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, Eberhard Karls University, Tübingen, Germany

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