

Engaging the Field: New Clinical Approaches to Disease Modification in HD

Time	Topic	Speaker
08:00-08:15	Gather – Coffee/Continental Breakfast	
08:15-08:30	Welcome	Dan van Kammen, MD, PhD
	<u>mHtt Lowering: Role of ASOs and RNAi</u>	
08:30-08:55	ASOs and RNAi Therapies for HD: An overview	Neil Aronin, MD
08:55-09:20	The ALS Experience: What are the challenges?	Timothy Miller, MD, PhD
	<u>HD and Energetics</u>	
09:20-09:45	Mitochondrial Dysfunction in HD: The state of the science	Bernhard Landwehrmeyer, MD
09:45-10:10	The MITO-HD Study	Ronald Haller, MD
10:10-10:20	Coffee Break	
	<u>Disease Modification in HD</u>	
10:20-10:55	The Use of a Disease Model in Planning Disease Modifying Clinical Trials	Doug Langbehn, MD, PhD John Warner, PhD
	Disease Modeling and Disease Modification in Parkinson's Disease	Yaning Wang, PhD
10:55-11:20	What are the best endpoints for HD disease modification?	Sarah J. Tabrizi, MD, PhD
11:20-11:45	A Regulator's Perspective on the Disease Modifying Challenge	Cristina Sampaio, MD, PhD
11:45-12:45	Lunch	
	<u>Workshop Session</u>	
12:45-13:00	Explanation of the Workshop Session	
13:00-14:30	Participants will break up into working groups – each group will deal with a different set of HD drug development questions	
14:30-14:40	Break	
14:40-16:00	Each working group will make a brief presentation on how they would deal with the HD drug development challenges	
16:00-16:15	Closing Remarks – Wrap-up	