

**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.

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 Temozolamide 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:

Christopher 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

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| 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. |
| <i>Ad Hoc</i> 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, Altanta, 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**