

NATIONAL INSTITUTES OF HEALTH (NIH)
RECOMBINANT DNA ADVISORY COMMITTEE (RAC)
135th MEETING
HYATT REGENCY BETHESDA
SEPTEMBER 11-12, 2013

Wednesday, September 11, 2013

1:20 PM

Donald Kohn, M.D., RAC Chair

Tab 2758 For Your Information
Notice of Meeting
Conflict of Interest Guidance

1:30 PM

Discussion of Human Gene Transfer Protocol #1307-1236 titled:
*Infusion of Allogeneic, 3rd Party CD19⁺-specific T cell (CD19RCD137⁺
T Cells) in Patients with Refractory CD19⁺ B-Lineage Malignancies*

PIs: Partow Kebriaei, M.D., University of Texas, MD Anderson
Cancer Center, Houston, TX

Laurence J.N. Cooper, M.D., Ph.D., University of Texas,
MD Anderson Cancer Center, Houston, TX

[Slide Presentation](#)

RAC Reviewers: Hans-Peter Kiem, M.D., Ph.D.
Joseph Pilewski, M.D.
Dawn Wooley, Ph.D.

Tab 2759 Protocol

Tab 2760 OBA Summary
OBA Letters to PI on In-Depth RAC Review and
Public Discussion
Reviews from Drs. Kiem, Pilewski and Wooley
PIs' Response

2:40 PM

Public Comments

(continued) Wednesday, September 11, 2013

2:45 PM

Update on the Use of Lentiviral Vectors for Long-Term Gene Correction for Wiskott Aldrich Syndrome and Metachromic Leukodystrophy

Presenter: Luigi Naldini, M.D., Ph.D., H San Raffaele – Tele Thon Institute for Gene Therapy –HSR-TIGET, University of Milan, Italy
[Slide Presentation](#)

3:15 PM

Discussion on Human Gene Transfer Protocol #1307-1241 titled: *A Phase I Study of Intracranially Administered Carboxylesterase-Expressing Neural Stem Cells in Combination with Intravenous Irinotecan for the Treatment of Recurrent High-Grade Gliomas*

PIs: Karen Aboody, M.D., City of Hope, Duarte, CA
To be posted

Jana Portnow, M.D., City of Hope, Duarte, CA
[Slide Presentation](#)

RAC Reviewers: Walter Koch, Ph.D.
Donald Kohn, M.D.
Laurie Zoloth, Ph.D.

Tab 2761 Protocol

Tab 2762 OBA Summary
OBA Letters to PI on In-Depth RAC Review and Public Discussion
Reviews from Drs. Koch, Kohn and Zoloth
PIs' Response

4:30 PM

Public Comments

4:35 PM

Gene Transfer Safety Assessment Board Report

RAC Reviewers: Hans-Peter Kiem, M.D., Ph.D.
Donald Kohn, M.D.
Joseph Pilweski, M.D.
Marshall Strome, M.D., M.S.
[Slide Presentation](#)

(continued) Wednesday, September 11, 2013

Tab 2763 Responses to M-I-C-1
Protocol List
Protocols Not Selected for In-Depth Review and
Public Discussion

4:45 PM **Adjourn**

Thursday, September 12, 2013

8:30 AM **Call to Order**

Minutes of RAC Meeting, June 11-12, 2013

RAC Reviewers: Joseph Pilewski, M.D.
 Marshall Strome, M.D., M.S.

8:35 AM **Unexpected False Positive PCR Test for HIV Following Lentiviral Gene
Transfer for X-SCID (OBA Protocol #0901-964)**

Presenter: Suk See De Ravin, M.D., National Institute of Allergy
 and Infectious Diseases, NIH, Bethesda, MD
[Slide Presentation](#)

9:00 AM **Discussion of Human Gene Transfer Protocol #1307-1239 titled: *Phase
Ib Study Using Patient-Derived Tumor Cells Expressing a Streptococcal
Antigen in Patients with Indolent Non-Hodgkin Lymphoma***

PI: Philip Bierman, M.D., University of Nebraska Medical
 Center, Omaha, NE

Sponsor: Morphogenesis, Inc.
[Slide Presentation](#)

RAC Reviewers: Saswati Chatterjee, Ph.D.
 Rebecca Dresser, J.D.
 Marshall Strome, M.D., M.S.

Tab 2764 Protocol

(continued) Thursday, September 12, 2013

Tab 2765 OBA Summary
OBA Letters to PI on In-Depth RAC Review and
Public Discussion
Reviews from Drs. Chatterjee, Strome and Professor
Dresser
PIs' Response

10:15 AM **Public Comments**

10:20 AM **BREAK**

10:30 AM **Guidance for Industry: Consideration for the Design of Early-Phase
Clinical Trials of Cellular and Gene Therapy Products**

Presenter: John Hyde, Ph.D., M.D., Food and Drug Administration,
Silver Spring, MD
[Slide Presentation](#)

10:55 AM **Public Comments**

11:00 AM **Discussion on Human Gene Transfer Protocol #1307-1240 titled: A
Phase I, Open-Label Study to Assess the Safety, Feasibility and
Engraftment of Zinc Finger Nucleases CCR5 Modified Autologous CD34⁺
Hematopoietic Stem/Progenitor Cells (Sb-728mr-Hspc) with Escalating
Doses of Busulfan in HIV-1 (R5) Infected Subjects with Suboptimal CD4
Levels on Antiretroviral Therapy**

PIs: Amrita Krishnan, M.D., City of Hope, Duarte, CA

John Zaia, M.D, City of Hope, Duarte, CA
[Slide Presentation](#)

RAC Reviewers: Norman Fost, M.D., M.P.H.
Marie Louise Hammarskjöld, M.D., Ph.D.
David Ornelles, Ph.D.

Tab 2766 Protocol

(continued) Thursday, September 12, 2013

Tab 2767 OBA Summary
OBA Letters to PI on In-Depth RAC Review and
Public Discussion
Reviews from Drs. Fost, Hammarskjöld and Ornelles
PIs' Response

12:40 PM **Public Comments**

12:45 PM **OBA Updates: Comments on Proposal to Exempt Certain Human Gene
Transfer Trials from Institutional Biosafety Committee Review and Updates
to Appendix B of the *NIH Guidelines for Research Involving Recombinant or
Synthetic Nucleic Acid Molecules***

Presenter: Jacqueline Corrigan-Curay, J.D., M.D., Acting Director, Office
of Biotechnology Activities, Office of Science Policy, NIH
[Slide Presentation](#)

1:15 PM **Adjourn**