



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
Zhexiong 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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Digestive and Kidney Diseases, NIH

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Chan Medical School

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