

NATIONAL INSTITUTES OF HEALTH (NIH)
RECOMBINANT DNA ADVISORY COMMITTEE (RAC)
137th MEETING
6701 ROCKLEDGE DRIVE
CONFERENCE ROOM 9100
MARCH 12, 2014
AGENDA

Wednesday, March 12, 2014

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

Donald Kohn, M.D., RAC Chair

Tab 2781 For Your Information
 Notice of Meeting
 Conflict of Interest Guidance

9:35 AM **Minutes**

Tab 2782 Minutes of the December 4-5, 2013, RAC Meeting

RAC Reviewers: David Ornelles, Ph.D.
 Walter Koch, Ph.D.

9:40 AM **Discussion on Human Gene Transfer Protocol #1401-1287 titled: *Phase I Study of Cellular Immunotherapy Using T Cells Lentivirally Transduced to Express a CD123-Specific, Hinge-Optimized, CD28-Costimulatory Chimeric Antigen Receptor and a truncated EGFR for Patients with Relapsed or Refractory CD123⁺ Acute Myeloid Leukemia***

PI: Elizabeth Budde, M.D., Ph.D, City of Hope, Duarte, CA

Project Leader: Stephen Forman, M.D., City of Hope Duarte, CA
[Slide Presentation](#)

RAC Reviewers: Rebecca Dresser, J.D.
 Hans-Peter Kiem, M.D., Ph.D.
 Michel Sadelain, M.D., Ph.D.

(continued) Wednesday, March 12, 2014

Tab 2783 Protocol

Tab 2784 OBA Summary
OBA Letter to PI on In-Depth RAC Review and
Public Discussion
Reviews from Drs. Kiem, and Sadelain, and Professor
Dresser
PI's Response

11:00 AM **Public Comments**

11:10 AM **Discussion on Human Gene Transfer Protocol #1401-1288 titled:** *Phase I Study to Determine the Effects of Mesenchymal Stem Cells Secreting Interferon Beta in Patients with Advanced Ovarian Cancer*

PI: Amanda Olson, M.D., The University of Texas MD Anderson
Cancer Center, Houston, TX

[Slide Presentation](#)

RAC Reviewers: Donald Kohn, M.D.
Marcella Sarzotti-Kelsoe, Ph.D.
Laurie Zoloth, Ph.D.

Tab 2785 Protocol

Tab 2786 OBA Summary
OBA Letter to PI on In-Depth RAC Review and
Public Discussion
Reviews from Drs. Kohn, Sarzotti-Kelsoe, and Zoloth
PI's Response

12:30 PM **Public Comments**

12:40 PM **Lunch**

1:20 PM **Discussion on Human Gene Transfer Protocol #1401-1289:** *Phase I/II Gene Transfer Clinical Trial of rAAV9.MCV.hNAGLU for Mucopolysaccharidosis (MPS) IIIB*

PI: Kevin M. Flanigan, M.D., The Research Institute at Nationwide
Children's Hospital, Columbus, OH

Sponsor: Abeona Therapeutics Inc.
[Slide Presentation](#)

RAC Reviewers: Michael Atkins, M.D.
Saswati Chatterjee, Ph.D.
Norman Fost, M.D. M.P.H.

Tab 2787 Protocol

Tab 2788 OBA Summary
OBA Letter to PI on In-Depth RAC Review and
Public Discussion
Reviews from Drs. Atkins, Chatterjee, and Fost
PI's Response

3:00 PM **Public Comments**

3:10 PM **Gene Transfer Safety Assessment Board Report**

RAC Reviewers: Michael Atkins, M.D.
William Curry, M.D.
Hans-Peter Kiem, M.D., Ph.D.
Donald Kohn, M.D.
Joseph Pilewski, M.D.
Michel Sadelain, M.D., Ph.D.
Marshall Strome, M.D., M.S.
Richard Whitley, M.D.

Tab 2789 Responses to M-I-C-1
Protocol List
Protocols Not Selected for In-Depth Review and
Public Discussion

3:30 PM **Adjourn**