MEETING AGENDA

FMRP Restoration:
Definitive Therapies in Fragile X

April 30-May 3, 2023
The Banbury Center, Lloyd Harbor, New York

This meeting was organized by
Joel Richter, University of Massachusetts Chan Medical School
Michael Tranfaglia, FRAXA Research Foundation

and generously funded by the
FRAXA Research Foundation

SUNDAY, APRIL 30

Afternoon Arrivals and check-in at Robertson House
6:00 pm Reception and dinner

MONDAY, MAY 1

7:15 am Breakfast

8:30 am Welcome; Overview of Meeting, Goals
Rebecca Leshan, The Banbury Center
Katie Clapp, FRAXA Research Foundation

8:50 am SESSION 1: Trial Operations: Collaborations Across Disciplines, Sectors, and Institutions to Enable Early Phase Clinical Trials
Michael Tranfaglia, FRAXA Research Foundation
Julia Sommer, Simons Foundation

10:20 am SESSION 2: ASO Restoration of FMRP
Joel Richter, University of Massachusetts Chan Medical School
Sneha Shah, University of Massachusetts Chan Medical School
Jonathan Watts, University of Massachusetts Chan Medical School
12:00 pm  Luncheon

2:00 pm  SESSION 3: Fragile X Model Systems
Nissim Benvenisty, Hebrew University of Jerusalem
Xinyu Zhao, University of Wisconsin - Madison
Zhexing Wen, Emory University

4:00 pm  SESSION 4: Fragile X Clinical Trials
(v) Craig Erickson, Cincinnati Children’s Hospital
Elizabeth Berry-Kravis, Rush University Medical Center

5:00 pm  Day One Wrap-up

6:00 pm  Reception and dinner

TUESDAY, MAY 2

7:15 am  Breakfast

8:30 am  SESSION 5: Reactivation
Jeannie T. Lee, Harvard Medical School and Massachusetts General Hospital
Daman Kumari, National Institute of Diabetes and Digestive and Kidney Diseases, NIH
Karen Usdin, National Institute of Diabetes and Digestive and Kidney Diseases, NIH

10:30 am  SESSION 6: RNA-based Therapeutics
Kathryn Whitehead, Carnegie Mellon University
Justin Fallon, Brown University
Michelle Hastings, University of Michigan

12:10 pm  Luncheon

2:10 pm  SESSION 7: Developing Therapeutics for Neurodevelopmental Disorders
Donald Lo, European Infrastructure for Translational Medicine (EATRIS)
Pamela Feliciano, Simons Foundation
Mansuo Shannon, Prevail Therapeutics, Eli Lilly

4:10 pm  SESSION 8: Human Electrophysiology
Jean-François Lepage, University of Sherbrooke
Ernest Pedapati, Cincinnati Children’s Hospital

5:10 pm  Day Two Wrap-up

6:00 pm  Reception and dinner
WEDNESDAY, MAY 3

7:15 am  Breakfast

8:30 am  SESSION 9: Integration of Cellular Mechanisms
Lynne E. Maquat, University of Rochester Medical Center
Peng Jin, Emory University School of Medicine
Carissa Sirois, University of Wisconsin – Madison

10:30 am  SESSION 10: Fragile X Syndrome: Where We Are and Where We Are Going
Group Discussion: meeting synthesis, outputs/next steps

12:00 pm  Luncheon
1:30 pm  Participant Departures

PARTICIPANTS

Nissim Benvenisty, Hebrew Univ. of Jerusalem
Elizabeth Berry-Kravis, Rush Univ. Medical Center
Katie Clapp, FRAXA Research Foundation
Craig Erickson, Cincinnati Children’s Hospital
Justin Fallon, Brown University
Pamela Feliciano, Simons Foundation
Michelle Hastings, University of Michigan
Peng Jin, Emory University School of Medicine
Daman Kumari, National Institute of Diabetes and Digestive and Kidney Diseases, NIH
Jeannie T. Lee, HHMI, Harvard Medical School and Massachusetts General Hospital
Jean-François Lepage, University of Sherbrooke
Donald Lo, European Infrastructure for Translational Medicine (EATRIS)
Lynne E. Maquat, Univ. of Rochester Medical Ctr
Sarah Neuhaus, Prevail Therapeutics, Eli Lilly
Ernest Pedapati, Cincinnati Children’s Hospital
Mira Puri, The Azrieli Foundation
Joel Richter, University of Massachusetts Chan Medical School
Sneha Shah, University of Massachusetts Chan Medical School
Mansuo Shannon, Prevail Therapeutics, Eli Lilly
Carissa Sirois, University of Wisconsin - Madison
Julia Sommer, Simons Foundation
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