



International GM1 Virtual
**COMMUNITY
CONFERENCE**

Friday September 20, 2024

Agenda

GM1 FAMILY SERIES, ADVOCACY AND DRUG DEVELOPMENT PROCESS

Moderated by Christine Waggoner

Time	Topic	Speaker
9:00 - 9:10 am	Welcome and Introduction	Christine Waggoner President, Founder Cure GM1 Foundation
9:10 - 9:30 am	GM1 Family Speaker Series	GM1 Families
9:30 - 9:50 am	Impossible to Possible: Bringing Light to Possibility	Sharon King Aldevron
9:50 - 10:10 am	Academia to Clinical Trials	Cara Weismann, PhD UPenn Orphan Disease Center
10:10 - 10:30 am	The Path to Approval: What I Learned Getting the World's Most Expensive Drug Approved	Maria Kefalas Cure MLD
10:30 - 10:45 am	Q & A	
10:45 - 11:00 am	BREAK	

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

CLINICAL TRIALS, PRECLINICAL RESEARCH AND NATURAL HISTORY

Moderated by Dawn Blessing

Time	Topic	Speaker
11:00 - 11:05 am	Session Moderation	Dawn Blessing Cure GM1 Foundation
11:05 - 11:20 am	GEMMA Biotherapeutics: GM1 AAV Gene Therapy	James M. Wilson GEMMA Biotherapeutics
11:20 - 11:35 am	Azafaros Clinical Program: Update on PRONTO and RAINBOW Study Data and Planning for Phase 3 Pivotal Study	Christian Freitag Azafaros
11:35 - 11:50 am	Gene Therapy: From the Basics to its Application in GM1	Rafael Escandon Cure GM1 Foundation
11:50 - 12:05 pm	Intravenous Delivery of AAV9-GLB1 Gene Therapy for GM1 Gangliosidosis and the Safety Outcomes	Precilla D'Souza NHGRI, NIH
12:05 - 12:20 pm	Q & A	
12:20 - 1:00 pm	LUNCH BREAK	
1:00 - 1:05 am	Session Moderation	Dawn Blessing Cure GM1 Foundation
1:05 - 1:20 pm	Burden of Caregiving in GM1 Gangliosidosis	Daisy Ng-Mak Sanofi
1:20 - 1:35 pm	Treatment of GM1 Gangliosidosis by AAV Systemic Expression of Lectin-Enhanced Enzyme	Walter Acosta BioStrategies
1:35 - 1:50 pm	GM1 gangliosidosis type II: Results of a 10-year Prospective Natural History Study	Jean Johnston NHGRI, NIH
1:50 - 2:05 pm	Q & A	
2:05 - 2:15 pm	BREAK	

CAREGIVING AND COMMUNITY

Moderated by David Law

Time	Topic	Speaker
2:15 - 2:20 pm	Introduction	David Law Cure GM1 Foundation
2:20 - 2:40 pm	Courageous Parents Network: What do I say now? How to Answer Difficult Questions?	Jennifer Siedman Courageous Parents Network
2:40 - 3:00 pm	Respiratory Care for GM1 Gangliosidosis	Kensho Iwanaga, MD UCSF
3:00 - 3:20 pm	The Ins and Outs: Managing GI Issues in GM1	Sonal Patel, MD Annique Hogan, MD CHOP
3:20 - 3:35 pm	Supporting Your Family with a Special Needs Master Plan	Mary McDirmid All Needs Planning
3:35 - 3:50 pm	Q & A	
3:50 - 4:15 pm	BREAK	

ADVOCACY AND PATIENT-LED EFFORTS

Moderated by Kylie Harrison

Time	Topic	Speaker
4:15 - 4:20 pm	Cure GM1 Overview, Initiatives, and Research	Christine Waggoner Cure GM1 Foundation
4:20 - 4:35 pm	Cure GM1 Data Sharing Initiative	Kathleen Kirby Cure GM1 Foundation
4:35 - 4:50 pm	xCures Platform for Patient-Centric Decentralized Research in Rare Disease	Timothy Stuhlmiller xCures
4:50 - 5:05 pm	Ultra Rare: Pathway to Treatments	Neena Nizar, EDD The Jansen's Foundation
5:05 - 5:20 pm	Q & A	
5:20 - 5:30 pm	Thank you and Wrap-up	Christine Waggoner