Agenda

Wednesday, November 20th – Cypress Room	Day 0
Welcome Reception	
5:30-6:00pm	**Registration**
6:00-6:10pm	**Opening Remarks**
6:15-6:45pm	**Elizabeth M. Berry-Kravis, MD, PhD; RUSH University Medical Center** *"Applying lessons learned from a drug development journey in rare diseases"*
6:45-7:00pm	**Jon Mink, MD; Consultant** *"Overview of the Batten Centers of Excellence"*
7:00-8:00pm	**Social -** *Cocktails and hors d'oeuvres served*

Thursday, November 21st – Magnolia Room	Day 1
7:00-8:00am	**Breakfast Buffet**
8:00-8:10am	**Welcome and Opening Remarks**
Developing new therapies for rare lysosomal disorders: Pitfalls and Promise Session Chair: Dr. Jill Weimer and Dr. Sandra Nusinoff Lehrman	
8:10-8:35am	**Jon Mink, MD; Consultant** *"Preparing for and conducting clinical trials in Batten Disease – Lessons learned and thoughts for the future"*
8:35-9:00am	**Erika Augustine, MD; Kennedy Krieger Institute, Johns Hopkins University** *"TBD"*
9:00-9:25am	**Angela Schulz, MD, PhD; University Medical Center Hamburg-Eppendorf Children's Hospital** ***"****Advantages of a multidisciplinary collaboration of pharma, academic and clinical teams to advance drug development for rare diseases"*
9:25-9:50am	**Patti Dickson, MD; Washington University in St. Louis** "*We need to do something: Developing therapies for MPS disorders"*
9:50-10:15am	**Moderated Session 1**
10:15-10:30am	**Break**
Innovative Strategies for patient care and clinical trial design Session Chair: Dr. Jon Mink & David Kennicott	
10:30-10:55am	**PJ Brooks, PhD; National Institutes of Health** *"Beyond 'one disease at a time': therapeutic platforms for clinical trials of monogenic disease"*
10:55-11:20am	**Joe Zabinski, PhD, MEM; OM1** *"Real-world data and AI: Improving understanding of diagnosis and patient trajectories in Batten Disease"*
11:20-11:45am	**Miriam Nickel, MD; Children's Hospital, University Medical Center Hamburg-Eppendorf** *"Leveraging natural history data in CLN1 clinical trial design: Can we overcome*

	phenotypic variability to advance clinical trial readiness?"
11:45-12:10pm	**Moderated Session 2**
12:10-1:00pm	**Lunch Buffet**
Natural History Studies & Diagnostic/Prognostic Biomarkers Session Chair: Dr. Erika Augustine & Dr. Ineka Whiteman	
1:00-1:25pm	**An Dang Do, MD, PhD; National Institutes of Health** *"CLN3 Natural History Study and the NCL Research Village"*
1:25-1:50pm	**Jennifer Vermillion, MD; University of Rochester** *"Partners in Discovery: The Essential Role of Family Collaboration in Advancing Rare Disease Natural History Research"*
1:50-2:15pm	**Jon Brudvig, PhD; Sanford Research** *"Blood-based Translational Multiomics Biomarker Discovery for NCLs"*
2:15-2:40pm	**Tom Wishart, PhD; Roslin Institute** *"Utility of Large Animal Models for Biomarker Identification"*
2:40-3:00pm	***Break***
3:00-3:25pm	**John Foxe, PhD; University of Rochester** *"Cross-species Neuromarkers in Batten Disease – Toward more sensitive outcome measures in clinical trials"*
3:25-3:50pm	**Ryan Roemmich, PhD; Kennedy Krieger Institute, Johns Hopkins University** *"Applications of Computer Vision in Human Movement Assessment"*
3:50-4:15pm	**Moderated Session 3**
Exploring Atypical Batten Disease Phenotypes Session Chair: Dr. Steven Gray & Jennifer VanHoutan	
4:15-4:40pm	**Jon Cooper, PhD; Washington University in St Louis, School of Medicine** *"Characterizing and Treating the Effects of Batten Disease Outside of the CNS"*
4:40-5:05pm	**Kourtney Santucci, MD; Colorado Children's** *"Atypical Presentations of Batten Disease"*
5:05-5:30pm	**Angela Schulz, MD, PhD; Children's Hospital, University Medical Center Hamburg-Eppendorf** *"TBD"*
5:30-5:55pm	**Moderated Session 4**
6:15-8:15pm	**Dinner – Lilac Room**

