

Genetic Strategies to Treat the Epilepsies Workshop AGENDA

June 20 – June 21, 2024

June 20, 2024 (Day 1)

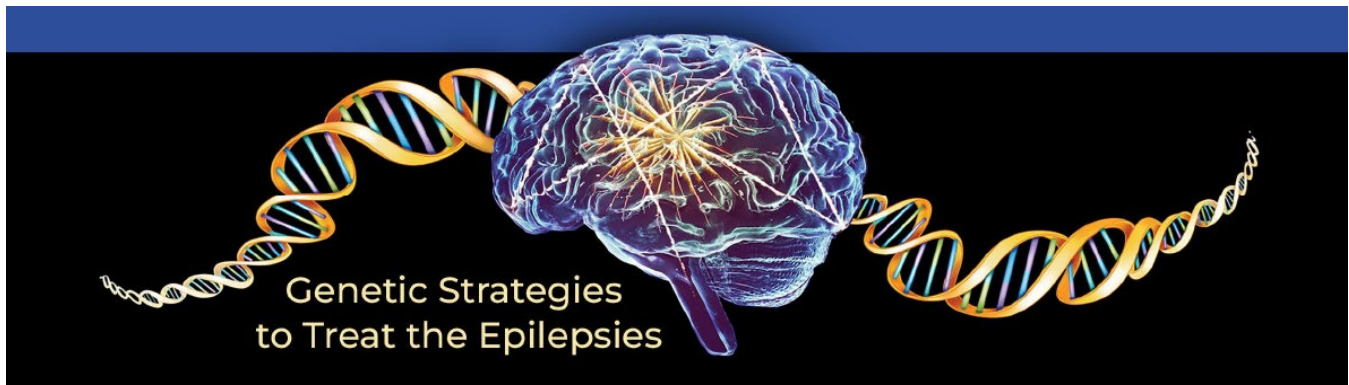
All times Eastern

- 9:00 AM Introduction and Welcome**
Walter Koroshetz, MD
Director, NIH/NINDS
- 9:15 AM Setting the Stage - Goals of the Workshop**
Vicky Whittemore, PhD
NIH/NINDS
- 9:25 AM Action Potential: An Effort to Develop Genetic Strategies for a Rare Neurological Disease**
Simon Frost
Tiber Capital Group

Session I: State-of-the-art in Genetic Strategies for Treatment

Moderator: Dan Lowenstein, MD; University of California, San Francisco

- 9:50 AM Introduction to Session**
Dan Lowenstein, MD
- 10:00 AM Developing Circuit Based Gene Therapies**
Beverly Davidson, PhD
University of Pennsylvania
- 10:25 AM A Clinically Editable Brain Is Within Sight: Track Record and Trajectory of CRISPR For Neurologic Disease**
Fyodor Urnov, PhD
University of California, Berkeley
- 10:50 AM Break**
- 11:10 AM Prenatal Therapies for Neurologic Diseases**
Tippi Mackenzie, MD
University of California, San Francisco (via Zoom)



11:35 AM **Panel Discussion**

12:00 PM **Lunch**

1:00 PM **Poster Session for Young/Early Career Investigators**

Session II: Progress, Pitfalls and Lessons Learned from Neurological and Non-Neurological Trials/Treatments

Moderator: Ann Poduri, MD, MPH; Boston Children's Hospital

2:00 PM **Introduction to Session**
Ann Poduri, MD, MPH

2:10 PM **Gene Therapy as a Platform: From Giant Axonal Neuropathy to the PaVeGT Project and the Bespoke Gene Therapy Consortium**
Carsten Bonnemann, MD
NIH/NINDS

2:35 PM **Use of ASOs for Genetic Correction of Angelman Syndrome**
Elizabeth Berry Kravis, MD, PhD
Rush University

3:00 PM **Risks and Treatment Choices for Neurological Diseases**
Florian Eichler, MD
Massachusetts General Hospital

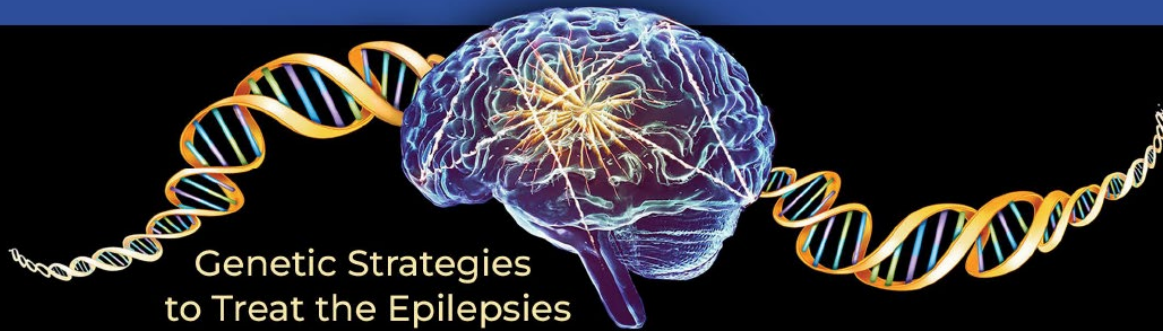
3:25 PM **Break**

3:45 PM **New Paths to the Waterfall: Lessons Learned from AAV Product Development**
Kathy High, MD
Rockefeller University

