

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

91st Meeting of the NIH RAC

**Bethesda Marriott Hotel
Bethesda, Maryland**

June 18-19, 2003

MEETING AGENDA

Wednesday, June 18, 2003

8:30 AM **Call to Order and Opening Remarks**
Theodore Friedmann, M.D., Chair, NIH RAC

Tab 2295 For Your Information
- Notice of Meeting
- Conflict of Interest Guidance

8:45 AM **Minutes of the February 10, 2003 RAC Meeting**
Tab 2296

RAC Reviewers: Theodore Friedmann, M.D.
Madison Powers, J.D., D.Phil.

Minutes of the March 6-7, 2003 RAC Meeting
Tab 2297

RAC Reviewers: Baruch Brody, Ph.D.
David Sidransky, M.D.

9:00 AM **Presentation of NIH Award of Merit to Dr. Theodore Friedmann and Dr. Linda Gooding**

9:30 AM **Discussion of Human Gene Transfer Protocol #0301-575 entitled: *A Phase I Study of NT-501, An Implant of Encapsulated Human NTC-201 Cells Releasing Ciliary Neurotrophic Factor (CNTF), in Patients With Retinitis Pigmentosa***

PI: Paul A. Sieving, M.D., Ph.D., National Institutes of Health

RAC Reviewers: Neal DeLuca, Ph.D.
Larry Johnson, M.D.
Maxine Linial, Ph.D.
Bernard Lo, M.D.

Ad Hoc Reviewer: Jean Bennett, M.D., Ph.D.

Wednesday, June 18, 2003 (continued)

Tab 2298 Protocol

Tab 2299 OBA Summary
OBA Letter to PI on in-depth RAC Review and Public Discussion
Outcome of Initial Review by RAC Members
Reviews from Drs. DeLuca, L. Johnson, Linial, Lo, and Bennett
PI's/Sponsor's Response

11:15 AM **Public Comment**
The Foundation Fighting Blindness

11:40 AM **BREAK**

11:55 AM **Data Management Report**
Tab 2300 Protocol List
Protocols Not Selected for RAC Public Review
Discussion of Amendments to Protocol 304

RAC Reviewers: Baruch Brody, Ph.D.
Linda Gooding, Ph.D.
Larry Johnson, M.D.
Robert Simari, M.D.
Diane Wara, M.D.

1:00 PM **LUNCH**

2:00 PM **Review of Selected American Society of Gene Therapy (ASGT) Annual Meeting Sessions Related to Retroviral Vectors**
Theodore Friedmann, M.D., Chair, NIH RAC

Tab 2301 "Ethical and Policy Dilemmas in Clinical Gene Therapy Studies:
Balancing Real Benefits and Real Risks"
"Report from the ASGT *Ad Hoc* Committee on Retroviral-mediated Gene Transfer to Hematopoietic Stem Cells"
"SCID-X1 Clinical Trial: Current State"

Recommendations of the United Kingdom Gene Therapy Advisory Committee (GTAC) and Committee on Safety of Medicines (CSM) Working Party on Retroviruses (April 2003)
Marina O'Reilly, Ph.D., Biotechnology Program Advisor, OBA

Tab 2302

3:00 PM **Public Comment**

3:10 PM **BREAK**

Wednesday, June 18, 2003 (continued)

3:25 PM **Retroviral Vectors: Topics for Future Presentations and Discussions at RAC Meetings**

Marina O'Reilly, Ph.D., Alexander Rakowsky, M.D., and Madison Powers, J.D., D.Phil.

Retrovirus Working Group Members: Martha Bohn, Ph.D.
Neal DeLuca, Ph.D.
Theodore Friedmann, M.D.
Larry Johnson, M.D.
Maxine Linial, Ph.D.
Robert Simari, M.D.
Diane Wara, M.D.

3:45 PM **Public Comment**

3:55 PM **Presentation of in-depth Assessment Regarding Containment-Level Requirements for Modified Vaccinia Ankara (MVA) Pox Viral Vector**

RAC Presenter: Emmett Barkley, Ph.D.

Tab 2303

Assessment Team: Mark Feinberg, M.D., Ph.D., Emory University
Bernard Moss, M.D., Ph.D., National Institute of Allergy and Infectious Diseases, NIH
Paul Spearman, M.D., Vanderbilt University

4:45 PM **Public Comment**

5:00 PM **ADJOURNMENT**

Thursday, June 19, 2003

8:30 AM **Call to Order and Opening Remarks**

Theodore Friedmann, M.D., Chair, NIH RAC

8:35 AM **Informed Consent**

What Consent Forms Say and What Researchers and Subjects Expect in Gene Transfer Research

Nancy M.P. King, J.D., and Gail Henderson, Ph.D., University of North Carolina, Chapel Hill

9:35 AM **Discussion**

Thursday, June 19, 2003 (continued)

9:50 AM **BREAK**

10:05 AM **NIH RAC Informed Consent Working Group (ICWG) Draft Guidance**
Nancy M.P. King, J.D., and Baruch Brody, Ph.D., Cochairs, ICWG

Tab 2304

RAC ICWG Members: James Childress, Ph.D.
Bernard Lo, M.D.
Diane Wara, M.D.

U.S. Department of Health and Human Services (DHHS) ICWG Members:

Suzanne Goodwin, OBA
Allan Shipp, OBA
Stephen Rose, Ph.D., OBA
Kristina Borrer, Ph.D., Office for Human Research
Protections, DHHS
Cynthia Rask, M.D., U.S. Food and Drug
Administration

10:35 AM **Discussion**

11:35 AM **Public Comment**

12:00 NOON **ADJOURNMENT**