

NATIONAL INSTITUTES OF HEALTH (NIH)  
RECOMBINANT DNA ADVISORY COMMITTEE (RAC)  
139 MEETING  
6701 ROCKLEDGE DRIVE  
CONFERENCE ROOM 9100  
SEPTEMBER 9, 2014  
DAY 1 AGENDA

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*Tuesday, September 9, 2014*

08:00 AM            **Orientation for New Members**

9:00 AM            **BREAK**

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

**Donald Kohn, M.D., RAC Chair**

Tab 2799        For Your Information  
                  Notice of Meeting  
                  Conflict of Interest Guidance

9:20 AM            **Discussion on Human Gene Transfer Protocol #1405-1315: *Phase I Trial of Oncolytic Adenovirus-mediated Cytotoxic and Interleukin 12 Gene Transfer for Radio-recurrent Prostate Cancer***

PI:        Svend Freytag, Ph.D., Henry Ford Health System, Detroit, MI

RAC Reviewers:    Michael Atkins, M.D.  
                          David Ornelles, Ph.D.  
                          Rebecca Dresser, J.D.

Tab 2800            Protocol

Tab 2801            OBA Summary  
                          OBA Letter to PI on In-Depth RAC Review and  
                          Public Discussion  
                          Reviews from Drs. Atkins, Ornelles, and Professor  
                          Dresser  
                          PI's Response

10:30 AM **Public Comments**

10:35 AM **BREAK**

10:45 AM **Discussion on Human Gene Transfer Protocol #1404-1304 titled: A Phase I, Multicenter, Open-Label, Single-Dose Study to Assess the Safety and Tolerability of Autologous Hematopoietic Stem Progenitor Cells (HSPC) Modified at the BCL11A Gene by Zinc Finger Nucleases (SB-BCLmR-HSPC) to Increase Fetal Hemoglobin Production in Subjects with Transfusion Dependent  $\beta$ -Thalassemia**

PI: Mark Walters, M.D., UCSF Benioff Children's Hospital Oakland, Oakland, CA

Sponsor: Sangamo BioSciences, Inc.

RAC Reviewers: Marie-Louise Hammarskjöld, M.D., Ph.D.  
Laurie Zoloth, Ph.D.

*Ad Hoc* Reviewers: David Segal, Ph.D. University of California, Davis, CA  
John Tisdale, M.D., National Heart, Lung, and Blood Institute, NIH, Bethesda, MD

Tab 2802 Protocol

Tab 2803 OBA Summary  
OBA Letter to PI on In-Depth RAC Review and Public Discussion  
Reviews from Drs. Hammarskjöld, Zoloth, Segal, Tisdale  
PI's Response

12:10 PM **Public Comments**

12:15 PM **LUNCH**

1:00 PM **RAC Member Training**

1:45 PM **Discussion on Human Gene Transfer Protocol #1405-1313 titled: A Phase I Trial of the Safety and Immunogenicity of a Multiple Antigen Vaccine (STEMVAC) in HER2 Negative Advanced Stage Breast Cancer Patients**

PI: Mary L. (Nora) Disis, M.D., University of Washington School of Medicine, Seattle, WA

RAC Reviewers: Saswati Chatterjee, Ph.D.  
Joseph Pilewski, M.D.

Angelica Hardison, M.B.A.

Tab 2804 Protocol

Tab 2805 OBA Summary  
OBA Letter to PI on In-Depth RAC Review and  
Public Discussion  
Reviews from Drs. Chatterjee and Pilewski, and Mrs.  
Hardison  
PI's Response

3:00 PM **Public Comments**

3:10 PM **Discussion on Human Gene Transfer Protocol # 1406-1320 titled:**  
*Phase I Study of T Cells Expressing an Anti-CD22 Chimeric Receptor in  
Children and Young Adults with B Cell Malignancies*

PI: Terry J. Fry, M.D., National Cancer Institute, NIH, Bethesda, MD

RAC Reviewers: Marcella Sarzotti-Kelsoe, Ph.D.  
Donald Kohn, M.D.  
Norman Fost, M.D., M.P.H.

Tab 2806 Protocol

Tab 2807 OBA Summary  
OBA Letter to PI on In-Depth RAC Review and  
Public Discussion  
Reviews from Drs. Fost, Kohn, and Sarzotti-Kelsoe  
PI's Response

4:30 PM **Public Comments**

4:35 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.  
Richard Whitley, M.D.

Tab 2808 Responses to M-I-C-1  
Protocol List  
Protocols Not Selected for In-Depth Review and  
Public Discussion

4:45 PM **ADJOURN**

