

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

129TH MEETING

NIH/BUILDING 31C, 6TH FLOOR
CONFERENCE ROOM 10

JUNE 19, 2012

AGENDA

Tuesday, June 19, 2012

9:15 AM **Call to Order and Opening Remarks**

Yuman Fong, M.D., RAC Chair

Tab 2704 For Your Information
Notice of Meeting
Conflict of Interest Guidance

9:20 AM **Approval of Minutes**

Tab 2705 Minutes of RAC Meeting, March 7-8, 2012

RAC Reviewers: Joseph Kanabrocki, Ph.D.
Margaret Mallino

9:25 AM **Update on Immunotherapy Trials for HIV and Cancer**

Presenter: Carl June, M.D., Perelman School of Medicine,
University of Pennsylvania, Philadelphia, PA

9:55 AM **Public Comment**

10:00 AM **Gene Transfer Safety Assessment Board Report**

Tab 2706 Responses to M-1-C-1
Protocol List
Protocols Not Selected for In-Depth Review and Public
Discussion

RAC Reviewers: Andrew Badley, M.D.
E. Antonio Chiocca, M.D., Ph.D.
Yuman Fong, M.D.
Hans-Peter Kiem, M.D., Ph.D.

(continued) Tuesday, June 19, 2012

Donald Kohn, M.D.
Marshall Strome, M.D., M.S.
James Yankaskas, M.D., M.S.

10:10 AM **First-in-Human use of Gene Transfer Agents in “Compassionate Use”
Protocols**

10:45 AM **Public Comment**

10:50 AM **Break**

11:00 AM **Discussion of Human Gene Protocol #1202-1164 titled: *A
Phase 1/2, Open Label Study Evaluating the Safety and Efficacy of Gene
Therapy in Subjects with β -Thalassemia Major by Transplantation of
Autologous CD34⁺ Stem Cells Transduced Ex Vivo with a Lentiviral β^{A-T87Q} -
Globin Vector.***

PI: Donald Kohn, M.D., University of California,
Los Angeles, CA

Sponsor: bluebird bio, Inc. Cambridge, MA

RAC Reviewers: Rebecca Dresser, J.D.
David Ornelles, Ph.D.
Susan Ross, Ph.D.

Tab 2707 Protocol

Tab 2708 OBA Summary
OBA Letter to PI on In-Depth RAC Review and
Public Discussion
Reviews from Professor Dresser, Drs. Ornelles and
Ross
PI’s Response

12:15 PM **Public Comment**

12:20 PM **LUNCH**

1:00 PM **Discussion of Human Gene Protocol #1202-1153 titled: *Stem Cell Gene
Therapy for HIV in AIDS Lymphoma Patients***

PI: Mehrdad Abedi, M.D., University of California Davis
Cancer Center, Sacramento, CA

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RAC Reviewers: Andrew Badley, M.D.
Saswati Chatterjee, Ph.D.
Norman Fost, M.D., M.P.H

Ad hoc: Ronald Mitsuyasu, M.D., University of California
Los Angeles, Los Angeles CA

Tab 2709 Protocol

Tab 2710 OBA Summary
OBA Letter to PI on In-Depth RAC Review and
Public Discussion
Reviews from Drs. Badley, Chatterjee and Fost
PI's Response

2:10 PM **Public Comment**

2:15 PM **Presentation of Certificates of Appreciation for Departing RAC
Members**

2:30 PM **Update on Protocol #0912-1016 titled:** *A Phase II Study Determine the
Efficacy and Safety of Allogeneic Human Chondrocytes Expressing TGF- β 1
In Patients with Grade 3 Chronic Degenerative Joint Disease of the Knee*

PI: Michael A. Mont, M.D., Sinai Hospital of Baltimore,
Baltimore, MD

Sponsor: Kwan Hee Lee, M.D., Ph.D., TissueGene, Inc.,
Rockville, MD

Ad hoc Martin Lotz, M.D., Research Institute, La Jolla, CA

3:20 PM **Public Comment**

3:30 PM **Adjournment**