



AGENDA

for the

Third aCDase Deficiency Global Forum For Community Alignment

facilitated by

SMA-PME RESEARCH, a 501(c)(3) Charity

April 28, 2026

9 AM – 1 PM United States Eastern Time

Please Convert All Times to Your Date and Time Zone

Zoom Link:

Zoom Meeting ID: 892 6761 7061

Password: 121212

Link: <https://us02web.zoom.us/j/89267617061?pwd=bEdoMmVBMXNibVNKaE1HZzA1N1kyQT09>

Pre-Forum ONE HALF HOUR PRIOR TO FORUM:

9 AM US ET: Meet and Greet the Participants (Optional)

9:15 AM US ET: Sound Checks

Ground Rules Question and Answer

Setup of Real-Time Translation and Training

(Non-English-speaking participants please attend)

(continued on next page)

FORUM

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Item	Subject	By	Minutes	East Coast US
1	Overview and Objectives, SMA-PME Research and The Fight Farber Foundation (confirmed)	Ms. Mary Irwin Dr. Reem Eissa	10 Presentation 5 Q&A	9:30 AM
2	A Clinician's View of SMA-PME: Adeline as a Case Study (confirmed)	Dr. Nicole Chemaly	20 Presentation 10 Q&A	9:45 AM
3	Intracisterna Magna Administration for AAV Gene Therapy (confirming)	TBD	20 Presentation 10 Q&A	10:15 AM
4	AAV.CPP.16-based ASAH1 Gene Supplementation (confirmed)	Dr. Fengfeng Bei et al	20 Presentation 10 Q&A	10:45 AM
5	BREAK	All	10	11:15 AM
6	JCR Pharmaceuticals Blood Brain Barrier (confirmed)	Dr. Mathias Schmidt	20 Presentation 10 Q&A	11:25 AM
7	High Content Phenotype Screening for Drug Repurposing in Farber Disease (confirmed)	Dr. Diego Medina et al	15 Presentation 5 Q&A	11:55 AM
8	Lentivirus Mediated Gene Therapy for the Treatment of SMA-PME and Farber Disease (confirmed)	Dr. Michelle Allen-Sharpley	20 Presentation 10 Q&A	12:10 PM
9	Wrap Up and Date of Next Forum Around the Room (All Bring Calendars)	Ms. Mary Irwin et al	5	12:40 PM
10	Social Time/ Fellowship	ALL	15	12:45 PM
	Total time		4 HOURS	1:00 PM

BACKUP PRESENTERS				
	Acid Ceramidase Deficiency Insights (potentially available)	Dr. Thierry Levade	20 Presentation 10 Q&A	N/A
	SMA-PME/ Farber Disease Birth Prevalence (confirmed)	Dr. Nils Confer	20 Presentation 10 Q&A	N/A