

**National Institutes of Health
Recombinant DNA Advisory Committee (RAC)
Bethesda Marriott Hotel
Bethesda, Maryland
March 7-8, 2002**

Meeting Agenda*

Thursday, March 7, 2002

8:00 AM **Call to Order and Opening Remarks**
Theodore Friedmann, M.D., RAC Chair

Tab 2238 For Your Information
 -Notice of Meeting
 -Conflict of Interest Statement

8:10 AM **OBA Updates**

- Roundtable Introduction of New RAC Members
- Final Action on Severe Adverse Events Reporting
- Clinical Gene Transfer Database (GeMCRIS)
- IBCs in a Changing Research Landscape: A Policy Conference: Debriefing

Information Contained in Table Folder

8:55 AM **Minutes of the December 6, 2001, RAC Meeting**

RAC Reviewer: Sue Levi-Pearl, RAC Member

Tab 2239 Minutes of the December 6, 2001 meeting

9:00 AM **Discussion of Human Gene Transfer Protocol #0201-513 entitled:** Phase I study of intravenous DOTAP:Cholesterol-Fus1 liposome complex (DOTAP:Chol-fus1) in patients with advanced non-small cell lung cancer (NSCLC) previously treated with chemotherapy

PI: Charles Lu, M.D., The University of Texas, MD Anderson Cancer Center

RAC Reviewers: Nancy King, J.D.
 Larry Johnson, M.D.
 Xandra Breakefield, Ph.D.

Tab 2240 Protocol

Tab 2241 OBA Summary
OBA Letter to PI on RAC Public Review
RAC Members Recommendation
Reviews from King, L. Johnson, Breakefield
Sponsor's Response

10:30 AM **BREAK**

10:45 AM **Informed Consent Issues of Human Gene Transfer Research**

Nancy King, J.D., RAC Member
Baruch Brody, Ph.D., RAC Member

Tab 2242 – “Defining and Describing Benefit Appropriately in Clinical
Trials” by Nancy M.P. King
– “Making Informed Consent Meaningful” by Baruch A.
Brody
– Appendix M3: Informed Consent

12:00 PM **Lunch**

1:15 PM **Detection of Adeno-Associated Virus Vector Sequence in Research
Participant Semen: Follow-Up**

Mark Kay, M.D., Ph.D., Stanford University
Kathy High, M.D., Children's Hospital of Philadelphia

RAC Discussants:

Philip Johnson, Jr., M.D., RAC Member
Xandra Breakefield, Ph.D., RAC Member
Nancy King, J.D., RAC Member
James Childress, Ph.D., RAC Member

Tab 2243 Summary of Highlights of Discussion
Transcript, December 2001 RAC Meeting
Issue Brief, December 2001 RAC Meeting

3:00 PM **Break**

3:15 PM **Discussion of Human Gene Transfer Protocol #0201-514 entitled:
Transduction of the upper and lower airway epithelium in healthy subjects by an
AAV2 vector that encodes human placental alkaline phosphatase**

PI: Moira L. Aitken, M.D., Fred Hutchinson Cancer Research
Center, University of Washington

A. Dusty Miller, Ph.D., Fred Hutchinson Cancer Research Center, University of Washington

RAC Reviewers: Larry Johnson, M.D.
Linda Gooding, Ph.D.
Baruch Brody, Ph.D.

Tab 2244 Protocol

Tab 2245 OBA Summary
OBA Letter to PI on RAC Public Review
RAC Members Recommendation
Reviews from L. Johnson, Gooding, Brody
Sponsor's Response

4:30 PM Adjournment

Friday, March 8, 2002

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

8:35 AM **Opening Remarks**
Ruth Kirschstein, M.D., Acting Director, National Institutes of Health

8:40 AM **Swearing In of New RAC Members**
Ruth Kirschstein, M.D.

8:50 AM **Discussion of Human Gene Transfer Protocol #0112-512 entitled: Phase I study of human growth hormone releasing hormone expressed by a plasmid DNA myogenic vector in patients with cancer cachexia**

PI: Malcolm K. Brenner, M.D., Ph.D., Baylor College of Medicine

Uday Popat, M.D., Baylor College of Medicine

RAC Reviewers: Nancy King, J.D.
Robert Simari, M.D.
Philip Johnson, M.D.

Ad Hoc Consultants: Thomas Gelehrter, M.D., University of Michigan
Thomas Ziegler, M.D., Emory University
Richard Vile, Ph.D., Mayo Clinic

Tab 2246 Protocol

Tab 2247 OBA Summary
OBA Letter to PI on RAC Public Review
RAC Members Recommendation
Reviews from Simari, P. Johnson, King, Gelehrter, Ziegler,
and Vile
Sponsor's Response

9:50 AM **Break**

10:05 AM **Continuing Discussion of Human Gene Transfer Protocol #0112-512**

11:00 AM **Data Management**

Tab 2248 Protocol List
Protocols Not Requiring Full RAC Review
Serious Adverse Event Summary Report
Amendments and Update Summary Table
Responses to the Requirements of Appendix M-1-C-1
Correspondence Regarding Protocol #0201-513

12:00 AM **Lunch**

1:30 PM **Discussion of Human Gene Transfer Protocol #0201-515 entitled:**
Gene therapy of progressive glioblastoma multiforme using a replication defective
HSV multigene vector NUREL-C2: a phase I clinical trial to determine the
maximum tolerable dose of vector in combination with ganciclovir and
radiosurgery

PI: L. Dade Lunsford, M.D., University of Pittsburgh School of
Medicine

Joseph C. Glorioso, Ph.D., University of Pittsburgh School
of Medicine

RAC Reviewers: Xandra Breakefield, Ph.D.

Diane Wara, M.D.

Maxine Linial, Ph.D.

James Childress, Ph.D

Ad Hoc Consultant: Richard Vile, Ph.D., Mayo Clinic

Tab 2249 Protocol

Tab 2250 OBA Summary
OBA Letter
RAC Member Recommendation

Reviews from Linial, Wara, Childress, Breakefield, and

Vile
Sponsor's Response

2:50 PM **Closing Remarks**

3:00 PM **Adjournment**

* Meeting agenda as of 2/19/02. Please check the OBA Website for updates. Times are approximate and subject to change.