

**NATIONAL INSTITUTES OF HEALTH
RECOMBINANT DNA ADVISORY COMMITTEE (RAC)**

**Gene Transfer Safety Symposium:
Current Perspectives on Gene Transfer for X-SCID**

**Bethesda Marriott Hotel
Bethesda, Maryland**

**March 15, 2005
Symposium Agenda**

Tuesday, March 15, 2005

8:00 AM **Welcome**
Amy P. Patterson, M.D., Office of Biotechnology Activities, NIH
Diane W. Wara, M.D., University of California, San Francisco

8:10 AM **Keynote Speaker**
Theodore Friedmann, M.D., University of California, San Diego

8:25 AM **Current SCID Gene Transfer Experience**

Moderators: Diane W. Wara, M.D., University of California, San Francisco
Bernard Lo, M.D., University of California, San Francisco

**Approaches to the Patient with X-Linked SCID Who Has Failed
Standard BMT Treatment**

Jennifer M. Puck, M.D., National Human Genome Research Institute, NIH

**Preliminary Results of an NIH Trial of Retroviral IL2RG Gene
Transfer as Salvage Treatment for Older Post-BMT SCID Patients**

Harry L. Malech, M.D., National Institute of Allergy and Infectious
Diseases, NIH

Retroviral Gene Transfer for Treatment of X-SCID and ADA-SCID

Fabio Candotti, M.D., National Human Genome Research Institute, NIH

Summary of Recent Safety Data Emerging from French X-SCID Trial

Theodore Friedmann, M.D., University of California, San Diego

U.S. Food and Drug Administration Perspectives

Carolyn A. Wilson, Ph.D., U.S. Food and Drug Administration

10:30 AM **BREAK**

Tuesday, March 15, 2005 (continued)

10:45 AM **Discussion**

11:30 AM **Retrovirus Integration and Insertional Mutagenesis**

Moderators: Naomi Rosenberg, Ph.D., Tufts University
Marina O'Reilly, Ph.D., Office of Biotechnology Activities, NIH

Gene Transfer and Insertional Mutagenesis

Christof von Kalle, M.D., Cincinnati Children's Hospital Medical Center

Gene Transfer Insertional Mutagenesis Insights

Neal G. Copeland, Ph.D., National Cancer Institute, NIH

Retroviral Integration Target Site Preferences

Frederic D. Bushman, Ph.D., University of Pennsylvania

12:45 PM **Discussion**

1:30 PM **Lunch**

2:30 PM **Bone Marrow Transplantation for SCID**

Moderators: Bernard Lo, M.D., University of California, San Francisco
Theodore Friedmann, M.D., University of California, San Diego
Thomas Holohan, M.D., Office of Biotechnology Activities, NIH

Overview of HLA Identical and Haploidentical BMT: Duke Experience

Rebecca H. Buckley, M.D., Duke University Medical Center

Overview of HLA Identical and Haploidentical BMT: Memorial Sloan-Kettering Experience

Richard J. O'Reilly, M.D., Memorial Sloan-Kettering Cancer Center

Overview of Cord Blood Transplantation

John E. Wagner, M.D., University of Minnesota

4:00 PM **BREAK**

4:15 PM **Discussion**

Tuesday, March 15, 2005 (continued)

5:00 PM **Wrap-Up and Next Steps**

Moderators: Theodore Friedmann, M.D., University of California, San Diego
Diane W. Wara, M.D., University of California, San Francisco
Amy P. Patterson, M.D., Office of Biotechnology Activities, NIH

6:00 PM **Adjournment**