

NIH Recombinant DNA Advisory Committee

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

March 15, 2005



Goals

- **Promote scientific understanding and public awareness of the latest research findings regarding insertional mutagenesis and its etiology**
- **Provide comprehensive updates on:**
 - **Current US and international trials studying gene transfer as a possible treatment for SCID**
 - **Latest research into retrovirus integration and insertional mutagenesis**
 - **Use of bone marrow and stem cell transplantation as an alternative treatment for SCID**

Goals (continued)

- **Provide a forum for public discussion of latest clinical experience and scientific findings**
 - Recent emergence of a third case of T-cell proliferation in the French X-SCID trial
- **Revisit NIH RAC 2003 Recommendations**

Background

- **NIH RAC reviewed the clinical and molecular data concerning two serious adverse events that occurred in a human gene transfer study conducted in France to correct X-linked SCID**
 - **December 5, 2002**
 - **February 10, 2003**
- **NIH RAC conducted an analysis of the available data from the France study and other gene transfer trials for SCID**

NIH RAC 2003 Recommendations

- **Pending further data or extenuating circumstances, studies should be reviewed on a case-by-case basis**
 - **Retroviral gene transfer studies for X-linked SCID should be limited to patients who have failed identical or haploidentical stem-cell transplantation or for whom no suitable stem cell donor can be identified**
- **Case-by-case review would include appropriate risk benefit analysis accompanied by implementation of appropriate informed consent and monitoring plans**

NIH RAC 2003 Recommendations

- **There are not sufficient data or reports of adverse events directly attributable to the use of retroviral vectors at this time to warrant cessation of other retroviral human gene transfer studies, including studies for non-X-linked SCID**
- **Such studies may be justified contingent upon appropriate risk benefit analysis accompanied by implementation of appropriate informed consent and monitoring plans**

AAAS Scientific Freedom and Responsibility Award

- **On March 10, the NIH RAC was awarded the Scientific Freedom and Responsibility award from the American Association for the Advancement of Science**
- **Awarded to the NIH RAC in recognition of:**
 - **Principles of transparency, public participation, and open scientific and ethical discourse underpinning the activities of the Committee for the last 30 years**
 - **Advancement of scientific understanding and progress**
- **X-SCID Safety Symposium is exemplary of this open and public process**

AWARDS

Presented at the 171st Annual Meeting
19 February 2005 ▶ Washington, DC



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To

Recombinant DNA Advisory Committee of the National Institutes of Health

For providing outstanding leadership and enabling society to proceed with recombinant DNA research and genetic therapy in a responsible manner thereby enhancing the opportunities presented by modern genetic advances.



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