



AGENDA

for the

Second aCDase Deficiency Global Forum For Community Alignment

facilitated by

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

November 13, 2023

4 PM – 6 PM United States Eastern Time

Please Convert All Times to Your Time Zone

Zoom Link:

Zoom Meeting ID: 892 6761 7061

Password: 121212

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

Pre-Forum ONE HOUR PRIOR TO FORUM:

3 PM US ET: Meet and Greet the Participants (Optional)

3:40 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, SMA-PME Research (Confirmed)	Ms. Mary Irwin	10 Presentation 5 Q&A	4:00 PM
2	Overview: Acid Ceramidase and ERT Status Update (Confirmed)	Dr. Ed Schuchman	15 Presentation 10 Q&A	4:15 PM
3	Cell Therapies for ACDase Deficiencies in Mouse Models (Confirmed)	Dr. Jeffrey Medin	20 Presentation 10 Q&A	4:25 PM
4	BREAK	All	10	4:55 PM
5	Natural History of SMA-PME based on 9 French and American patients	Dr. Sylvestre Cunait	20 Presentation 5 Q&A	5:05 PM
6	Intentions and Plans for the Aceragen Successor Company (Confirmed)	Dr. Nils Confer	15 Presentation 5 Q&A	5:30 PM
7	Wrap Up and Date of Next Forum Around the Room (All Bring Calendars)	Ms. Mary Irwin et al	10	5:50 PM
8	Total time		2 HOURS	6:00 PM

BACKUP PRESENTER				
9	How to Adapt Clinical Trials and Clinical Outcomes for (very) Small Numbers of Patients (Confirmed)	Dr. Laurent Sauvais	20 Presentation 5 Q&A	N/A