

10TH ANNUAL

Sanford Virtual Rare Disease Symposium

Friday, October 16th, 2020

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RESEARCH

## 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