

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

100th Meeting

**Bethesda Marriott Hotel
Bethesda, Maryland**

**June 15-16, 2005
Meeting Agenda**

Wednesday, June 15, 2005

8:30 AM Call to Order and Opening Remarks

Diane Wara, M.D., Chair, NIH RAC

Tab 2373 For Your Information
Notice of Meeting
Conflict of Interest Guidance

8:40 AM Minutes of the March 16, 2005, RAC Meeting

RAC Reviewers: Nicholas Muzyczka, Ph.D.
Emmett Barkley, Ph.D.

Tab 2374 Minutes of the March 16, 2005, RAC Meeting

8:45 AM Certificates of Appreciation

Presenter: Raynard S. Kington, M.D., Ph.D., Deputy Director, National
Institutes of Health

**8:55 AM Followup from March 15, 2005, Gene Transfer Safety Symposium:
Current Perspectives on Gene Transfer for X-SCID**

Presenters: Diane Wara, M.D.
Marina O'Reilly, Ph.D., Office of Biotechnology Activities

**9:20 AM Discussion of Human Gene Transfer Protocol #0311-613: *A Phase I Dose-
Escalation Study of Intratumoral Herpes Simplex Virus-1 Mutant rRp450 in
Patients with Refractory Sarcoma or Neuroblastoma***

PI: Timothy P. Cripe, M.D., Ph.D., Cincinnati Children's Hospital
Medical Center, Cincinnati, OH

RAC Reviewers: Neal DeLuca, Ph.D.
Terry Kwan, M.S.Ed.
Thomas Gelehrter, M.D.

Wednesday, June 15, 2005 (continued)

Tab 2375 Protocol

Tab 2376 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 and Gelehrter and Ms. Kwan
PI's/Sponsor's Response

10:50 AM **Public Comment**

11:00 AM **BREAK**

11:15 AM **Discussion of Human Gene Transfer Protocol #0502-699: *A Pilot Study of Temozolomide and O⁶-Benzylguanine for Treatment of High-Grade Glioma Using Autologous Peripheral Blood Stem Cells Genetically Modified for Chemoprotection***

PI: Lars Martin Wagner, M.D., Cincinnati Children's Hospital
Medical Center/University of Cincinnati College of Medicine,
Cincinnati, OH

Submitter: Christof von Kalle, M.D., Cincinnati Children's Hospital Medical
Center/University of Cincinnati College of Medicine, Cincinnati,
OH

RAC Reviewers: Bernard Lo, M.D.
Glen Nemerow, Ph.D.
Naomi Rosenberg, Ph.D.

Tab 2377 Protocol

Tab 2378 OBA Summary
OBA Letter to PI on In-Depth RAC Review and
Public Discussion
Outcome of Initial Review by RAC Members
Reviews from Drs. Lo, Nemerow, and Rosenberg
PI's/Sponsor's Response

12:45 PM **Public Comment**

12:55 PM **LUNCH**

Wednesday, June 15, 2005 (continued)

1:55 PM **Data Management Report**

Tab 2379 Response M-1-C-1
 Protocol List
 Protocols Not Selected for RAC Public Review

RAC Reviewers: Steven Albelda, M.D.
 Helen Heslop, M.D.
 Robert Simari, M.D.
 Diane Wara, M.D.

2:15 PM **Discussion of Human Gene Transfer Protocol #0504-703: *A Phase I Safety, Dose-Escalating Study of MultiGeneAngio in Patients with Peripheral Arterial Disease***

PI: P. Michael Grossman, M.D., University of Michigan/
 VA Ann Arbor Health Systems, Ann Arbor, MI

Sponsor: Moshe Flugelman, M.D., Multigene Vascular Systems, Ltd., Haifa,
 Israel

RAC Reviewers: Stephen Dewhurst, Ph.D.
 Madison Powers, J.D., D.Phil.
 Robert Simari, M.D.

Ad Hoc Reviewer: Stephen Epstein, M.D., Washington Hospital Center,
 Washington, DC

Tab 2380 Protocol

Tab 2381 OBA Summary

OBA Letter to PI on In-Depth RAC Review and
Public Discussion
Outcome of Initial Review by RAC Members
Reviews from Drs. Dewhurst, Powers, Simari, and Epstein
PI's/Sponsor's Response

3:45 PM **Public Comment**

4:00 PM **ADJOURNMENT**

Thursday, June 16, 2005

8:00 AM **Call to Order and Opening Remarks**

Diane Wara, M.D., Chair, NIH RAC

8:10 AM **Discussion of Human Gene Transfer Protocol #0410-677: *Phase I Trial of Ocular Subretinal Injection of a Recombinant Adeno-Associated Virus (rAAV-RPE65) Gene Vector in Patients with Retinal Disease Due to RPE65 Mutations***

PI: Samuel G. Jacobson, M.D., Ph.D., Scheie Eye Institute, University of Pennsylvania School of Medicine, Philadelphia, PA

Submitter: Philip J. Cross & Associates, Inc., Wilmington, DE

RAC Reviewers: Terry Kwan, M.S.Ed.
Helen Heslop, M.D.
Richard Vile, Ph.D.

Ad Hoc Reviewer: Karl Csaky, M.D., Ph.D., National Eye Institute, National Institutes of Health

Tab 2382 Protocol

Tab 2383 OBA Summary
OBA Letter to PI on In-Depth RAC Review and Public Discussion
Outcome of Initial Review by RAC members
Reviews from Drs. Heslop, Vile, and Csaky and Ms. Kwan
PI's/Sponsor's Response

9:50 AM **Public Comment**

10:00 PM **BREAK**

10:15 AM **Biological Containment for Work with Noncontemporary Strains of Influenza: Harmonization of the *NIH Guidelines* with Other Federal Guidances**

Introduction: Kathryn Harris, Ph.D., Contractor, Office of Biotechnology Activities

Presenter: Jacqueline Katz, Ph.D., U.S. Centers for Disease Control and Prevention, Atlanta, GA

Thursday, June 16, 2005 (continued)

11:00 AM **Discussion of Human Gene Transfer Protocol #0504-707: *Dose-Finding and Safety Study of an Oncolytic Polio/Rhinovirus against Malignant Glioma***

PI: Darrell D. Bigner, M.D., Ph.D., Duke University Medical Center,
Durham, NC

Submitter: Matthias Gromeier, M.D., Duke University, Durham, NC

RAC Reviewers: Neal DeLuca, Ph.D.
Philip Johnson, M.D. (*written review*)
Bernard Lo, M.D.
Diane Wara, M.D.

Ad Hoc Reviewer: Ellie Ehrenfeld, Ph.D., National Institute of Allergy and
Infectious Diseases, National Institutes of Health

Tab 2384 Protocol

Tab 2385 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, Johnson, Lo, Wara, and Ehrenfeld
PI's/Sponsor's Response

12:30 PM **Public Comment**

12:45 PM **ADJOURNMENT**