ABOUT AVROBIO

AVROBIO’s mission is to free people from a lifetime of genetic disease with a single dose of gene therapy. We aim to halt or reverse disease progression throughout the body by driving durable expression of functional protein even in hard-to-reach tissues and organs including the brain, muscle and bone. Our clinical-stage programs include Fabry disease, Gaucher disease and cystinosis and we are also advancing a program in Pompe disease. AVROBIO is powered by plato™, our streamlined platform designed to enhance patient outcomes and deliver anticipated large-scale commercial manufacturing. We are headquartered in Cambridge, Mass., with field offices in Toronto, Ontario.

For additional information, visit avrobio.com.

AGENDA

6:45 – 7:05 PM  
Chair’s welcome: Gene therapy - past, present and future  
Harry L. Malech, M.D.  
National Institutes of Health, Bethesda, MD

7:05 – 7:20 PM  
Cystinosis: Investigative gene therapy to deliver functional lysosomal transporter protein  
Stephanie Cherqui, M.D.  
University of California, San Diego, San Diego, CA

7:20 – 7:25 PM  
Q & A

7:25 – 7:40 PM  
Gaucher disease: Targeting the underlying genetic cause to address unmet medical needs  
Aneal Khan, M.Sc., M.D., FRCPC, FCCMG  
University of Calgary Cumming School of Medicine, Calgary, Canada

7:40 – 7:45 PM  
Q & A

7:45 – 8:00 PM  
Fabry disease Phase 1: Updated interim results and perspectives  
Jeffrey A. Medin, Ph.D.  
Medical College of Wisconsin, Milwaukee, WI

8:00 – 8:15 PM  
Fabry disease Phase 2, treatment-naïve patients: Updated interim results and perspectives  
Mark Thomas, MBBS (Syd.), FRACP  
Royal Perth Hospital, Perth, Australia

8:15 – 8:25 PM  
Q & A

8:25 – 8:30 PM  
Chair’s concluding remarks  
Harry L. Malech, M.D.