NAF/CureDRPLA Agenda
Externally Led Patient-Focused Drug Development Meeting
Polyglutamine Spinocerebellar Ataxias (SCA)/DRPLA
September 25, 2020
(All times in EST)

9:30-10:00 a.m. Online Registration: [https://ataxia.org/el-pfdd-meeting/](https://ataxia.org/el-pfdd-meeting/)

10:00-10:05 a.m. Welcome Remarks – NAF Executive Director: Andrew Rosen & Cure DRPLA President & Co-Founder: Andrea Compton

10:05-10:15 a.m. FDA Opening Remarks – Dr. Wilson W. Bryan, MD (Deputy Director of Office of Tissues and Advanced Therapies)

10:15-10:30 a.m. Clinical Features of SCA & Therapeutic Approaches – Dr. George Wilmot, MD, PhD, Associate Professor in Movement Disorders at Emory University

10:30-10:35 a.m. Meeting Overview – James Valentine, Moderator

10:35-10:40 a.m. Demographic Polling – James Valentine

10:40 a.m.-11:20 p.m. Topic 1: Living with SCA /DRPLA (How do the symptoms and health effects impact patient lives? What most worries patients?)

- Panel 1A (4 panelists): Ataxia and other neuromuscular impacts (balance, chorea, spasticity, weakness, reflexes, and gait) (20 mins)
- Panel 1B (4 panelists): Other symptoms and health effects (visual problems, speech, swallowing, memory and dementia, epilepsy, behavior issues) (20 mins)

11:20-12:30 p.m. Polling & Audience Discussion

12:30-1:00 p.m. Lunch

1:00-1:05 p.m. Featured Video of Severely Impacted Individual
1:05-1:35 p.m.- **Topic 2: Approaches to Treatments for SCA/DRPLA**
(What considerations for clinical trials are most important to SCA/DRPLA patients? How do current treatment approaches help and what burdens do they add? What do patients/caregivers want from a future treatment?)
- Panel 2 (30 mins)

1:35-2:45 p.m.- **Polling & Audience Discussion**

2:45-2:50 p.m.- **Summary Remarks** *(Larry Bauer)*

2:50-3:00 p.m.- **Concluding Remarks & Next Steps** – NAF & CureDRPLA Leadership