

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

117th Meeting

NIH Main Campus, Building 31, Floor 6C, Room 6

June 16–17, 2009
Agenda

Tuesday, June 16, 2009

1:50 PM

Call to Order and Opening Remarks

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

Tab 2553 For Your Information
 Notice of Meeting
 Conflict of Interest Guidance

1:55 PM

Gene Transfer Safety Assessment Board Report

Discussion of Observation of Clonal Population of Cells on European Trial: Lentiviral vector containing β -globin gene for β -Thalassemia

Tab 2554 Responses to M-I-C-1
 Protocol List
 Protocols Not Selected for In-Depth Review and
 Public Discussion

RAC Reviewers: Howard J. Federoff, M.D., Ph.D.
 Scott E. Strome, M.D.
 David A. Williams, M.D.
 James R. Yankaskas, M.D., M.S.
 John A. Zaia, M.D.

2:20 PM

Discussion of Human Gene Transfer Protocol #0904-977: *Direct CNS Administration of a Replication Deficient Adeno-Associated Virus Gene Transfer Vector Serotype rh.10 Expressing the Human CLN2 cDNA to Children with Late Infantile Neuronal Ceroid Lipofuscinosis*

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

Sponsor: Ronald G. Crystal, M.D.
 [Slide Presentation](#)

Tuesday, June 16, 2009 (continued)

RAC Reviewers: Jeffrey S. Bartlett, Ph.D.
Howard J. Federoff, M.D., Ph.D.
Jane S. Flint, Ph.D.

Ad hoc Reviewer: Ellen Wright Clayton, M.D., J.D., Vanderbilt
University School of Law, Nashville, TN

Tab 2555 Protocol

Tab 2556 OBA Summary
OBA Letter to PI on In-Depth RAC Review and
Public Discussion
Reviews from Drs. Bartlett, Clayton, Federoff
and Flint
PI's Response

4:30 PM **Public Comment**

4:35 PM **Break**

4:45 PM **Biosafety Considerations for the Cloning of the Risk Group 4
Mononegavirales – Marburg, Nipah and Hendra
viruses – in Non-Pathogenic *E. coli***

Speakers: Michael J. Buchmeier, Ph.D., M.S., RAC Member
[Slide Presentation](#)

Heinz Feldmann, M.D., Ph.D., Rocky Mountain
Laboratories, (RML), National Institute of Allergy and
Infectious Diseases (NIAID), National Institutes of
Health (NIH), Hamilton, MT
[Slide Presentation](#)

Participants: Thomas Arminio, R.N., M.P.H., RML, NIAID, NIH
Marshall Bloom, M.D., RML, NIAID, NIH
Hideki Ebihara, Ph.D., RML, NIAID, NIH

Tab 2557 Background Materials

5:45 PM **ADJOURNMENT**

Wednesday, June 17, 2009

8:20 AM

Call to Order and Opening Remarks

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

8:25 AM

Minutes

RAC Reviewers: Louis V. Kirchhoff, M.D., M.P.H.
Joseph A. Kanabrocki, Ph.D.

Tab 2558 Minutes of the March 3-4, 2009, RAC Meeting

8:30 AM

Certificates of Appreciation for RAC Member Service to NIH

Presenter: Lawrence A. Tabak, D.D.S., Ph.D., Office of the
Director, National Institutes of Health, Bethesda, MD

RAC Members: Prediman K. Shah, M.D.
Robyn S. Shapiro, J.D.
Nikunj V. Somia, Ph.D.

8:50 AM

Discussion of Human Gene Transfer Protocol #0904-975: *A Phase I Dose-escalation Clinical Trial to Evaluate the Safety and Immunogenicity of a Replication-Defective HIV-1 Vaccine (HIVAXTM) in HIV-1 Infected Subjects Receiving Highly Active Antiretroviral Therapy*

PI: Margaret A. Fischl, M.D., University of Miami School
of Medicine, Miami, FL
[Slide Presentation](#)

Sponsor: GeneCure Biotechnologies L.L.C.

RAC Reviewers: Jeffrey P. Kahn, Ph.D., M.P.H.
Nikunj V. Somia, Ph.D.
John A. Zaia, M.D.

Tab 2559 Protocol

Tab 2560 OBA Summary
OBA Letter to PI on In-Depth RAC Review and
Public Discussion
Reviews from Drs. Kahn, Somia and Zaia
PI's Response

10:30 AM

Public Comment

10:40 AM

Break

Wednesday, June 17, 2009 (continued)

10:50 AM **Discussion of Human Gene Transfer Protocol #0904-981: A Phase 1/2 Trial Assessing the Safety and Efficacy of Bilateral Intraputaminial and Intranigral Administration of CERE-120 (Adeno-Associated Virus Serotype 2 [AAV2]-Neurturin [NTN]) in Subjects with Idiopathic Parkinson's Disease**

PIs: William J. Marks, Jr., M.D., University of California, San Francisco, CA
Mark Stacy, M.D. Duke University, Durham, NC
[¹Slide Presentation](#)

Sponsor: Ceregene, Inc.

RAC Reviewers: Hildegund C.J. Ertl, M.D.
Robyn S. Shapiro, J.D.
Lee-Jen Wei, Ph.D.
James R. Yankaskas, M.D., M.S.

Ad hoc Reviewers: John D. Elsworth, Ph.D., Yale University, New Haven, CT
Thomas Freeman, M.D., University of South Florida, Tampa, FL

Tab 2561 Protocol

Tab 2562 OBA Summary
OBA Letter to PI on In-Depth RAC Review and Public Discussion
Reviews from Drs. Elsworth, Ertl, Freeman, Wei, Yankaskas and Ms. Shapiro
PI's Response

12:45 PM **Public Comment**

1:00 PM **Lunch**

1:45 PM **Discussion of Human Gene Transfer Protocol #0904-976: A Phase 1 Ascending Dose Trial of the Safety and Tolerability of Toca 511 in Patients with Recurrent Glioblastoma Multiforme**

PI: Manish Aghi, M.D., Ph.D., University of California, San Francisco, CA
[Slide Presentation](#)

Sponsor: Tocagen Inc.

¹ The Sponsor has withheld slides containing unpublished data – please refer to [Webcast](#) for complete presentation.

Wednesday, June 17, 2009 (continued)

RAC Reviewers: Hung Y. Fan, Ph.D.
Kalle Gerritz, Ed.D.
David A. Williams, M.D.

Tab 2563 Protocol

Tab 2564 OBA Summary
OBA Letter to PI on In-Depth RAC Review and
Public Discussion
Reviews from Drs. Fan, Gerritz and Williams
PI's Response

3:30 PM **Public Comment**

3:45 PM **ADJOURNMENT**