Agenda:

9:00 a.m.

Welcome Address

Benjamin Forred – Director, Clinical Research – Genetics & Genomics and the CoRDS Registry

9:15 a.m.

The Role of Autophagy in Beta-Propeller Protein-Associated Neurodegeneration (BPAN)

Tassula Proikas-Cezanne, PhD - Professor of Molecular Biology and Cell Biology

10:15 a.m.

Making Miracles: Patient-Driven Therapies for Rare Diseases

Patricia Dickson, MD - Professor of Pediatrics and Genetics, Washington University in St. Louis

11:15 a.m.

On the Brink of Treatment Revolution for Inherited Nervous System Disorders

Steven Gray, PhD - Associate Professor, University of Texas Southwestern Medical Center

12:15 p.m.

The Involvement of Astrocytes in Rare Neurological Diseases

Kevin Francis, PhD - Assistant Scientist, Sanford Research

1:15 p.m.

Importance of properly glycosylated rhGAA for developing effective enzyme replacement therapy for Pompe disease.

Hung Do, PhD – Chief Scientific Officer, Amicus Therapeutics