

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

104th Meeting

**NIH Campus
Building 31, Sixth Floor, C Wing, Room 6**

**June 21, 2006
Agenda**

Wednesday, June 21, 2006

8:00 AM Call to Order and Opening Remarks

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

Tab 2413 For Your Information
Notice of Meeting
Conflict of Interest Guidance

8:05 AM Certificates of Appreciation for RAC Member Service to NIH

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

8:20 AM Minutes of the March 15, 2006, RAC Meeting

RAC Reviewers: Nikunj Somia, Ph.D.
David Weber, M.D., M.P.H.

Tab 2414 Minutes of the March 15, 2006, RAC Meeting

**8:30 AM Discussion of Human Gene Transfer Protocol #0604-769: *A Phase I
Randomized, Placebo-Controlled, Open-Label, Cross-Over Safety and
Pharmacodynamic Study of BHT-3021 in Subjects with Recent Onset Type 1
Diabetes Mellitus***

PI: Peter A. Gottlieb, M.D., University of Colorado Health Sciences Center,
Aurora, CO

Submitter: Patricia Murphy, Vice President, Regulatory Affairs, Bayhill
Therapeutics, Inc., Palo Alto, CA

RAC Reviewers: Stephen Dewhurst, Ph.D.
Helen Heslop, M.D. (*written review*)
Robyn Shapiro, J.D.

Wednesday, June 21, 2006 (continued)

Tab 2415 Protocol

Tab 2416 OBA Summary
OBA Letter to PI on In-Depth RAC Review and
Public Discussion
Reviews from Drs. Dewhurst and Heslop and Ms. Shapiro
PI's/Sponsor's Response

10:15 AM **Public Comment**

10:30 AM **Discussion of Human Gene Transfer Protocol #0604-774: *A Phase I Multicenter Study Evaluating the Safety and Potential Activity of Three Escalating Doses of hMaxi-K Gene Transfer in Female Participants with Overactive Bladder Syndrome and Detrusor Overactivity: Double-Blind, Imbalanced Placebo-Controlled Design Within Three Sequential Active Treatment Groups***

PI: Andrew McCullough, M.D., New York University School of Medicine,
New York, NY

Submitter: Arnold Melman, M.D., Albert Einstein College of Medicine,
New York, NY

RAC Reviewers: Terry Kwan, M.S.Ed.
Howard Federoff, M.D., Ph.D.
Glen Nemerow, Ph.D.

Ad hoc Reviewer: Debuene Chang, M.D., National Institute of Diabetes and
Digestive and Kidney Diseases, National Institutes of
Health, Bethesda, MD

Tab 2417 Protocol

Tab 2418 OBA Summary
OBA Letter to PI on In-Depth RAC Review and
Public Discussion
Reviews from Drs. Federoff, Nemerow, and Chang and Ms. Kwan
PI's/Sponsor's Response

11:45 AM **Public Comment**

Wednesday, June 21, 2006 (continued)

12:00 NOON Gene Transfer Safety Assessment Board Report

Tab 2419 Response to M-I-C-1
Protocol List
Protocols Not Selected for RAC Public Review

RAC Reviewers: Steven Albelda, M.D.
Howard Federoff, M.D., Ph.D.
Helen Heslop, M.D.
Diane Wara, M.D.

12:30 PM WORKING LUNCH

Biosafety Considerations for Research Involving Lentiviral Vectors

Presenter: Stephen Dewhurst, Ph.D., RAC Member

1:30 PM Discussion of Human Gene Transfer Protocol #0602-758: *Lentiviral-Mediated, Hematopoietic-Directed Gene Therapy for MPS VII*

PI: Mark S. Sands, Ph.D., Washington University School of Medicine, St. Louis, MO

Submitter: Mark S. Sands, Ph.D., Washington University School of Medicine, St. Louis, MO

RAC Reviewers: Howard Federoff, M.D., Ph.D.
Robyn Shapiro, J.D.
Nikunj Somia, Ph.D.

Ad hoc Reviewer: Hugo Moser, M.D., Johns Hopkins University, Baltimore, MD

Tab 2420 Protocol

Tab 2421 OBA Summary
OBA Letter to PI on In-Depth RAC Review and
Public Discussion
Reviews from Drs. Federoff, Somia, and Moser and Ms. Shapiro
PI's/Sponsor's Response

3:15 PM Public Comment

Wednesday, June 21, 2006 (continued)

3:30 PM **Discussion of Human Gene Transfer Protocol #0604-767:** *ADV/RSV-TK Followed by Valganciclovir for Treatment of Patients with Retinoblastoma Complicated by Vitreous Seeds*

Discussion of Human Gene Transfer Protocol #0604-768: *Pediatric Phase I Study of ADV/RSV-TK Followed by Valganciclovir for Treatment of Patients with Retinoblastoma*

PI: Richard L. Hurwitz, M.D., Baylor College of Medicine, Houston, TX
(Dr. Hurwitz is the PI for Protocols # 0604-767 and 0604-768.)

Submitter: Richard L. Hurwitz, M.D., Baylor College of Medicine, Houston, TX

RAC Reviewers: Madison Powers, J.D., D.Phil. (*via teleconference*)
Richard Vile, Ph.D.
Diane Wara, M.D.

Ad hoc Reviewers: David H. Abramson, M.D., Memorial Sloan-Kettering Cancer Center, New York, NY (*via teleconference*)

Karl Csaky, M.D., Ph.D., National Eye Institute, National Institutes of Health, Bethesda, MD (*no written review*)

Tab 2422 Protocol

Tab 2423 OBA Summary
OBA Letter to PI on In-Depth RAC Review and
Public Discussion
Reviews from Drs. Powers, Vile, Wara, and Abramson
PI's/Sponsor's Response

4:45 PM **Public Comment**

5:00 PM **ADJOURNMENT**