



GM1 EL-PFDD WITH FDA

Friday October 14th, 2022

GM1 GANGLIOSIDOSIS EXTERNALLY-LED PATIENT-FOCUSED DRUG DEVELOPMENT MEETING

PT	EST	Topic	Speaker
7:00-7:05 am	10:00-10:05 am	Welcome from Cure GM1 Foundation	Christine Waggoner Founder & President Cure GM1 Foundation
7:05-7:10 am	10:05-10:10 am	Invited FDA Speaker	Wilson Bryan, MD CBER, FDA
7:10-7:25 am	10:10-10:25 am	GM1 Gangliosidosis Disease Clinical Overview	Cynthia Tifft, MD, PhD National Human Genome Research Institute
7:25-7:35 am	10:25-10:35 am	Introduction and Meeting Overview	James Valentine, JD Hyman, Phelps, McNamara
7:35-7:40 am	10:35-10:40 am	Audience Demographic Polling	
7:40-9:30 am	10:40am-12:30 pm	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
8:30-10 am	11:30 am-1pm	Lunch Break	
10:00-10:10 am	1:00-1:10 pm	GM1 Gangliosidosis Research and Treatment Landscape	Jeanine Jarnes, PharmD University of Minnesota
10:10 am-11:45am	1:10-2:45 pm	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
11:45-11:55 am	2:45-2:55 pm	Summary of the Day	Larry Bauer, RN, MA Hyman, Phelps & McNamara
11:45am-12:00pm	2:45-3:00 pm	Closing Remarks from Cure GM1 Foundation	Christine Waggoner President & Founder Cure GM1 Foundation