



RECOMBINANT DNA ADVISORY COMMITTEE MEETING

Porter Neuroscience Research Center (Building 35)
NIH Campus, Bethesda, MD



National Institutes
of Health

AGENDA - SEPTEMBER 9, 2015

ADMINISTRATIVE ORIENTATION – CLOSED SESSION

- 8:30 AM RAC Overview and Updates**
Lyric Jorgenson, PhD – RAC Executive Secretary, NIH
- 9:00 AM Roles and Responsibilities of Federal Advisory Committees**
Anna Snouffer – Deputy Director, Office of Federal Advisory Committee Policy, NIH
- 9:20 AM Ethics Overview**
Kevette Burwell – Team Lead, NIH Ethics Office, NIH
- 9:40 AM BREAK**

RAC MEETING – OPEN SESSION

- 10:00 AM Call to Order and Opening Remarks**
Hans-Peter Kiem, MD, PhD – RAC Chair
Tab 2840: For Your Information
Notice of Meeting
Conflict of Interest Guidance
- 10:05 AM Minutes – Day 1**
RAC Reviewers: William Curry, MD
Dawn Wooley, PhD
Tab 2841: Minutes of the June 9, 2015 RAC Meeting
- 10:10 AM Discussion on Human Gene Transfer Protocol #1507-1446 titled: *A Phase I, Open-Label, Ascending Dose Study to Assess the Safety and Tolerability of AAV2/6 Factor IX Gene Therapy via Zinc Finger Nuclease (ZFN) Mediated Targeted Integration of SB-FIX in Adult Subjects with Severe Hemophilia B***
Presenters: Nadia Ewing, MD, Director, Hemophilia and Sickle Cell Program, City of Hope, Duarte, California
John Zaia, MD, Aaron D. Miller and Edith Miller Chair in Gene Therapy, City of Hope, Duarte, California

(CONTINUED) TUESDAY, SEPTEMBER 9, 2015

Sponsor: Sangamo BioSciences, Inc.

RAC Reviewers: Kevin Donahue, MD
Marie-Louise Hammarskjöld, MD, PhD
Patrick Hearing, PhD

Tab 2842: Protocol

Tab 2843: OBA Summary
OBA Letter to PI on In-depth RAC Review and Public Discussion
Reviews from Drs. Donahue, Hammarskjöld, and Hearing
PI's Response

11:25 AM Public Comments

11:30 AM LUNCH

12:30 PM Discussion on Human Gene Transfer Protocol #1507-1449 titled: *A Phase I/II Multicenter, Open-Label Study to Evaluate the Safety, Tolerability, and Pharmacodynamics of RGX-111 in Subjects with MPS I (IDUA Deficiency)*

Presenter: Stephen Yoo, MD, Chief Medical Officer Regenxbio, Inc., Rockville, Maryland

Raymond Wang, MD, Director of Multidisciplinary Lysosomal Storage
Disorder Program CHOC Children's, Orange, California

Christian Hinderer, PhD, Gene Therapy Program, Department of Pathology
and Laboratory Medicine, Perelman School of Medicine, University
of Pennsylvania, Philadelphia, Pennsylvania

Sponsor: RegenxBio, Inc.

RAC Reviewers: Michael Atkins, MD
Saswati Chatterjee, PhD
Angelica Hardison, MBA

Tab 2844: Protocol

Tab 2845: OBA Summary
OBA Letter to PI on In-depth RAC Review and Public Discussion
Reviews from Drs. Atkins and Chatterjee, and Ms. Hardison
PI's Response

1:45 PM Public Comments

(CONTINUED) TUESDAY, SEPTEMBER 9, 2015

1:50 PM BREAK

2:00 PM Discussion on Human Gene Transfer Protocol #1507-1445 titled: *Phase I/II Study Administering Peripheral Blood Lymphocytes Transduced with a CD70-binding Chimeric T-Cell Receptor to Patients with CD70 Expressing Cancers*

Presenter: James Yang, MD, Senior Investigator, Surgery Branch, National Cancer Institute, National Institutes of Health, Bethesda, Maryland

RAC Reviewers: Hans-Peter Kiem, MD, PhD
Joseph Pilewski, MD
Lainie Ross, MD, PhD

Tab 2846: Protocol

Tab 2847: OBA Summary
OBA Letter to PI on In-depth RAC Review and Public Discussion
Reviews from Drs. Kiem, Pilewski, and Ross
PI's Response

3:15 PM Public Comments

3:20 PM BREAK

3:30 PM Gene Transfer Safety Assessment Board Report

RAC Reviewers: Michael Atkins, MD
William Curry, MD
Kevin Donahue, MD
Howard Kaufman, MD
Hans-Peter Kiem, MD, PhD
Dean Lee, MD, PhD
Joseph Pilewski, MD
Richard Whitley, MD

Tab 2848: Responses to M-I-C-1
Protocol List
Protocols Not Selected for In-Depth Review and Public Discussion

4:00 PM ADJOURN