

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

**Bethesda Marriott Hotel
Bethesda, Maryland**

December 4-6, 2002

MEETING AGENDA

Wednesday, December 4, 2002

- 1:00 PM **Call to Order and Opening Remarks**
Theodore Friedmann, M.D., RAC Chair
- Tab 2277 For Your Information
 - Notice of Meeting
 - Conflict of Interest Guidance
- 1:15 PM **Minutes of the September 19-20, 2002, Meeting**
- RAC Reviewers: W. Emmett Barkley, Ph.D.
 Terry Kwan, M.S.Ed.
- Tab 2278 Minutes of the September 19-20, 2002, Meeting
- 1:25 PM **Data Management Report**
- Tab 2279 Protocol List
 Protocols Not Selected for RAC Public Review
- RAC Reviewers: Robert Simari, M.D.
 Diane Wara, M.D.
- 1:40 PM **An Adverse Event in a Gene Transfer Clinical Trial for X-Linked Severe Combined
Immunodeficiency Disease (SCID)**
- 1:40 PM **Case Presentation and Followup**
Christof von Kalle, M.D., Cincinnati Children's Hospital Research Foundation
- 2:00 PM **Questions and Answers**
- 2:10 PM **Review of FDA Biological Response Modifiers Advisory Committee Meeting**
Stephanie L. Simek, Ph.D., U.S. Food and Drug Administration
- 2:30 PM **Review of October 29, 2002, RAC Planning Session**
Stephen Rose, Ph.D., and/or Theodore Friedmann, M.D.

Wednesday, December 4, 2002 (continued)

2:50 PM **Adverse Events in Clinical Trials Using Retroviral Vectors**
Alexander Rakowsky, M.D., Office of Biotechnology Activities, NIH

3:10 PM **BREAK**

3:25 PM **Monitoring Research Participants in Clinical Trials Using Retroviral Vectors**
Theodore Friedmann, M.D., and Diane Wara, M.D., Moderators

Fabio Candotti, M.D., National Human Genome Research Institute (NHGRI), NIH
Dale Hammerschmidt, M.D., University of Minnesota
Harry Malech, M.D., National Institute of Allergy and Infectious Diseases (NIAID), NIH
David Sidransky, M.D., RAC Member
Robert Simari, M.D., RAC Member
Christof von Kalle, M.D., Cincinnati Children's Hospital Research Foundation
Kenneth Weinberg, M.D., University of Southern California (USC) School of Medicine/Children's Hospital of Los Angeles

4:55 PM **Public Comment**

5:15 PM **ADJOURNMENT**

Thursday, December 5, 2002

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

8:35 AM **An Adverse Event in a Gene Transfer Clinical Trial for X-Linked SCID**

8:35 AM **Informed Consents for Clinical Trials Using Retroviral Vectors**
James Childress, Ph.D., and Madison Powers, J.D., D.Phil., Moderators

Fabio Candotti, M.D., NHGRI, NIH
Dale Hammerschmidt, M.D., University of Minnesota
Harry Malech, M.D., NIAID, NIH
Jennifer Puck, M.D., NHGRI, NIH
Christof von Kalle, M.D., Cincinnati Children's Hospital Research Foundation
Diane Wara, M.D., RAC Member
Kenneth Weinberg, M.D., USC School of Medicine/Children's Hospital of Los Angeles

10:00 AM **BREAK**

10:15 AM **Informed Consents for Clinical Trials Using Retroviral Vectors**
(continued)

Thursday, December 5, 2002 (continued)

10:15 AM **Informed Consents for Clinical Trials Using Retroviral Vectors
(continued)**

11:00 AM **Summary of RAC Recommendations and Next Steps**

Monitoring

Theodore Friedmann, M.D., and Diane Wara, M.D., Moderators

Informed Consent

James Childress, Ph.D., and Madison Powers, J.D., D.Phil., Moderators

12:00 NOON **LUNCH**

1:00 PM **Discussion of Human Gene Transfer Protocol #0210-556 entitled:**
*A Phase I, Open-Label, Dose-Escalation Trial Evaluating the Safety and Immunogenicity of
Sequential Administration of Recombinant DNA and Adenovirus Expressing L523S Protein in
Patients With Early-Stage Non-Small Cell Lung Cancer (NSCLC)*

PI: John J. Nemunaitis, M.D., US Oncology, Dallas, Texas

RAC Reviewers: James Childress, Ph.D.
Neal DeLuca, Ph.D.
David DeMets, Ph. D.
Larry Johnson, M.D.

Tab 2280 Protocol

Tab 2281 OBA Summary
OBA Letter to PI on in-depth RAC Review and Public Discussion
Outcome of Initial Review by RAC Members
Reviews From Drs. Childress, DeLuca, DeMets, and L. Johnson
PI's / Sponsor's Response

2:45 PM **BREAK**

3:00 PM **Discussion of Human Gene Transfer Protocol #0210-557 entitled:**
*A Double-Blind, Placebo-Controlled, Dose-Escalation Pilot Study To Assess the Safety and
Effects of AMG0001 in Patients With Ischemic Heart Disease (IHD) Not Amenable to
Coronary Artery Bypass Graft (CABG) or Percutaneous Coronary Intervention (PCI)*

PIs: Michael Simons, M.D., Dartmouth Medical School
Brian H. Annex, M.D., Duke University School of Medicine

RAC Reviewers: Bernard Lo, M.D.
David Sidransky, M.D.
Robert Simari, M.D.

Thursday, December 5, 2002 (continued)

Tab 2282 Protocol

Tab 2283 OBA Summary
OBA Letter to PIs on in-depth RAC Review and Public Discussion
Outcome of Initial Review by RAC Members
Reviews From Drs. Lo, Sidransky, and Simari
PIs' / Sponsor's Response

4:30 PM **ADJOURNMENT**

Friday, December 6, 2002

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

8:35 AM **Report From NIH RAC Informed Consent Working Group**
Nancy King, J.D., or James Childress, Ph.D., or Bernard Lo, M.D.

8:45 AM **Discussion of Human Gene Transfer Protocol #0208-550 entitled:**
*A Phase I/II Study of an Antitumor Vaccination Using $\alpha(1,3)$ Galactosyltransferase
Expressing Allogeneic Tumor Cells in Patients With Relapsed or Refractory Breast Cancer*

PI: Roscoe F. Morton, M.D., Stoddard Cancer Research Institute, Iowa Methodist
Medical Center

Discussion of Human Gene Transfer Protocol #0210-552 entitled:
*A Phase I/II Study of an Antitumor Vaccination Using $\alpha(1,3)$ Galactosyltransferase
Expressing Allogeneic Tumor Cells in Patients With Refractory or Recurrent NSCLC*

PI: John C. Morris, M.D., National Cancer Institute, NIH

RAC Reviewers: Linda Gooding, Ph.D.
Madison Powers, J.D., D.Phil.
Diane Wara, M.D.

Ad Hoc Reviewer: John Iacomini, Ph.D., Massachusetts General Hospital
(via teleconference)

Tab 2284 Protocol

Friday, December 6, 2002 (continued)

Tab 2285 OBA Summary
OBA Letter to PI on in-depth RAC Review and Public Discussion
Outcome of Initial Review by RAC Members
Reviews From Drs. Gooding, Powers, Wara, and Iacomini
PI's / Sponsor's Response

10:45 AM

ADJOURNMENT