

NATIONAL INSTITUTES OF HEALTH  
RECOMBINANT DNA ADVISORY COMMITTEE (RAC)

96<sup>th</sup> Meeting

Bethesda Marriott Hotel  
Bethesda, Maryland

June 8-9, 2004  
Meeting Agenda

---

*Tuesday, June 8, 2004*

8:30 AM     **Call to Order and Opening Remarks**  
Diane Wara, M.D., Chair, NIH RAC

Tab 2339     For Your Information  
                  Notice of Meeting  
                  Conflict of Interest Guidance

8:35 AM     **Minutes of the March 9-11, 2004, RAC Meeting**

RAC Reviewers:     Maxine Linial, Ph.D.  
                          David DeMets, Ph.D.

Tab 2340     Minutes of the March 9-11, 2004, Meeting

8:45 AM     **Update on Protocol 0107-488: *A Phase I, Open-Label Clinical Trial of the Safety and Tolerability of Single Escalating Doses of Autologous CD4 T Cells Transduced with VRX496 in HIV-Positive Subjects*** (In-depth review and public discussion at the September 2001 RAC meeting)

Presenter:     Boro Dropulic, Ph.D.  
                          VIRxSYS Corporation, Gaithersburg, MD

9:45 AM     **Discussion of Human Gene Transfer Protocol #0404-642: *A Phase I Trial of Gene Transfer during VAD Support with SERCA2a***

PI:     Barry London, M.D., Ph.D.  
          University of Pittsburgh Medical Center

RAC Reviewers:     Larry Johnson, M.D.  
                          Bernard Lo, M.D.  
                          Robert Simari, M.D.

Tab 2341     Protocol

**Tuesday, June 8, 2004 (continued)**

Tab 2342      OBA Summary  
OBA Letter to PI on In-Depth RAC Review and  
Public Discussion  
Outcome of Initial Review by RAC Members  
Reviews from Drs. L. Johnson, Powers, and Simari  
PI's/Sponsor's Response

11:15 AM      **Public Comment**

11:40 AM      **Synopsis of RAC Discussion and RAC Recommendations**

11:50 AM      **Introduction to Upcoming Safety Symposium: Safety Considerations in  
Recombinant DNA Research with Highly Pathogenic Viruses**

Speakers:      Kanta Subbarao, M.D., M.P.H.  
Laboratory of Infectious Diseases, National Institute of Allergy  
and Infectious Diseases, NIH

Christina Cassetti, Ph.D.  
Division of Microbiology and Infectious Diseases, National  
Institute of Allergy and Infectious Diseases, NIH

12:30 PM      **LUNCH**

1:00 PM      **Discussion of Human Gene Transfer Protocol #0403-633: *Phase I Trial of  
Immunotherapy with BHT-3009 Alone or Combined with Atorvastatin in Patients  
with Multiple Sclerosis***

PI:      Timothy L. Vollmer, M.D.  
Barrow Neurological Institute, St. Joseph's Hospital and Medical Center

RAC Reviewers:      Martha Bohn, Ph.D.  
Neal DeLuca, Ph.D.  
Madison Powers, J.D., D.Phil.  
Nicholas Muzyczka, Ph.D.

*Ad Hoc* Reviewer:      Stephen D. Miller, Ph.D.  
Northwestern University  
(written response)

Tab 2343      Protocol

**Tuesday, June 8, 2004 (continued)**

Tab 2344      OBA Summary  
OBA Letter to PI on In-Depth RAC Review and  
Public Discussion  
Outcome of Initial Review by RAC Members  
Reviews from Drs. Bohn, DeLuca, Powers, and Muzyczka  
PI's/Sponsor's Response

2:30 PM      **Public Comment**

2:55 PM      **Synopsis of RAC Discussion and RAC Recommendations**

3:05 PM      **Discussion of Human Gene Transfer Protocol #0404-641: A Phase I, Open-Label, Nonrandomized, Dose-Escalation, Multicenter Therapeutic Trial of the Safety, Immunogenicity and Efficacy of GI-4000, an Inactivated Recombinant *Saccharomyces cerevisiae* Immunotherapeutic Expressing Three Different Mutations of the Ras Oncoprotein, in Patients with Solid Tumors Expressing Mutations in Ras**

PI:      Bert O'Neil, M.D.  
University of North Carolina School of Medicine, Chapel Hill, NC

Timothy C. Rodell, M.D.  
GlobeImmune, Inc.

RAC Reviewers:      Emmett Barkley, Ph.D.  
Neal DeLuca, Ph.D.  
Terry Kwan, M.S.Ed.

Tab 2345      Protocol

Tab 2346      OBA Summary  
OBA Letter to PI on In-Depth RAC Review and  
Public Discussion  
Outcome of Initial Review by RAC Members  
Reviews from Drs. Barkley and DeLuca and Ms. Kwan  
PI's/Sponsor's Response

4:35 PM      **Public Comment**

5:05 PM      **Synopsis of RAC Discussion and RAC Recommendations**

5:15 PM      **Update on the RAC Gene Transfer Clinical Trial Design Working Group**

**Tuesday, June 8, 2004 (continued)**

Presenters: David DeMets, Ph.D.  
NIH RAC

Bernard Lo, M.D.  
NIH RAC

Cheryl McDonald, M.D.  
NIH Office of Biotechnology Activities

5:25 PM **ADJOURNMENT**

**Wednesday, June 9, 2004**

9:00 AM **Call to Order and Opening Remarks**  
Diane Wara, M.D., Chair, NIH RAC

9:05 AM **Presentation of NIH Award of Merit to Dr. Larry Johnson, Dr. James Childress, Dr. David Sidransky, and Dr. Maxine Linial**

9:35 AM **Data Management Report**

Tab 2347 Protocol List  
Protocols Not Selected for RAC Public Review

RAC Reviewers: Larry Johnson, M.D.  
Robert Simari, M.D.  
Diane Wara, M.D.

9:55 AM **Discussion of Human Gene Transfer Protocol #0404-638: *Phase I Trial of Intramuscular Injection of a Recombinant Adeno-Associated Virus 1-Alpha 1-Antitrypsin (rAAV1-CB-hAAT) Gene Vector to AAT-Deficient Adults***

PI: Terence R. Flotte, M.D.  
University of Florida

RAC Reviewers: James Childress, Ph.D.  
Thomas Gelehrter, M.D.  
Larry Johnson, M.D.

Tab 2438 Protocol

**Wednesday, June 9, 2004 (continued)**

Tab 2349      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, Gelehrter, and Johnson  
                  PI's/Sponsor's Response

11:35 AM      **Public Comment**

12:00 PM      **Synopsis of RAC Discussion and RAC Recommendations**

12:10 PM      **LUNCH**

1:15 PM      **Discussion of Human Gene Transfer Protocol #0404-643: A Phase I/II Dose-  
Escalation Trial of Intravesical CG0070 for Superficial Transitional Cell  
Carcinoma of the Bladder after Bacillus Calmette-Guerin Failure**

PI:      John Nemunaitis, M.D.  
            US Oncology, Inc., Dallas, TX

RAC Reviewers:      Helen Heslop, M.D.  
                            Bernard Lo, M.D.  
                            David Sidransky, M.D.  
                            Glen Nemerow, Ph.D.

Tab 2350      Protocol

Tab 2351      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, Lo, Sidransky, and Nemerow  
                  PI's/Sponsor's Response

2:45 PM      **Public Comment**

3:10 PM      **Synopsis of RAC Discussion and RAC Recommendations**

3:20 PM      **ADJOURNMENT**