

NETWORK-LINKED OPEN CONFERENCE AGENDA
VIRTUAL MEETING

Friday, May 30, 2025 10:00 AM – 4:00 PM ET

INTRODUCTION	
10:00 AM – 10:15 AM	Welcome and Day Overview <i>Jennifer Vermilion, MD</i> <i>University of Rochester Medical Center</i>
	Rare Disease Clinical Trials <i>Erika Augustine, MD, MS</i> <i>Kennedy Krieger Institute</i>
MASTER PROTOCOL DESIGN AND CASE EXAMPLES	
10:15 AM – 11:30 AM	Accelerating Drug Development Through Innovative Clinical Trial Design <i>Melanie Quintana, PhD</i> <i>Berry Consultants</i>
	Driving Innovation in ALS: The HEALEY ALS Platform Trial <i>Sabrina Paganoni, MD, PhD</i> <i>Massachusetts General Hospital</i>
	Neurofibromatosis Platform Trial <i>Scott Plotkin, MD, PhD</i> <i>Massachusetts General Hospital</i>
	Q&A
DESIGNING A MASTER PROTOCOL TRIAL IN BATTEN DISEASE	
12:15 PM – 1:30 PM	Clinical Trials Transformation Initiative: Approach to Master Protocol Design <i>Sara Cahvert, PharmD</i> <i>Duke University</i> <i>Clinical Trials Transformation Initiative</i>
	Towards Trial Readiness in the NCLs <i>Jennifer Vermilion, MD</i> <i>University of Rochester Medical Center</i>
	The Batten Disease Support Research and Advocacy Foundation <i>Ineka Whiteman, PhD</i> <i>BDSRA Foundation</i>
	Emerging Therapies: The NCL Pipeline <i>Raymond Wang, MD</i> <i>Children's Hospital Orange County</i>
	Q&A
BREAKOUT SESSIONS	
1:45 PM – 4:00 PM	Breakout Session Topics: <i>Umbrella Trial Development</i> • <i>Basket Trial Development</i> Session 1 Session 2 Breakout Summaries Summary and Action Items