Public Workshop: Committee on the Independent Review and Assessment of the Activities of the NIH Recombinant DNA Advisory Committee (RAC)

Draft Agenda - August 6, 2013

Keck Center
The National Academies
500 Fifth Street, NW
Washington, DC

Room 100

Workshop Objectives:
- To obtain background information on the state of oversight of gene transfer research
- To obtain background information on the current risks associated with gene transfer
- To explore other models of oversight for related areas of research
- To seek public comment and investigator input about the scientific necessity for extra oversight of gene therapy

SESSION 1: THE STATE OF GENE TRANSFER RESEARCH OVERSIGHT

9:00 a.m. Welcome and Introductory Remarks
Larry Gostin J.D., Committee Chair

9:10 a.m. Panel Discussion
Session Objectives: Understand the current state of regulation of gene transfer research and compare its regulatory landscape to other areas of science. Discuss any models that exist for gene transfer oversight, including those that add value or may no longer be necessary. Explore the investigator experience of individual gene transfer protocol review by the RAC.

Moderator: Howard Federoff, M.D., Ph.D.
Committee Member

Panelists: Barry Byrne, M.D., Ph.D.
Director of the UF Powell Gene Therapy Center
Professor of Pediatrics, Molecular Genetics & Microbiology
Associate Chair of Pediatrics
University of Florida
Helen Heslop, M.D.  
Professor in the Department of Medicine  
Director of Adult Stem Cell Transplant Program  
Baylor College of Medicine

Elizabeth Hohmann, M.D.  
Chair and Director of Partners Human Research Committee  
Partners Healthcare

Carl June, M.D.  
Richard W. Vague Professor in Immunotherapy  
Director of the Translational Research Program  
University of Pennsylvania

Margaret Riley, J.D.  
Professor of Law  
Professor of Medicine  
University of Virginia

10:30 a.m. Panel Discussion  
Session Objectives: Explore the state of clinical gene transfer oversight from the perspective of those who analyze the regulatory context when making decisions about whether to invest finances or scientific resources in the field.

Moderator: Alta Charo, J.D.  
Committee Member

Panelists: Jeffrey Chulay, M.D.  
Chief Medical Officer and Vice President of Regulatory Affairs  
Applied Genetic Technologies Corporation

Todd Foley, M.B.A.  
Managing Director  
MPM Capital

Manuel Litchman, M.D.  
Vice President & Global Program Head, CTL019 Oncology Global Development  
Novartis Pharmaceuticals Corp.
SESSION 3: PATIENT ADVOCACY EFForts AND PERSPECTIVES

11:15 p.m.  **Panel Discussion**

**Session Objectives:** Discuss the patient, consumer, and public perspective on oversight of clinical gene transfer protocols and how patients who may benefit from future gene therapies view the relevant regulatory landscape.

**Moderator:** Sharon Terry  
  *Committee Member*

**Panelists:**  
Nicholas Dainiak, M.D., F.A.C.P.  
*Clinical Professor of Medicine and a Chairman of Medicine*  
*Yale University School of Medicine*

Jennifer Farmer, M.S., C.G.C.  
*Executive Director*  
*Friedreich's Ataxia Research Alliance*

Margie Frazier, Ph.D., L.I.S.W.-S.  
*Executive Director*  
*Batten Disease Support and Research Association*

12:30 p.m.  **Lunch**

SESSION 4: OVERSIGHT OF CONTROVERSIAL SCIENCE

1:45 p.m.  **Panel Discussion**

**Session Objectives:** Explore the policy implications of emerging sciences and the underlying reasons for establishing layers of oversight. Understand overlapping ethical, legal and social issues that warrant elevated scrutiny of gene transfer research and other areas of scientific research. Discuss assessments of oversight in gene transfer research and other areas.

**Moderator:** Jeffrey Kahn, Ph.D., M.P.H.  
  *Levi Professor of Bioethics and Public Policy*  
  *Berman Institute of Bioethics*  
  *Johns Hopkins University*

**Panelists:**  
Alexander Capron, L.L.B.  
*Professor of Law and Medicine*  
*University of Southern California*
Ellen Wright Clayton, M.D., J.D.
Professor of Pediatrics
Professor of Law
Vanderbilt University

Hank Greely, J.D.
Professor of Law
Stanford University

Peter Palese, Ph.D.
Professor and Chair of the Department of Microbiology
Mount Sinai Icahn School of Medicine

Steven Rosenberg, M.D., Ph.D.
Chief of Surgery
National Cancer Institute
National Institutes of Health

3:00 p.m.  Public Comment Period

Harry Malech, M.D.
President-Elect
American Society of Gene and Cell Therapy

3:15 p.m.  Concluding Remarks
Larry Gostin J.D., Committee Chair