Friday, November 22nd – Magnolia Room	Day 2
7:00-8:00am	**Breakfast Buffet**
Small Molecules, ERTs, Chaperones, and Biologicals: Session Chair: Dr. Jon Cooper & Karen Khan	
8:00-8:25am	**May Khanna, PhD; University of Florida** *"In Silico Targeting of Protein and RNA for Batten Disease Therapeutics"*
8:25-8:50am	**Lisa Julian, PhD; Simon Fraser University** *"Human Pluripotent Stem Cell Modeling Approaches to Precipitate Therapeutic Compound Screening in CLN3 Batten Disease"*
8:50-9:15am	**Russell Gotschall; M6P Therapeutics** *"Harnessing the CIMPR to Develop Effective Therapies for Lysosomal Storage Diseases"*
9:15-9:40am	**Sean Ekins, PhD, DSc.; Collaborations Pharmaceuticals Inc.** *"An ERT for CLN1: Progress Toward the Clinic"*
9:40-10:05am	**Moderated Session 5**
10:05-10:20am	**Break**
Viral Mediated Gene Correction – AAV Based: Session Chair: Dr. Michelle Hastings & Amy Fenton Parker	
10:20-10:45am	**Steve Gray, PhD; UT Southwestern** *"The Translational Journey of Gene Therapies for CLN1, CLN5, and CLN7"*
10:45-11:10am	**Christina Ohnsman, MD; Tern Therapeutics** *"Update on Tern Therapeutics Gene Therapy Programs for the Treatment of CLN2 Disease"*
11:10-11:35pm	**Elizabeth M Berry-Kravis, MD, PhD; Rush University Medical Center** *"Gene Therapy for CLN1: A Creative Mechanism for Access"*
11:35 -12:00pm	**Nadia Mitchell, PhD; Lincoln University, NZ** *"Translational CLN5 Gene Therapies: Lessons to be Learnt from Sheep"*
12:00-1:00pm	**Lunch Buffet - Magnolia Room**
1:00-1:25pm	**Moderated Session 6**
Viral Mediated Gene Correction – Ex vivo Lentiviral Approaches: Session Chair: Dr. May Khanna & Steve Lehrman	
1:25-1:50pm	**Alessandra Biffi, MD; Padua University Hospital Italy** *"TBD"*
1:50-2:15pm	**Paul Heal; Orchard Therapeutics** *"Regulatory Approval for Ex-Vivo HSC Therapies – A Case Study of Lenmeldy"*
2:15-2:40pm	**Jeffrey Medin, PhD; Medical College of Wisconsin** *"Lentivirus-mediated Gene Therapy Using Autologous Cells for LSDs that Have Soluble Hydrolases"*

2:40-3:05pm	**Moderated Session 7**
3:05-3:20pm	**Break**
Nucleic Acid and Gene Editing Therapeutic Approaches: Session Chair: Dr. Tom Wishart & Tracy VanHoutan	
3:20-3:45pm	**Michelle Hastings, PhD; Pfizer Upjohn Professor of Pharmacology, Director RNA Therapeutics; University of Michigan** *"Project Butterfly: The Making of a Personalized Antisense Oligonucleotide Medicine"*
3:45-4:10pm	**Tim Yu, Associate Professor; Boston Children's Hospital Harvard Medical School** *"Individualized ASO Strategies for CLN7 Batten Disease"*
4:10-4:35pm	**Tiffany Sepp, Program Leader, ForeBatten Foundation CEO; Vanguard Clinical, Inc.** *"The Metamorphosis of Project Butterfly: An N of 2 Road Map"*
4:35-5:00pm	**Moderated Session 8**