

Research Update: Public Private Partnerships for ALS and Other Rare Neurodegenerative Diseases

June 5, 9am – 12pm ET

Meeting Agenda

Time	Session	Speakers
9:00-9:10 ET	Welcome & Opening	
9:00 – 9:05 am ET	Open, Welcome, Housekeeping	<p>Tanya Williams, MPH Sr. Project Manager, CP-RND Critical Path Institute (C-Path)</p> <p>Megan Miller, PhD Director of Outreach</p>
9:05 – 9:10 am ET	Opening remarks: Four years of progress in ALS and rare neurodegenerative diseases pursuant to the ACT for ALS and Other Initiatives	<p>Teresa Buracchio, MD Director, Office of Neuroscience, CDER U.S. Food and Drug Administration (FDA)</p>
9:10-9:25 ET	Community Perspectives: Research Partnerships in ALS	
9:10 – 9:25 am ET	Moderated Discussion	<p><u>Moderator:</u> Megan Miller, PhD Director of Outreach, C-Path</p> <p><u>Panelists:</u> Cathy Collet Caregiver and Independent ALS Advocate</p> <p>Lynn Brielmaier Person living with ALS</p>
9:25-10:30 ET	Updates from FDA & C-Path: Building a Trial Ready System for ALS and other Rare Neurodegenerative Diseases	
9:25 – 9:55 am ET	Updates from FDA	<p>Michelle Campbell, PhD Associate Director, Stakeholder Engagement and Clinical Outcomes, OND U.S. Food and Drug Administration</p> <p>Katherine Needleman, MS, PhD, RAC Director, Orphan Products Grants Program U.S. Food and Drug Administration</p>
9:55 – 10:10 am ET	Updates from CP-RND: ALS	Collin Hovinga, PharmD, MS, FCCP

		Vice President, Rare, Orphan and Pediatric Diseases Critical Path Institute
10:10 -10:20 am ET	Updates from CP-RND: HD	Terina Martinez, PhD Executive Director, Huntington’s Disease Regulatory Science Consortium (HD-RSC) and Critical Path to Therapeutics for the Ataxias (CPTA) Critical Path Institute
10:20 – 10:25 am ET	Community Perspective: Research Partnerships in HD	Lauren Holder Producer and Host of <i>Help 4 HD Live</i>
10:25 – 10:30 am ET	Audience Q&A	Katie O’Keefe, MS Scientific Director Critical Path for Rare Neurodegenerative Diseases (CP-RND) Critical Path Institute
10:30-10:40 ET	Break	
10:40-11:25 ET	Updates from NIH and FINIH: Collaborating to Accelerate Research in ALS	
10:40 – 11:02 am ET	Updates from NIH	Amelie Gubitz, Ph.D. Program Director, Division of Neuroscience National Institute of Neurological Disorders and Stroke
11:02 – 11:20 am ET	Updates from AMP-ALS	Shubhangi Lal, B. Pharm, MS, MBA Senior Project Manager, Translational Science Neuroscience Foundation for the National Institutes of Health
11:20 – 11:25 am ET	Audience Q&A	Katie O’Keefe, MS Scientific Director, Rare Neurodegenerative Diseases Critical Path for Rare Neurodegenerative Diseases (CP-RND) Critical Path Institute
11:25-11:55 ET	Community Perspectives: The Future of RND Research	
11:25 – 11:55 am ET	Moderated Panel Discussion	<u>Moderator:</u> Michelle Campbell, PhD

		<p>Associate Director, Stakeholder Engagement and Clinical Outcomes, OND U.S. Food and Drug Administration</p> <p><u>Panelists:</u></p> <p>Fernando Vieira, MD CEO and Chief Science Officer ALS-TDI</p> <p>Andrew Rosen, MBA Chief Executive Officer National Ataxia Foundation</p> <p>Brian Lin, PhD Director, Research Portfolio Muscular Dystrophy Association, USA</p> <p>Penny Dacks PhD Chief Scientific Officer, AFTD President, FTD Disorders Registry</p>
11:55-12:00 ET	Closing	
11:55 am – 12:00 pm ET	Closing Remarks	<p>Collin Hovinga, PharmD, MS, FCCP Vice President, Rare, Orphan and Pediatric Diseases Critical Path Institute</p>

