

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
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: 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

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**