	Therapy Dogs	Brad Wilson Canine Companions
12:30 pm - 1:30 pm	LUNCH BREAK and Play with Therapy Dogs/Pixar artist drawings	Valley Humane Society Auction items available to bid on!



PART THREE – DISEASE MANAGEMENT

Moderated by David Law

Time	Topic	Speaker
1:30 pm - 1:50 pm	Preventing and Managing Medical Trauma in GM1 Families	Melissa Hogan, JD, MPH [virtual]
1:50 pm - 2:10 pm	Neurologist's View of Helping Kids with GM1: Hypotonia, Dystonia, and Spasticity, Seizures Q & A	Alex Fay, MD UCSF
2:10 pm - 2:35 pm	Mastering Your GM1 Community Story Series, GM1 Family Stories	Rebecca Stockley, Communication and Executive Coach GM1 Family Speakers: Shilpa Banerjee [recording], Kylie Harrison, Jenny Bragg [virtual]
2:35 pm - 2:45 pm	Q & A	
2:45 pm - 3:00 pm	Break	Auction items available to bid on!

PART FOUR – ADVOCACY AND CAREGIVER SUPPORT

Moderated by Kylie Harrison

Time	Topic	Speaker
3:00 pm - 3:15 pm	The Importance of Sharing Your Story and GM1 Community Advocacy	GM1 Parents: Kylie and Kyle Harrison Ronnie Caruana [recording] David Law, MD
3:15 pm - 3:25 pm	From Parent to Parent Advocate: How You Can Advocate for Newborn Screening in Your State	Lesa Brackbill [recorded] Leukodystrophy Newborn Screening Action Network



Time	Topic	Speaker
3:25 pm - 3:35 pm	Give an Hour Overview	Mindy Pace Give an Hour
3:35 pm - 3:45 pm	Driving Progress Together and Cure GM1's Work	Christine Waggoner Cure GM1 Foundation
3:45 - 3:50 pm PT	Q & A	
3:50 pm - 4:00 pm	Group photo for in-person attendees/ Zoom group photo for virtual attendees	All
4:00 pm - 5:00 pm	GM1 Family Time In-Person Private Session GM1 Family Peer Support Virtual Session	Cindy Field, MSW George Mark Children's House Mindy Pace Give an Hour
4:30 pm - 5:30 pm	Auction Winner Pick Up Non-GM1 families 4:30 - 5 / GM1 families 5 - 5:30	

PART FIVE – OPTIONAL ACTIVITIES & DINNER

Time	Topic	Speaker
4:00 pm - 6:00 pm	Video Documentary Interviews	Scheduled Participants
4:00 pm - 5:45 pm	Family Photo Shoots Professional Headshot	Christine Retzer Photography
6:00 pm - 8:00 pm	Dinner at Chevy's Fresh Mex 1890 Powell St. Emeryville, CA	Registered Participants - All attendees are invited.



SATURDAY SEPTEMBER 23ND, 2023 OPTIONAL SOCIAL ACTIVITIES

Time	Topic	Speaker
10:45 am - 12:00 pm	Pixar Animation Studios Tour	Limited number of spots First come, first serve to reserve
1:07 pm - 4:00 pm	Free tickets to Oakland A's v. Detroit Tigers baseball game available on request	Limited number of spots First come, first serve to reserve

