

NATIONAL ACADEMIES HUMAN EMBRYONIC STEM CELL RESEARCH ADVISORY COMMITTEE
Board on Life Sciences, National Research Council • Board on Health Sciences Policy, Institute of Medicine

Symposium on Translation of Stem Cells into Clinical Stem Cell Therapeutics

Thursday, November 20, 2008

National Academy of Sciences • Auditorium
2100 C Street, N.W. • Washington, D.C. 20418

AGENDA

8:00 a.m. **Registration**

A light breakfast will be available in the Great Hall. Please note that no food or drink is allowed in the Auditorium.

8:30 a.m. **Welcome and Introduction of Key Issues**

- Richard O. Hynes (committee co-chair), *Daniel K. Ludwig Professor for Cancer Research and Howard Hughes Medical Institute investigator, Massachusetts Institute of Technology*

9:00 a.m. **Session 1: Regulatory issues associated with stem cell therapeutics**

Session Objective: This session will offer perspectives on regulatory issues associated with stem cell therapies. It will help frame the discussion for the rest of the symposium by encouraging participants to consider the steps in the regulatory process that stem cell therapies will have to achieve.

Session Chair: John H. Wagner, Jr. (committee member), *Professor of Pediatrics and Scientific Director of Clinical Research, Stem Cell Institute, University of Minnesota*

Perspective from the U.S. Food and Drug Administration

- Deborah A. Hursh, *Senior Investigator, Division of Cellular and Gene Therapies, Center for Biologics Research and Review, U.S. Food and Drug Administration*

Unique issues associated with stem cell therapeutics

- Darin J. Weber, *Senior Consultant, Cell & Gene Therapies, Biologics Consulting Group, Inc.*

10:00 a.m. Break

10:15 a.m. **Session 2: Case Study on Treatment of Heart Disease**

Session Objective: The first of two case studies on the use of stem cell therapeutics will focus on the treatment of heart disease. Among the issues to be addressed through a series of presentations and discussions are the status of and needs of *in vitro* studies, preclinical steps in animals—including proof of principle, cell toxicity, and human-animal cell chimeras—and proposed clinical trial design—which would include consideration of patient population and the risk:benefit ratio.

Session Chair: Kenneth Chien, *Scientific Director, Cardiovascular Research Center, Massachusetts General Hospital; Harvard Stem Cell Institute; Charles Addison and Elizabeth Ann Sanders Professor of Medicine and Professor of Cell Biology, Harvard Medical School and Harvard Stem Cell Institute*

State of knowledge on clinical applications

- Richard T. Lee, *Department of Medicine, Brigham and Women's Hospital, and Associate Professor of Medicine, Harvard Medical School*

Use of animal models to cardiovascular application

- Christine Mummery, *Professor of Developmental Biology and Chair, Department of Anatomy and Embryology, Leiden University Medical Center*

Technical and clinical challenges in stem cell therapeutics

- Kenneth Chien, *Scientific Director, Cardiovascular Research Center, Massachusetts General Hospital; Harvard Stem Cell Institute; Charles Addison and Elizabeth Ann Sanders Professor of Medicine and Professor of Cell Biology, Harvard Medical School and Harvard Stem Cell Institute*

12:15 p.m. **Lunch**

Box lunches will be available in the Great Hall. Please note that no food or drink is allowed in the Auditorium.

1:15 p.m. **Session 3: Case Study on the Treatment of Parkinson's Disease**

Session Objective: The second case study on the use of stem cell therapeutics will focus on the treatment of Parkinson's Disease and applications to the central nervous system. Among the issues to be addressed through a series of presentations and discussions are the status of and needs of *in vitro* studies, preclinical steps in animals—including proof of principle, cell toxicity, and human-animal cell chimeras—and proposed clinical trial design—which would include consideration of patient population and the risk:benefit ratio.

The committee wishes to thank Fred H. Gage, *Salk Institute for Biological Studies*, for his help in organizing this session.

Session Chair: Lawrence S.B. Goldstein, *Howard Hughes Medical Institute Investigator, Professor of Cellular and Molecular Medicine, and Director, UCSD Stem Cell Program, University of California, San Diego*

A clinician's view on stem cell therapies for Parkinson's Disease

- Olle Lindvall, *Professor of Neurology, Laboratory of Neurogenesis and Cell Therapy, Wallenberg Neuroscience Center, Lund University Hospital, Sweden*

Research challenges of stem cell therapeutics

- Lorenz Studer, *Director, Laboratory of Stem Cell & Tumor Biology, and Associate Member, Memorial Sloan-Kettering Cancer Center*

Ethical concerns of stem cell therapeutics for Parkinson's Disease

- Lawrence S.B. Goldstein, *Howard Hughes Medical Institute Investigator, Professor of Cellular and Molecular Medicine, and Director, UCSD Stem Cell Program, University of California, San Diego*

3:15 p.m. Break

3:30 p.m. **Session 4: General issues in stem cell therapeutics**

Session Objective: This session will include a panel of stakeholders including representatives from the research, oversight, and patient communities to react to the case studies and discuss cross-cutting issues related to stem cell therapeutics.

Moderator: Susan L. Solomon, *Co-founder and Chief Executive Officer, The New York Stem Cell Foundation*

- Marie Csete, *Chief Scientific Officer, California Institute for Regenerative Medicine*
- Insoo Hyun, *Associate Professor of Bioethics, Case Western Reserve University; Co-Chair, Task Force on Clinical Translation of Stem Cells and Chair, Ethics and Public Policy Committee, International Society for Stem Cell Research*
- Jane S. Lebkowski, *Senior Vice President of Tissue Regenerative Medicine, Geron Corporation*
- Amy Comstock Rick, *Chief Executive Officer, Parkinson's Action Network; President, Coalition for the Advancement of Medical Research*
- Jeremy Sugarman, *Harvey M. Meyerhoff Professor of Bioethics and Medicine, Professor of Medicine, Professor of Health Policy and Management, and Deputy Director for Medicine, Berman Institute of Bioethics, Johns Hopkins University*

5:00 p.m. **Additional discussion and public comment**

Moderator: R. Alta Charo (committee co-chair), *Warren P. Knowles Professor of Law and Bioethics, University of Wisconsin–Madison*

5:30 p.m. Symposium adjourns

The Human Embryonic Stem Cell Research Advisory Committee is a project of the National Research Council's Board on Life Sciences and the Institute of Medicine's Board on Health Sciences Policy. The project is sponsored by The Ellison Medical Foundation, The Greenwall Foundation, and the Howard Hughes Medical Institute.