

**DEVELOPMENT OF T-ALL IN TWO SUBJECTS
IN
A GENE TRANSFER CLINICAL TRIAL FOR X-SCID
NIH RECOMBINANT DNA ADVISORY COMMITTEE MEETING
A Gene Transfer Safety Symposium**

Building 31C
Conference Room 6
Bethesda, MD

February 10, 2003

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|----------------------------|---|
| 8:30 AM - 8:45 AM | Welcome and Opening Remarks
Review of Purpose and Objectives
Amy P. Patterson, M.D., Director
NIH Office of Biotechnology Activities

Theodore Friedmann, M.D.
Chair, NIH Recombinant DNA Advisory Committee
University of California, San Diego |
| 8:45 AM – 9:00 AM | Roundtable Introductions |
| 9:00 AM – 10:00 AM | Case Presentation and Molecular Analyses
Alain Fischer, M.D., Ph.D. (teleconference)
Hôpital Necker-Enfants Malades
Paris, France

Christof von Kalle, M.D.
Cincinnati Children's Hospital Research Foundation |
| 10:00 AM – 10:30 AM | Pediatric T-ALL
Gregory Reaman, M.D.
Group Chair, Children's Oncology Group
Executive Director, The Center of Cancer and Blood Disorders,
The Children's National Medical Center |
| 10:30 AM – 10:45 AM | BREAK |
| 10:45 AM – 11:15 AM | Retroviruses and Insertional Mutagenesis
Naomi Rosenberg, Ph.D.
Tufts University |

11:15 AM – 11:45 AM	LMO Genes in Leukemogenesis Peter Aplan, M.D. National Cancer Institute, NIH
11:45 AM- 12:15 PM	Defective Cytokine Signaling in X-linked Severe Combined Immunodeficiency Warren Leonard, M.D. National Heart, Lung and Blood Institute, NIH
12:15 PM – 1:00 PM	LUNCH
1:00 PM – 1:15 PM	FDA Actions in Response to the Second Serious Adverse Event Philip Noguchi, M.D., Acting Director The Office of Cellular, Tissues, and Gene Therapies, CBER, FDA
1:15 PM – 1: 45 PM	Public Questions and Comments
1:45 PM – 3:30 PM	Retroviral-Mediated Gene Transfer into Hematopoietic Cells: Points to Consider (see attached)
3:30 PM – 3:45 AM	BREAK
3:45 PM – 4:15 PM	Public Questions and Comments
4:15 PM – 4:45 PM	RAC Recommendations and Planning for the Retroviral Vector Safety Symposium at the March 2003 RAC meeting Theodore Friedman, Robert Simari and Diane Wara, M.D., Moderators
4:45 PM	Adjournment