



AGENDA

INTERNATIONAL GM1 VIRTUAL COMMUNITY CONFERENCE
HOSTED BY CURE GM1 FOUNDATION
OCTOBER 12TH, 14TH 2022

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

DAY ONE – CURE GM1 VIRTUAL CONFERENCE

Wednesday October 12th, 2022

PART ONE – INTRODUCTION & KEYNOTE

Moderated by Christine Waggoner

Time	Topic	Speaker
9-9:10 am	Welcome and Introduction	Christine Waggoner President, Founder Cure GM1 Foundation
9:10-9:40 am	Keynote - GM1 Gangliosidosis: The Therapeutic Journey Over the Next Five Years	Phil Reilly, JD, MD BridgeBio, Third Rock Ventures
9:40-9:55am	Lessons from SMA Clinical Trials: Where We Are and Where Are We Going	Jackie Glascock, PhD VP Research Cure SMA
9:55am-10:10am	GM1 gangliosidosis: A caregiver study of symptom impact and most important symptoms to treatment	Holly Peay, RTI International
10:10-10:25 am	Q & A	
10:25 - 10:50am	BREAK	



PART TWO – RESEARCH, NATURAL HISTORY STUDIES & CLINICAL TRIALS

Moderated by Dawn Blessing

Time	Topic	Speaker
10:50am-11:00 am	CRISPR/Cas Genome Editing as a Therapeutic for GM1 Gangliosidosis	Allisandra K. Rha, PhD Children’s Hospital of Orange County
11:00am-11:10am	Employing the PS System to Correct Beta-Galactosidase Expression in GM1 gangliosidosis	Michael Pryzbilla, PhD University of Minnesota
11:10 am -11:20am	Your Children and Our Quest for a Cure: UPenn GTP GM1 Natural History Study	Michelle Miller UPenn Gene Therapy Program
11:20 am -11:30 am	Azafaros Clinical Trial and PRONTO Study	Ruben Giorgino, MD Azafaros
11:30 am -11:40 am	Gene Therapy Update – Lysogene	Marie Trad, MD Chief Clinical Officer Lysogene
11:40 am - 11:50 pm	Gene Therapy Update – Passage Bio	David Weinstein, MD, MMSc SVP Clinical Development Passage Bio
11:50 am -12:00 pm	TEGA Therapeutics Enzyme Replacement Therapy	Jillian Brown, PhD TEGA Therapeutics
12:00 - 12:10 pm	Q & A	
12:10 pm - 1 pm	LUNCH BREAK	



PART THREE – FAMILY EXPERIENCES, ADVOCACY, & PALLIATIVE CARE

Moderated by Kylie Harrison

Time	Topic	Speaker
1:00 - 1:30 pm	The Rare Sibling Experience and Family Connections	-Emily Holl, MFA, MSW Director, Sibling Support Project -David Rintell, EdD Head of Patient Advocacy BridgeBio -Katrina E GM1 Sibling
1:30 - 1:50 pm	GM1 Family Clinical Trial Experiences and What to Expect	-Moderator: Dawn Blessing -Caroline Hastings, MD GM1 -Family Panel: Maria B, Kylie Harrison, David Law, Martha L
1:50-2:00 pm	Power to the Patients - Making a Difference to Drive Progress	Vanessa Rangel Miller Cure GM1 Foundation
2:00pm-2:10pm	A Cure For Violet - Family Story, Fundraising, and Sharing Your Story	David Law, MD Cure GM1 Foundation, GM1 Dad
2:10pm-2:25pm	“Please Tell Us About Your Child” : How a Palliative Care Team Can Walk With You on Journey With Serious Illness	Tom McNalley, MD UCSF
2:25pm-2:35pm	Q & A	
2:35pm-2:40pm	Closing Remarks, About the GM1 EL-PFDD on October 14th	Christine Waggoner Founder & President Cure GM1 Foundation



PART FOUR – FAMILIES ONLY BREAKOUT

Moderated by Dr. Al Freedman

Time	Topic	Speaker
2:40-2:50pm	Every Day is a Gift	Al Freedman, PhD
2:50-3:30pm	Family Connections	GM1 Families Only with Dr. Al



DAY TWO – GM1 EL-PFDD WITH FDA

Friday October 14th, 2022

GM1 GANGLIOSIDOSIS EXTERNALLY-LED PATIENT-FOCUSED DRUG DEVELOPMENT MEETING

Time	Topic	Speaker
7:00-7:05am	Welcome from Cure GM1 Foundation	Christine Waggoner Founder & President Cure GM1 Foundation
7:05-7:10am	Invited FDA Speaker	Wilson Bryan, MD CBER, FDA
7:10-7:25am	GM1 Gangliosidosis Disease Clinical Overview	Cynthia Tiff, MD, PhD National Human Genome Research Institute
7:25-7:35am	Introduction and Meeting Overview	James Valentine, JD Hyman, Phelps, McNamara
7:35-7:40am	Audience Demographic Polling	
7:40-9:30am	Topic 1: Living with GM1 Gangliosidosis Panel Discussion Audience and Remote Polling Moderated Audience Discussion	Abby T - Bence S Megan W - Kylie H Doug D - Brittany B Jenny B - Lindy S Cindy E - Stephanie H
8:30-10am	Lunch Break	
10:00-10:10am	GM1 Gangliosidosis Research and Treatment Landscape	Jeanine Jarnes, PharmD University of Minnesota
10:10am- 11:45am	Topic 2: Current and Future Treatments for GM1 Panel Discussion Audience and Remote Polling Moderated Audience Discussion	Ronnie C - Niclas F Rojan V - Tom M Ryan B - Jessica C David L - Marilee L Maria B - Martha L
11:45am- 11:55am	Summary of the Day	Larry Bauer, RN, MA Hyman, Phelps & McNamara
11:45am- 12:00pm	Closing Remarks from Cure GM1 Foundation	Christine Waggoner President & Founder Cure GM1 Foundation