



RECOMBINANT DNA ADVISORY COMMITTEE

Office of Biotechnology Activities - Office of Science Policy
National Institutes of Health
Natcher Conference Center (Building 45)
Bethesda, MD



AGENDA - JUNE 9, 2015

12:00 PM Call to Order and Opening Remarks

Donald Kohn, MD, RAC Chair

Tab 2831 For Your Information
Notice of Meeting
Conflict of Interest Guidance

12:05 PM Minutes

Tab 2832 Minutes of the March 10, 2015 RAC Meeting

RAC Reviewers: Saswati Chatterjee, PhD
Angelica Hardison, MBA

12:15 PM Design and Analysis of Shedding Studies for Virus or Bacteria-Based Gene Therapy and Oncolytic Products

Tab 2833

Presenter: Zenobia Taraporewala, PhD, Division of Cellular and Gene Therapies,
Food and Drug Administration

12:45 PM Outgoing Members Award Ceremony

Presenter: Lawrence Tabak, DDS, PhD, Principal Deputy Director, NIH

12:55 PM Discussion on Human Gene Transfer Protocol #1504-1415 titled: *HD-CELL: MSC Engineered to Produce BDNF for the Treatment of Huntington's Disease*

PI: Vicki Wheelock, MD, Clinical Professor, University of California,
Davis Medical Center

RAC Reviewers: William Curry, MD
Donald Kohn, MD
Laurie Zoloth, PhD

Ad Hoc Reviewer: Wendy Galpern, MD, PhD, National Institute of
Neurological Disorders and Stroke, NIH

(CONTINUED) TUESDAY, JUNE 9, 2015

Tab 2834 Protocol

Tab 2835 OBA Summary
OBA Letter to PI on In-depth RAC Review and
Public Discussion
Reviews from Drs. Curry, Galpern, Kohn, and Zoloth
PI's Response

2:15 PM Public Comments

2:25 PM BREAK

2:35 PM Discussion on Human Gene Transfer Protocol #1504-1396 titled: *A Phase III, Open-Label Study Evaluating the Efficacy and Safety 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 (LentiGlobin BB305 Drug Product) in Subjects ≥ 5 and < 12 Years of Age*

PI: Alexis Thompson, MD, MPH, Ann & Robert H. Lurie Children's
Hospital of Chicago

Sponsor: bluebird bio, Inc.

RAC Reviewers: Rebecca Dresser, JD
Marie-Louise Hammarskjöld, MD, PhD
Hans-Peter Kiem, MD, PhD

Ad Hoc Reviewer: Harvey Luksenburg, MD, National Heart
Lung, and Blood Institute, NIH

Tab 2836 Protocol

Tab 2837 OBA Summary
OBA Letter to PI on In-depth RAC Review and
Public Discussion
Reviews from Drs. Hammarskjöld, Kiem, and Professor
Dresser
PI's Response

3:55 PM Public Comments

4:00 PM Update on Human Gene Transfer Protocol #0810-946 titled: *Phase I Trial of Intratumoral Injection of Vesicular Stomatitis Virus Expressing Human Interferon Beta in Patients with Sorafenib Refractory/Interlant Hepatocellular Carcinoma and Advanced Solid Tumors with Liver Predominant Locally dvanced/Metastatic Disease*

Presenter: Mitesh Borad, MD, Mayo Clinic, Scottsdale, Arizona

(CONTINUED) TUESDAY, JUNE 9, 2015

4:15 PM Vector Design Influences Hepatic Genotoxicity after Adeno-Associated Virus Gene Therapy

Presenter: Charles Venditti, MD, PhD National Human Genome Research Institute, NIH

Tab 2838 Presentation

4:45 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
 Donald Kohn, MD
 Joseph Pilewski, MD
 Michel Sadelain, MD, PhD
 Richard Whitley, MD

Tab 2839 Responses to M-I-C-1
 Protocol List
 Protocols Not Selected for In-Depth Review and
 Public Discussion

Tab 2839a Update on Protocol #0810-946: *Phase I Trial of Intratumoral Injection of Vesicular Stomatitis Virus Expressing Human Interferon Beta in Patients with Sorafenib Refractory/Intolerant Hepatocellular Carcinoma and Advanced Solid Tumors with Liver Predominant Locally Advanced/Metastatic Disease*

5:00 PM ADJOURN