Ethical and Social Policy Considerations of Novel Techniques for Prevention of Maternal Transmission of Mitochondrial DNA Diseases: Workshop

March 31 – April 1, 2015

Keck Center, Room 208
500 Fifth St. NW
Washington, DC

TUESDAY, MARCH 31, 2015

OPEN SESSION

8:30 a.m. Welcome and overview of workshop
Jeffrey Kahn, Johns Hopkins University, Committee Chair

SESSION I: ETHICAL AND SOCIAL IMPLICATIONS OF MRT

Session Objectives:
- Highlight key characteristics of proposed MRT techniques, raising ethical and social issues.
- Discuss the distinctive ethical and social issues that would arise with MRT techniques.

Session Co-Chairs:
R. Alta Charo, University of Wisconsin, Committee Member
Laurie Strongin, Hope for Henry Foundation, Committee Member

9:00 a.m. Kevin FitzGerald, Georgetown University
Thomas Murray, The Hastings Center
Heather Ward, Personal Representative
Hugh Whittall, Nuffield Council on Bioethics
Laurie Zoloth, Northwestern University

9:50 a.m. Discussion with committee and workshop participants

10:20 a.m. Break

1 Committee convened in closed session Monday, March 30 from 1:30 – 5:00 pm.
SESSION II: GERMLINE MODIFICATION

Session Objectives:

- Discuss whether the manipulation of mitochondrial content raises social and ethical issues related to genetic germline modification, and whether the issues raised are similar or different from modification of nuclear DNA.
- Discuss the historical prohibitions on germline genetic modification, the social and ethical considerations that shaped these restrictions, and whether they should be revisited.
- Consider whether it is advisable to establish controls between genetic modification for therapeutic/prevention purposes and for enhancement purposes. What controls could be effective at maintaining this distinction?

Session Co-Chairs:

Jim Childress, University of Virginia, Committee Member
Vamsi Mootha, Harvard Medical School, Committee Member

10:35 a.m. Annelien Bredenoord, University Medical Center Utrecht
Marcy Darnovsky, Center for Genetics and Society
John Evans, University of California, San Diego
John Harris, University of Manchester

11:15 a.m. Discussion with committee and workshop participants
11:45 a.m. Lunch (Cafeteria located on third floor)

SESSION III: POLICY ANALOGUES

Session Objective:

- Discuss unique characteristics of MRT shared with similarly innovative techniques throughout history, and how the policy debates and eventual formulation of policy can be instructive for MRT.

Session Chair:

Jeffrey Kahn, Johns Hopkins University, Committee Chair

12:30 p.m. IVF (including donor gametes)
Nick Hopwood, University of Cambridge (via WebEx)
Rene Almeling, Yale University

12:50 p.m. Discussion with committee and workshop participants

1:10 p.m. Gene transfer in pediatric populations
Benjamin Wilfond, Seattle Children’s Hospital

1:20 p.m. Discussion with committee and workshop participants

1:40 p.m. Human growth hormone (hGH) use in children
Lainie Ross, University of Chicago
1:50 p.m. Discussion with committee and workshop participants

2:10 p.m. Embryo and embryonic stem cell (hES) research
  *Patricia King, Georgetown Law*

2:20 p.m. Discussion with committee and workshop participants

2:40 p.m. Public comment period
  *David McKeon, New York Stem Cell Foundation (3 min)*

2:43 p.m. Break

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**SESSION IV: SCIENCE AND MEDICINE**

Session Objectives:
- Discuss key scientific questions regarding MRT and their ethical and social implications.

  Session Co-Chairs:
  *Alan DeCherney, National Institutes of Health, Committee Member*
  *Marni Falk, The Children’s Hospital of Philadelphia, Committee Member*

3:00 p.m. Data on attitudes of women with mtDNA mutations towards MRT
  *Michio Hirano, Columbia University Medical Center*

3:15 p.m. Discussion with committee and workshop participants

3:30 p.m. Patient perspective on MRT
  *Kirah Fasano, Personal Representative*

3:40 p.m. Discussion with committee and workshop participants

3:55 p.m. Scientific and ethical considerations of mtDNA segregation and the bottleneck phenomenon as it applies to MRT
  *Eric Shoubridge, McGill University*

4:10 p.m. Potential alternative to preventing transmission of mtDNA diseases: heteroplasmym shift therapy
  *Carlos Moraes, University of Miami*

4:25 p.m. Haplogroup compatibility and how mtDNA can influence traits beyond disease
  *Doug Wallace, The Children’s Hospital of Philadelphia*

4:40 p.m. Discussion with committee and workshop participants

5:00 p.m. Adjourn Day One
WEDNESDAY, APRIL 1, 2015

OPEN SESSION

8:30 a.m. Welcome and recap of day one
Jeffrey Kahn, Johns Hopkins University, Committee Chair

SESSION IV: SCIENCE AND MEDICINE (CONTINUED)

Session Objectives:
• Discuss key scientific questions regarding MRT.

Session Co-Chairs:
Alan DeCherney, National Institutes of Health, Committee Member
Marni Falk, The Children’s Hospital of Philadelphia, Committee Member

8:45 a.m. Practical challenges of implementing MRT and potential effects on outcomes
Jacques Cohen, Reprogenetics, LLC

9:00 a.m. Discussion with committee and workshop participants

9:15 a.m. Consideration of potential epigenetic effects of MRT
George Daley, Boston Children’s Hospital

9:30 a.m. Discussion with committee and workshop participants

SESSION V: CLINICAL INVESTIGATIONS

Session Objectives:
• Discuss the preclinical evidence base necessary to support first-in-human MRT research.
• Consider earlier precedents for the collection of safety and efficacy information for novel techniques (e.g., systematically collecting evidence in surgical innovation or IVF).
• Discuss what an ethical clinical investigation of MRT might look like and consider the decision milestones that would occur across the evaluation of MRT.

Session Chair:
Jeffrey Botkin, University of Utah, Committee Member

9:45 a.m. Preclinical evidence base to support an IND for MRT
Wei Liang, U.S. Food and Drug Administration
John Gearhart, University of Pennsylvania
Insoo Hyun, Case Western Reserve University

10:15 a.m. Discussion with committee and workshop participants
10:30 a.m. Designing a systematic investigation of MRT techniques  
Steven Goodman, Stanford Medical School (via WebEx)  
Douglas Turnbull, University of Newcastle upon Tyne (via WebEx)

10:50 a.m. Discussion with committee and workshop participants

11:05 a.m. Designing an ethically acceptable investigation of MRT in the United States  
Rebecca Dresser, Washington University in St. Louis  
Robert Nelson, U.S. Food and Drug Administration

11:25 a.m. Discussion with committee and workshop participants

11:40 a.m. Toleration of uncertainty for new reproductive technologies such as MRT  
Aaron Kesselheim, Harvard Medical School/Brigham and Women’s Hospital  
John Robertson, University of Texas, Austin

12:00 p.m. Discussion with committee and workshop participants

12:15 p.m. Public comment period  
- Brendan Foht, The New Atlantis (3 min)  
- Rick Leach, World Food Program USA (3 min)

12:21 p.m. Adjourn Open Session/Committee Convenes in Closed Session

| CLOSED SESSION |