

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

129TH MEETING

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

JUNE 19, 2012

AGENDA

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*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  
                                                    **Slide Presentation**
- 10:25 AM            **Public Comment**
- 10:30 AM            **First-in-Human use of Gene Transfer Agents in “Compassionate Use”  
                         Protocols**
- Slide Presentation**
- 10:45 AM            **Public Comment**
- 11:00 AM            **Break**
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(continued) Tuesday, June 19, 2012

11:10 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  $\beta$ -Thalassemia Major by Transplantation of Autologous CD34<sup>+</sup> Stem Cells Transduced Ex Vivo with a Lentiviral  $\beta^{A-T87Q}$ -Globin Vector.**

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

Sponsor:            bluebird bio, Inc. Cambridge, MA  
**Slide Presentation**

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

12:10 PM            **Public Comment**

12:15 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.

Donald Kohn, M.D.  
Marshall Strome, M.D., M.S.  
James Yankaskas, M.D., M.S.  
**Slide Presentation**

12:20 PM            **LUNCH**

*(continued) Tuesday, June 19, 2012*

- 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  
**Slide Presentation**
- 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- $\beta$ 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  
**Slide Presentation**
- 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**