

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

**99<sup>th</sup> Meeting**

**Bethesda Marriott Hotel  
Bethesda, Maryland**

**March 16, 2005  
Meeting Agenda**

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**Wednesday, March 16, 2005**

**8:00 AM Call to Order and Opening Remarks**

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

Tab 2364 For Your Information  
Notice of Meeting  
Conflict of Interest Guidance

**8:10 AM Minutes of the December 16, 2004, RAC Meeting**

RAC Reviewers: Thomas Gelehrter, M.D.  
Emmett Barkley, Ph.D.

Tab 2365 Minutes of the December 16, 2004, RAC Meeting

**8:20 AM Update on Human Gene Transfer Protocol #9906-322: *A Phase I Study of NGF Ex Vivo Gene Therapy for Alzheimer's Disease***

Presenter: Mark H. Tuszynski, M.D., Ph.D., University of California, San Diego

**8:40 AM Update on Human Gene Transfer Protocol # 0401-623: *A Phase I/II, Dose-Escalating, Randomized and Controlled Study to Assess the Safety, Tolerability, and Efficacy of CERE-110 (Adeno-Associated Virus [AAV]-Based, Vector-Mediated Delivery of Beta-Nerve Growth Factor [NGF]) in Subjects with Mild to Moderate Alzheimer's Disease***

Presenter: Raymond T. Bartus, Ph.D., Ceregene, Inc.

**8:45 AM Discussion of Human Gene Transfer Protocol #0501-689: *A Phase I, Open-Label Study of CERE-120 (Adeno-Associated Virus Serotype 2 [AAV2]-Neurturin [NTN]) to Assess the Safety and Tolerability of Intrastratial Delivery to Subjects with Idiopathic Parkinson's Disease***

PI: William J. Marks, Jr., M.D., University of California, San Francisco

**Wednesday, March 16, 2005 (continued)**

RAC Reviewers: Martha Bohn, Ph.D.  
Philip Johnson, M.D.  
Terry Kwan, M.S.Ed.

*Ad Hoc* Reviewer: Howard J. Federoff, M.D., Ph.D., University of Rochester,  
Rochester, NY

Tab 2366 Protocol

Tab 2367 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, Johnson, and Federoff and Ms. Kwan  
PI's/Sponsor's Response

10:10 AM **Public Comment**

10:20 AM **BREAK**

10:35 AM **Discussion of Human Gene Transfer Protocol #0501-691:** *Phase I Trial of Systemic Administration of Edmonston Strain of Measles Virus, Genetically Engineered to Express NIS, with or without Cyclophosphamide, in Patients with Recurrent or Refractory Multiple Myeloma*

PIs: Morie A. Gertz, M.D., Mayo Clinic, Rochester, MN  
Angela Dispenzieri, M.D., Mayo Clinic, Rochester, MN

Presenter: Stephen J. Russell, M.D., Ph.D., Mayo Clinic, Rochester,  
MN

RAC Reviewers: Steven Albelda, M.D.  
Stephen Dewhurst, Ph.D.  
Bernard Lo, M.D.

*Ad Hoc* Reviewer: Diane E. Griffin, M.D., Ph.D., Johns Hopkins University,  
Baltimore, MD (*via teleconference*)

Tab 2368 Protocol

Tab 2369 OBA Summary  
OBA Letter to PIs on In-Depth RAC Review and  
Public Discussion  
Outcome of Initial Review by RAC Members  
Reviews from Drs. Albelda, Dewhurst, Lo, and Griffin  
PIs'/Sponsor's Response

**Wednesday, March 16, 2005 (continued)**

12:05 PM     **Public Comment**

12:15 PM     **Presentation of American Association for the Advancement of Science  
Award to RAC Members**

Elias A. Zerhouni, M.D., Director, National Institutes of Health

12:30 PM     **LUNCH**

1:30 PM     **General Clinical Research Center Resources for Long-Term Followup**

Presenters:           Elaine Collier, M.D., Deputy Director, Division of Clinical  
Research Resources, (DCRR), National Center for  
Research Resources (NCRR), NIH

Richard Knazek, M.D., (Contractor to NCRR), DCRR,  
NCRR, NIH

1:45 PM     **Data Management Report**

Tab 2370     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.

2:05 PM     **Discussion of Human Gene Transfer Protocol #0411-681:**

*Phase Ia/Ib Trial of Anti-PSMA Designer T Cells in Advanced Prostate Cancer  
after Nonmyeloablative Conditioning*

PI:                    Richard Paul Junghans, M.D., Ph.D., School of Medicine,  
Boston University

RAC Reviewers:     Madison Powers, J.D., D.Phil.  
                          Naomi Rosenberg, Ph.D.  
                          Diane Wara, M.D.

*Ad Hoc* Reviewer:   Theodore Friedmann, M.D., University of California,  
San Diego

Tab 2371     Protocol

**Wednesday, March 16, 2005 (continued)**

Tab 2372      OBA Summary  
OBA Letter to PI on In-Depth RAC Review and  
Public Discussion  
Outcome of Initial Review by RAC Members  
Reviews from Drs. Friedmann, Powers, Rosenberg, and Wara  
PI's/Sponsor's Response

3:35 PM      **Public Comment**

3:45 PM      **BREAK**

4:00 PM      **Update and Discussion of Human Gene Transfer Protocol #0312-619:**  
*Administration of a Replication-Deficient Adeno-Associated Virus Gene Transfer  
Vector Expressing the Human CLN2 cDNA to the Brain of Children with Late  
Infantile Neuronal Ceroid Lipofuscinosis*

Presenter:      Ronald G. Crystal, M.D., Weill Medical College, Cornell  
University, New York, NY

5:30 PM      **Public Comment**

5:45 PM      **ADJOURNMENT**