

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

115th Meeting

**Terrace Level
5635 Fishers Lane
Rockville, Maryland**

**December 3-4, 2008
Agenda**

Wednesday, December 3, 2008

9:30 AM **Call to Order and Opening Remarks**

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

Tab 2527 For Your Information
 Notice of Meeting
 Conflict of Interest Guidance

9:35 AM **Minutes of the September 9-10, 2008, RAC Meeting**

Tab 2528 Minutes of the September 9-10, 2008, RAC Meeting

RAC Reviewers: Prediman K. Shah, M.D.
 James R. Yankaskas, M.D., M.S.

9:40 AM **Followup on *NIH Guidelines for Research Involving Recombinant DNA Molecules: Human H2N2, Reconstructed 1918 H1N1, and Highly Pathogenic Avian Influenza H5N1 Viruses***

Tab 2539 Background Materials

Presenter: Joseph A. Kanabrocki, Ph.D. – [Slide Presentation](#)

10:40 AM **Public Comment**

10:50 AM **Discussion of Human Gene Transfer Protocol #0808-946: *Phase I Trial of Intratumoral Injection of Vesicular Stomatitis Virus Expressing Interferon Beta in Patients with Hepatocellular Carcinoma***

PIs: Mitesh J. Borad, M.D., Mayo Clinic, Scottsdale, AZ
 Jorge Rakela, M.D., Mayo Clinic, Rochester, MN
 Richard G. Vile, Ph.D., Mayo Clinic, Rochester, MN
 [Slide Presentation](#)

Wednesday, December 3, 2008 (continued)

RAC Reviewers: Jeffrey Bartlett, Ph.D.
Jeffrey P. Kahn, Ph.D., M.P.H.
Bernard Roizman, Sc.D.

Tab 2529 Protocol

Tab 2530 OBA Summary
OBA Letter to PIs on In-Depth RAC Review and
Public Discussion
Reviews from Drs. Bartlett, Kahn, and Roizman

12:15 PM **Public Comment**

12:20 PM **Lunch**

1:00 PM **Gene Transfer Safety Assessment Board Report**

Tab 2531 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.

1:15 PM **Discussion of Human Gene Transfer Protocol #0808-942: A Phase Ib, Multicenter, Single-Blinded, Placebo-Controlled, Sequential Dose-Escalation Study to Assess the Safety of Topically Applied AGO13 in Subjects Receiving Induction Chemotherapy for the Treatment of Cancers of the Head and Neck**

Sponsor: ActoGeniX, NV – [Slide Presentation](#)

PI: Barbara Murphy, M.D., Vanderbilt University, Nashville, TN

RAC Reviewers: David A. Alland, M.D.
Scott E. Strome, M.D.
John A. Zaia, M.D.

Tab 2532 Protocol

Tab 2533 OBA Summary
OBA Letter to PI on In-Depth RAC Review and
Public Discussion
Reviews from Drs. Alland, Strome, and Zaia

Wednesday, December 3, 2008 (continued)

2:50 PM **Public Comment**

2:55 PM **Break**

3:15 PM **Discussion of Human Gene Transfer Protocol #0808-934:** *A Phase I, Open-Label Study of the Safety, Tolerability, and Therapeutic Activity of JVRS-100 Cationic Lipid-DNA Complex in Patients with Chronic Hepatitis C Infection Who Relapsed After Receiving Interferon-Ribavirin Treatment*

PI: John McHutchison, M.D., Duke University Medical Center, Durham, NC

Sponsor: Juvaris BioTherapeutics, Inc., CA – [Slide Presentation](#)

AND

Discussion of Human Gene Transfer Protocol #0808-936: *Phase I Trial of the Immunostimulant JVRS-100 for the Treatment of Patients with Relapsed or Refractory Leukemia*

PI: David F. Claxton, M.D., Penn State Hershey Cancer Institute, Hershey, PA

Sponsor: Juvaris BioTherapeutics, Inc., CA – [Slide Presentation](#)

RAC Reviewers: Hildegund C.J. Ertl, M.D.
 Louis V. Kirchhoff, M.D., M.P.H.
 Robyn J. Shapiro, J.D.
 David A. Williams, M.D.

Tab 2534 Protocol

Tab 2535 Protocol

Tab 2536 OBA Summary
 OBA Letter to PI on In-Depth RAC Review and
 Public Discussion
 Reviews from Drs. Ertl, Kirchhoff, and Williams and
 Ms. Shapiro

4:40 PM **Public Comment**

4:45 PM **ADJOURNMENT**

Thursday, December 4, 2008

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

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

8:10 AM **Update on Allogeneic Bone Marrow X-Transplantation for X-SCID**

Presenters: Rebecca H. Buckley, M.D., Duke University Medical Center,
Durham, NC – [Slide Presentation](#)

Morton J. Cowan, M.D., UCSF Children’s Hospital,
San Francisco, CA – [Slide Presentation](#)

Richard J. O’Reilly, M.D., Memorial Sloan-Kettering
Cancer Center, New York, NY – [Slide Presentation](#)

9:40 AM **Break**

9:50 AM **Discussion of Human Gene Transfer Protocol #0808-950: *Gene Therapy for SCID-X1 Using a Self-Inactivating (SIN) Gammaretroviral Vector***

PIs: Luigi Notarangelo, M.D., Children’s Hospital Boston, Boston, MA
Adrian Thrasher, M.D., Institute of Child Health, London, England

Sponsor: David Williams, M.D., Children’s Hospital Boston, Boston,
MA – [Slide Presentation](#)

RAC Reviewers: Hung Y. Fan, Ph.D.
Howard J. Federoff, M.D., Ph.D.
Eric D. Kodish, M.D.

Ad hoc Reviewer: Naomi Rosenberg, Ph.D., Tufts University, Boston,
MA

Tab 2537 Protocol

Tab 2538 OBA Summary
OBA Letter to PI on In-Depth RAC Review and
Public Discussion
Reviews from Drs. Fan, Federoff, Kodish, and Rosenberg

12:00 NOON **Public Comment**

12:10 PM **ADJOURNMENT**