BDSRA foundation

2025 Family Conference

SOWING HOPE FOR A CURE
July 11 - 13
Lincoln, Nebraska

RESEARCH PLENARY PROGRAM

Friday July 11, 9am-12noon

Session Welcome & Overview – Dr Ineka Whiteman

Part I. RAPID FIRE ABSTRACTS

Talk No.	NAME	INSTITUTE/AFFILIATION	Presentation title
1	David Pearce	International Rare Disease Research Consortium (IRDiRC), SD	Global Programs Impacting Rare Diseases
2	Jon Cooper	Washington University St Louis, MI	Setting the Scene - An Overview of Batten Disease Research
3	Jon Cooper	Washington University St Louis, MI	What is happening outside the brain? Bowels, nerves and muscles
4	Katie Lyons	Washington University St Louis, MI	Friend or foes? What is the brain's immune system doing in CLN1 disease?
5	Ruchira Singh	University of Rochester Medical Center, NY	Retina structure and function in CLN3 disease patients and patient-derived retina cell model guide development of rational therapeutics.
6	Amy Vierhile &	University of Rochester Batten Center, NY	From Onset to Outcome: Understanding the Natural History of the NCLs
7	Bridget Patterson	Parent Representative	Natural History Studies: A Family Perspective
8	Melissa Pratt	Sanford Research, SD	Identifying Biomarker Signatures for Batten Disease Through Blood-Based Analyses
9	An Dang Do *(VIRTUAL)*	National Institute of Child Health and Human Development, National Institutes of Health, Bethesda, MD	CLN3 Observational Study and Biomarker Discoveries
10	Paul Trippier	University of Nebraska Medical Center, NE	Early-Stage Drug Discovery for CLN3 and CLN6 Disease
11	Eve Lang	University Rochester School Medicine, NY	Investigation of basic auditory and language processing using high density EEG in Batten disease
12	Jennifer Vermilion	University of Rochester Medical Center, NY	Building Excellence: Year One of the BDSRA Foundation Centers of Excellence Program
13	Jennifer Vermilion	University of Rochester Medical Center, NY	Towards Innovation in Batten Disease Clinical Trials: Results of a National Multi-stakeholder Conference on Master Protocol Design in Rare Disease
14	Jenna Soper	Latus Bio Inc., PA	Preclinical studies evaluating the biodistribution, safety, and pharmacodynamics of LTS-101, a novel gene therapy for CLN2 Batten Disease.

Part II. UPDATES FROM THE CLINICAL TRIAL PIPELINE

Talk No.	NCL TYPE	AFFILIATION	SPEAKER	TITLE
1	Overview	BDSRA Foundation	Ineka Whiteman	Understanding clinical trials
2	CLN1	Rush University Medical Center/ Taysha Gene Therapies	Elizabeth Berry-Kravis *(VIRTUAL)*	CLN1 gene therapy program update
3	CLN2	BioMarin Pharmaceutical	Ineka Whiteman (BDSRA)	Cerliponase alfa (Brineura) ongoing programs update
4	CLN2	LatusBio	Suzanne Plezier	LTS-101 gene therapy program update (pre-IND)
5	CLN2	Tern Therapeutics	Christina Ohnsman	TTX-381 and TTX-181 gene therapy program update
6	CLN3	Alcyone Therapeutics	Kathrin Meyer	CLN-301 gene therapy program update
7	CLN3	Beyond Batten Disease Foundation/Theranexus	Mary Beth Kiser	Batten-1 (miglustat) program update
8	CLN3	ForeBatten Foundation/UNC	Yael Shiloh-Malawsky	FBF-001 (Zebronkysen) Antisense Oligonucleotide (ASO) program update
9	CLN3	Vanguard Clinical Rare Disease Foundation	Tiffany Sepp	VCRDF-CLN3 ASO for CLN3 common mutation (pre-IND)
10	CLN5	Neurogene Inc	Ineka Whiteman (BDSRA)	NGN-101 gene therapy program update
11	CLN6	Charlotte & Gwenyth Gray Foundation	Tiffany Sepp Kristen Gray *(VIRTUAL)*	Gene therapy program update
12	CLN7	University of Texas Southwestern / Elpida Therapeutics	Ineka Whiteman (BDSRA)	Gene therapy program update