4:10 PM **From N of 1 to Population Health to Treat Genetic Epilepsies**
Wendy Chung, MD, PhD
Boston Children's Hospital (via Zoom)

4:35 PM **Panel Discussion**

5:00 PM **Adjourn**



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Session III: Considerations for Designing and Implementing Therapy for the Epilepsies

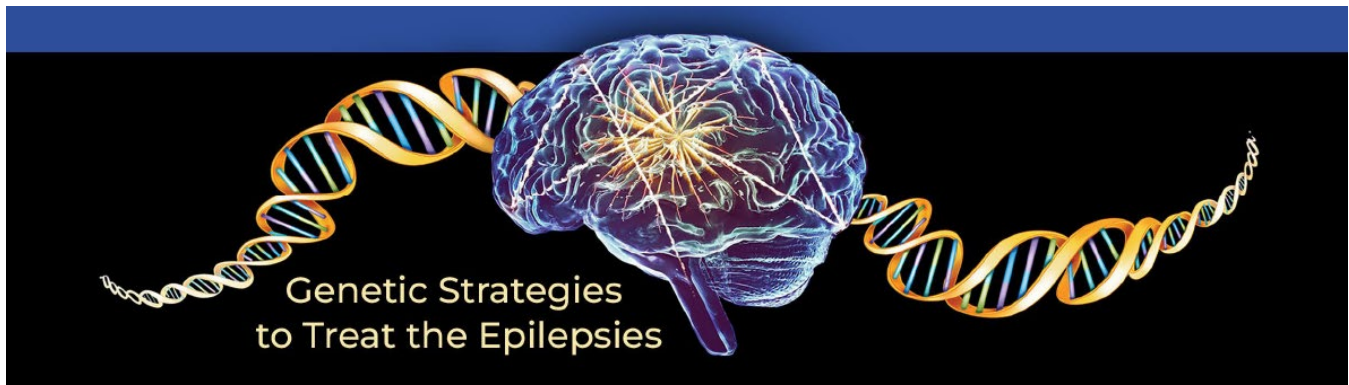
Moderators: Lori Isom, PhD; University of Michigan & Heather Mefford, MD, PhD; St. Jude's Children's Hospital

- 9:00 AM** **Introduction to Session**
Lori Isom, PhD & Heather Mefford, MD, PhD
- 9:10 AM** **Predicting Pathogenicity – Which Variants (Will) Cause Disease? Considering Mechanism – Approaches for Gain vs Loss of Function**
Gemma Carvill, PhD
Northwestern University (via Zoom)
- 9:35 AM** **TANGO as a Therapeutic Strategy for Epilepsy Design & Implementation: Successes and Challenges**
Isabel Aznarez, PhD
Stoke Therapeutics
- 10:00 AM** **Pinpointing the Perfect Timing: Critical Time Windows for Gene Therapy Delivery**
Gaia Colasante, PhD
San Raffaele Hospital
- 10:25 AM** **Panel Discussion**
- 10:45 AM** **Break**

Session IV: Crucial Issues Beyond the Genetic Strategy

Moderators: Vicky Whittemore, PhD; NIH/NINDS & George Umanah, PhD; NIH/NINDS

- 11:00 AM** **Introduction to Session**
Vicky Whittemore, PhD



- 11:10 AM** **Enhancing Global Rare Epilepsy Care Through Online Knowledge Resources**
Dennis Lal, PhD
University of Texas - Houston
- 11:35 AM** **Becoming a Treatable Genetic Condition**
Mary Ann Meskis
Dravet Syndrome Foundation (via Zoom)
- 12:05 PM** **Lunch**
- 1:00 PM** **Informed and Efficient Clinical Trials Design Using Natural History Data and Disease Modeling**
Melanie Quintana, PhD
Berry Consultants
- 1:25 PM** **Considerations for Remote Phenotyping in Pediatric Research**
Heather Hazlett, PhD
University of North Carolina
- 1:50 PM** **Ethical Issues Raised in the Development of Genetic Strategies to Treat Rare Neurological Disorders**
Lainie Ross, MD, PhD
University of Rochester
- 2:15 PM** **Panel Discussion with Discussant**
Rosa Sherafat, MD
FDA
- 2:35 PM** **Wrap-up Discussion**
Moderator: Dan Lowenstein, MD
- 3:00 PM** **Adjourn**