



The Baltimore Marriott Inner Harbor at Camden Yard  
110 S. Eutaw St., Baltimore, MD 21201

TIME	PRESENTATION	SPEAKER(S)	INSTITUTION
9:00 AM - 9:15 AM	<b>SLC6A1 Connect Update</b>	Amber N. Freed	SLC6A1 Connect
9:15 AM - 9:20 AM	<b>Moderator Introduction</b>	Kimberly Nye	Tess Research Foundation
<b>Clinical Overview</b>			
9:20 AM - 9:35 AM	<b>The Clinical Presentation of SLC6A1</b>	Katherine L. Helbig, MC, LCGC	Children's Hospital of Philadelphia
9:35 AM - 9:55 AM	<b>Simons Searchlight Registry Findings for SLC6A1</b>	Jennifer Bain, MD, PhD	Columbia University Medical Center
9:55 AM - 10:05 AM	Q&A		
<b>Characterization &amp; Mouse Models</b>			
10:05 AM - 10:25 AM	<b>Characterization of Variant Tolerant and Intolerant Sites in SLC6A1 Dysfunction</b>	Dennis Lal, PhD	Cleveland Clinic & Broad Institute
10:25 AM - 10:45 AM	<b>Cellular In Vitro Modeling for SLC6A1</b>	Julieth Sierra Delgado, D, MD, M.Sc.	Nationwide Children's Hospital
10:45 AM - 11:00 AM	<b>Mouse Models for SLC6A1</b>	Frances Shaffo, PhD	University of Texas Southwestern
11:00 AM - 11:15 AM	Q&A		
<b>Lunch</b>			
11:15 AM - 12:05 PM	<b>Buffet Lunch and Networking</b>		
<b>The Basics of GABA Science</b>			
12:05 PM - 12:20 PM	<b>GABAergic Systems and the Role of GAT1</b>	Amy Brooks-Kayal, MD	Children's Hospital of Colorado
12:20 PM - 12:40 PM	<b>Model SLC6A1 Mutation Associated Disorders from Stem Cells to Mice</b>	Katty Kang, MD, PhD	Vanderbilt University
12:40 PM - 12:50 PM	Q&A		
<b>Pharmacological Treatment</b>			
12:50 PM - 1:10 PM	<b>Pharmacological Thoughts for SLC6A1</b>	Michael Rogawski, MD, PhD	University of California, Davis
1:10 PM - 1:25 PM	<b>Potential Drug Targets for SLC6A1</b>	Ana C. Puhl, PhD	Collaborations Pharmaceutical
1:25 PM - 1:40 PM	<b>Flourescent Sensors for Neuroscience</b>	Jonathan Marvin, PhD	Howard Hughes Medical Institute
1:40 PM - 1:55 PM	Q&A		
<b>Anti-Sense Oligonucleotides</b>			
1:55 PM - 2:15 PM	<b>Anti-Sense Oligonucleotide Treatment</b>	Athma Pai, PhD	University of Massachusetts
2:15 PM - 2:35 PM	<b>Stoke's TANGO Upregulation Update</b>	Gene Liau, PhD	Stoke Therapeutics
2:35 PM - 2:45 PM	Q&A		
<b>Gene Replacement Therapy</b>			
2:45 PM - 3:15 PM	<b>Gene Replacement Therapy</b>	Steven Gray, PhD	University of Texas Southwestern
3:15 PM - 3:25 PM	Q&A		
<b>Bathroom &amp; Snack Break</b>			
3:25 PM - 3:35 PM			
<b>Gene Therapy Manufacturing Considerations</b>			
3:35 PM - 3:50 PM	<b>Considerations and Approaches for GMP Manufacturing of Viral Vectors for Gene Therapy</b>	Nick Conley, PhD	Locus Biosciences
3:50 PM - 4:00 PM	Q&A		
<b>Panel Discussions</b>			
4:00 PM - 4:30 PM	<b>Clinical Research &amp; Trial Panel</b> <b>Moderator:</b> Scott Demarest, MD <b>Topics:</b> Natural History Study/Clinical Trial Design/Outcome Measures	Katherine L. Helbig, MC, LCGC Katrine M Johannesen, MD, PhD Kimberly Goodspeed, MD Scott Demarest, MD Tracy Dixon - Salazar, PhD	Children's Hospital of Philadelphia The Danish Epilepsy Center University of Texas Southwestern Children's Hospital of Colorado LGS Foundation
4:30 PM - 5:00 PM	<b>Basic/Translation Research Panel</b> <b>Moderator:</b> Steven Gray, PhD <b>Topics:</b> Model Development/Gene Therapy/Drug Screening/Future Areas of Focus	Amy Brooks-Kayal, MD Athma Pai, PhD Michael Rogawski, MD, PhD Steven Gray, PhD	Children's Hospital of Colorado University of Massachusetts University of California, Davis University of Texas Southwestern