



Externally Led Patient-Focused Drug Development Session on Cerebral Creatine Deficiency Syndromes (EL-PFDD on CCDS)

January 24, 2023 | 10:00 AM-3:00 PM EST

Agenda

10:00 – 10:05 AM	Welcome and Opening Remarks: Heidi Wallis, ACD Executive Director
10:05 – 10:15 AM	FDA Remarks - The Role of Patients in Drug Development: Dr. Anna Choe, MD, CDER, FDA
10:15 – 10:30 AM	Clinical Overview of CCDS: Dr. Andreas Schulze, The Hospital for Sick Children
10:30 – 10:35 AM	Overview of Discussion Format: Larry Bauer, Hyman, Phelps, & McNamara Meeting moderator
10:35 – 10:40 AM	*Audience Demographic Polling Questions
<u>Morning Session: Living with CCDS - Symptoms and Daily Impact</u>	
10:40 – 11:10 AM	Panel 1: Patient & caregiver perspectives on CCDS symptoms and daily impacts- 6 panelists (5 minutes each) including: <ul style="list-style-type: none">● 1 AGAT presentation● 2 GAMT presentations● 3 CTD presentations
11:10 – 12:30 PM	*Audience Discussion and remote polling on Topic 1 including: <ul style="list-style-type: none">● 5 panelist discussion starters (3 CTD, 2 GAMT)● *Audience remote polling● *Moderated audience discussion (call in and write in)
12:30 – 1:00 PM	Break
<u>Afternoon Session: Current & Future Approaches to Treatment for CCDS</u>	
1:00 – 1:10 PM	Introduction to Session 2: Dr. Nicola Longo, University of Utah
1:10 – 1:35 PM	Panel 2: Patient & caregiver perspectives on current and future CCDS treatments- 6 panelists (5 minutes each) including: <ul style="list-style-type: none">○ 1 AGAT presentation○ 2 GAMT presentations○ 3 CTD presentations
1:35 – 2:45 PM	*Audience Discussion and remote polling on Topic 2 including: <ul style="list-style-type: none">○ 5 panelist discussion starters (3 CTD, 2 GAMT)○ Audience remote polling○ Moderated audience discussion (call in and write in)
2:45 – 2:55 PM	Meeting Summary: Dr. Sangeetha Iyer, ACD Scientific Advisor
2:55 – 3:00 PM	Wrap Up and Thank You: Heidi Wallis, ACD Executive Director