

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

**114th Meeting**

**NIH Campus  
Natcher Building, Rooms E1 and E2  
Bethesda, MD  
September 9-10, 2008  
Agenda**

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**Tuesday, September 9, 2008**

1:00 PM

**Call to Order and Opening Remarks**

Howard Federoff, M.D., Ph.D., Chair, NIH RAC

Tab 2514 For Your Information  
Notice of Meeting  
Conflict of Interest Guidance  
Articles of Interest

1:05 PM

**Gene Transfer Safety Assessment Board Report**

Tab 2516 Responses to M-I-C-1  
Protocol List  
Protocols Not Selected for In-Depth Review and  
Public Discussion

RAC Reviewers: Howard Federoff, M.D., Ph.D.  
Scott Strome, M.D.  
David Williams, M.D.  
John Zaia, M.D.

1:30 PM

**Discussion of Human Gene Transfer Protocol #0807-927: *Phase I Translational Trial of Oncolytic Virotherapy with Recombinant Vesicular Stomatitis Virus (rVSV(MΔ51)-M3) by Hepatic Arterial Delivery in Patients with Primary Hepatocellular Carcinoma or Metastatic Colorectal Carcinoma in the Liver***

PIs: Savio L.C. Woo, Ph.D., and Max W. Sung, M.D., Mount Sinai School of Medicine, New York, NY

RAC Reviewers: Hung Fan, Ph.D.  
Jeffrey Kahn, Ph.D., M.P.H.  
John Zaia, M.D.

**Tuesday, September 9, 2008 (continued)**

*Ad hoc* Reviewer: Roberto Cattaneo, Ph.D., Mayo Clinic, Rochester, MN  
(via teleconference)

Tab 2517 Protocol

Tab 2518 Office of Biotechnology Activities (OBA) Summary  
OBA Letter to PIs on In-Depth RAC Review and  
Public Discussion  
Reviews from Drs. Fan, Kahn, Zaia, and Cattaneo  
PIs'/Sponsor's Response

3:05 PM **Public Comment**

3:15 PM **BREAK**

3:30 PM **Discussion of Human Gene Transfer Protocol #0807-930: *A Double-Blind, Placebo-Controlled (Sham Surgery), Randomized, Multicenter Study Evaluating CERE-110 Gene Delivery in Subjects with Mild to Moderate Alzheimer's Disease***

PI: Paul S. Aisen, M.D., University of California, San Diego,  
La Jolla, CA

RAC Reviewers: Robyn Shapiro, J.D.  
Scott Strome, M.D.  
David Williams, M.D.

*Ad hoc* Reviewer: Bradley T. Hyman M.D., Ph.D., Massachusetts  
General Hospital, Charlestown, MA

Tab 2519 Protocol

Tab 2520 OBA Summary  
OBA Letter to PI on In-Depth RAC Review and  
Public Discussion  
Reviews from Drs. Strome, Williams, and Hyman and  
Ms. Shapiro  
PI's/Sponsor's Response

5:10 PM **Public Comment**

5:15 PM **ADJOURNMENT**

**Wednesday, September 10, 2008**

8:15 AM                    **Call to Order and Opening Remarks**

Howard Federoff, M.D., Ph.D., Chair, NIH RAC

8:20 AM                    **Minutes of the June 17-18, 2008, RAC Meeting**

RAC Reviewers:        Jane Flint, Ph.D.  
                                  Nikunj Somia, Ph.D.

Tab 2515        Minutes of the June 17-18, 2008, RAC Meeting

8:30 AM                    **Discussion of Human Gene Transfer Protocol #0807-932: *A Randomized, Placebo-Controlled, Double-Blind, Dose-Escalation Study to Evaluate the Safety, Tolerability, and Pharmacodynamics of Multiple Intravenous Doses of ANZ-521 in Treatment-Naïve Hepatitis C Patients***

PI:                    Eric J. Lawitz, M.D., Alamo Medical Research, San Antonio, TX

RAC Reviewers:        Hildegund Ertl, M.D.  
                                  Jeffrey Kahn, Ph.D., M.P.H.  
                                  Lee-Jen Wei, Ph.D.

Tab 2521                    Protocol

Tab 2522                    OBA Summary  
                                  OBA Letter to PI on In-Depth RAC Review and  
                                  Public Discussion  
                                  Reviews from Drs. Ertl, Kahn, and Wei  
                                  PI's/Sponsor's Response

10:10 AM                    **Public Comment**

10:20 AM                    **BREAK**

10:30 AM                    **Discussion of Human Gene Transfer Protocol #0807-931: *A Phase I/II Trial of Diaphragm Delivery of Recombinant Adeno-Associated Virus Acid Alpha-Glycosidase (rAAV1-CMV-GAA Gene Vector) in Patients with Pompe Disease***

PI:                    Barry J. Byrne, M.D., Ph.D., University of Florida, Gainesville, FL

RAC Reviewers:        Jeffrey Bartlett, Ph.D.  
                                  Eric Kodish, M.D.  
                                  James Yankaskas, M.D., M.S.

**Wednesday, September 10, 2008 (continued)**

*Ad hoc* Reviewer: Priya Kishnani, Ph.D., Duke University, Durham, NC  
(via teleconference)

Tab 2523 Protocol

Tab 2524 OBA Summary  
OBA Letter to PI on In-Depth RAC Review and  
Public Discussion  
Reviews from Drs. Bartlett, Kodish, Yankaskas, and  
Kishani  
PI's/Sponsor's Response

12:10 PM **Public Comment**

12:20 PM **WORKING LUNCH**

12:40 PM **Food and Drug Administration (FDA) Regulatory Updates:**

**Foreign Trials for Investigational New Drugs**

Presenter: Donna Przepiorka, M.D., Ph.D., FDA, Rockville, MD

**FDA Guidance on Current Good Manufacturing Practice for  
Phase I Trials**

Presenter: Daniel Takefman, Ph.D., FDA, Rockville, MD

1:15 PM **Discussion of Human Gene Transfer Protocol #0807-923:** *Compassionate  
Trial of Nanocomplex Mediated GNE Gene Replacement in Hereditary  
Inclusion Body Myopathy-2*

PI: John J. Nemunaitis, M.D., Mary Crowley Cancer Research Centers,  
Dallas, TX

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

Tab 2525 Protocol

Tab 2526 OBA Summary  
OBA Letter to PI on In-Depth RAC Review and  
Public Discussion  
Reviews from Dr. Federoff and Ms. Shapiro  
PI's/Sponsor's Response

2:30 PM **Public Comment**

**Wednesday, September 10, 2008 (continued)**

2:45 PM            **Discussion of Single Subject Human Gene Transfer Trials  
("Compassionate Use") and the Role of the RAC**

Presenter:            Jacqueline Corrigan-Curay, M.D., J.D.

Moderator:            Howard Federoff, M.D., Ph.D.

3:45 PM            **Public Comment**

3:55 PM            **BREAK**

4:05 PM            **Followup on *NIH Guidelines for Research Involving Recombinant DNA  
Molecules: Noncontemporary Influenza and Highly Pathogenic Avian  
Influenza***

Presenter:            Joseph Kanabrocki, Ph.D.

4:50 PM            **Public Comment**

5:00 PM            **ADJOURNMENT**