

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

134TH MEETING

NIH/BUILDING 31, 6TH FLOOR
CONFERENCE ROOM 6

JUNE 11-12, 2013

Tuesday, June 11, 2013

8:00 AM

Call to Order and Opening Remarks

Yuman Fong, M.D., RAC Chair

Tab 2740 For Your Information
Notice of Meeting
Conflict of Interest Guidance

8:05 AM

Minutes of the March 12, 2013, RAC Meeting

RAC Reviewers: Marie-Louise Hammarskjöld, M.D., Ph.D.
Margaret Mallino

Tab 2741 Minutes of March 12, 2013, RAC Meeting

8:10 AM

Discussion of Human Gene Transfer Protocol #1304-1230 titled: *A Phase 1 Ascending Dose Trial of the Safety and Tolerability of Toca 511, a Retroviral Replicating Vector, Administered Intravenously to Subjects Undergoing Subsequent Resection for Recurrent High Grade Glioma and Followed by Treatment with Toca FC, Extended-Release 5-FC*

PI: Steven N. Kalkanis, M.D., Henry Ford Hospital, Detroit, MI

Sponsor: Tocagen, Inc.
[Slide Presentation](#)

RAC Reviewers: Paula Cannon, Ph.D.
Hans-Peter Kiem, M.D., Ph.D.
Dawn Wooley, Ph.D.

TAB 2742 Protocol

Tuesday, June 11, 2013 (continued)

TAB 2743 OBA Summary
OBA Letter to PI In-Depth RAC Review and
Public Discussion
Reviews from Drs. Cannon, Kiem and Wooley
PI's Response

9:40 AM **Public Comments**

9:45 AM **Break**

10:00 AM **Discussion of Human Gene Transfer Protocol #1304-1219 titled: A Phase I/II, Open-Label Dose Escalation Study to Evaluate the Safety and Efficacy of Single Doses of TT-034 in Patients with Chronic Hepatitis C (CHC) Infection**

PI: Keyur Patel, M.D., Duke University Medical Center,
Durham, NC

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

Ad hoc Reviewers: Willis Maddrey, M.D., The University of Texas
Southwestern Medical Center, Dallas, TX
Scott Harper, Ph.D., Nationwide Children's Hospital,
Columbus, OH

RAC Reviewers: Yuman Fong, M.D.
Donald Kohn, M.D.
Laurie Zoloth, Ph.D.

TAB 2744 Protocol

TAB 2745 OBA Summary
OBA Letter to PI In-Depth RAC Review and
Public Discussion
Reviews from Drs. Fong, Harper, Kohn, Maddrey
and Zoloth
PI's Response

11:55 AM **Public Comments**

Tuesday, June 11, 2013 (continued)

12:00 PM **Gene Transfer Safety Assessment Board Report**

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.
 Joseph Pilewski, M.D.
 Marshall Strome, M.D., M.S.
 [Slide Presentation](#)

Tab 2746 Responses to M-I-C-1
 Protocol List
 Protocols Not Selected for In-Depth Review
 and Public Discussion
 Background Materials

Update on Human Gene Transfer Protocol #0510-738 titled: *A Phase I Dose Escalation Study of Intratumoral Herpes Simplex Virus-1 Mutant HSV1716 in Patients with Refractory Sarcoma or Neuroblastoma*

Presenter: Timothy Cripe, M.D., Ph.D., Nationwide Children's
 Hospital, Columbus, OH
 [Slide Presentation](#)

12:45 PM **Lunch**

1:30 PM **Discussion of NIH Protocol #0001-381 titled:**
Long-Term Follow-Up After Gene Therapy for Canavan Disease

Presenter: Paola Leone, Ph.D., University of Medicine & Dentistry
 of New Jersey, Stratford, NJ
 [Slide Presentation](#)

TAB 2747 Background Materials

2:00 PM **Discussion of Human Gene Transfer Protocol #1304-1226 titled:** *A Phase I Study of Guanylyl Cyclase C (GCC)-Encoding Replication-Deficient Human Type 5 Recombinant Adenovirus Vaccine (Ad5-hGCC-PADRE) in Stage I and II Colon Cancer Patients*

PI: Takami Sato, M.D., Ph.D., Thomas Jefferson University
 Hospital, Philadelphia, PA
 [Slide Presentation](#)

Tuesday, June 11, 2013 (continued)

RAC Reviewers: Yuman Fong, M.D.
David Ornelles, Ph.D.
Joseph Pilewski, M.D.

TAB 2748 Protocol

TAB 2749 OBA Summary
OBA Letter to PI In-Depth RAC Review and
Public Discussion
Reviews from Drs. Fong, Ornelles and Pilewski
PI's Response

3:30 PM **Public Comments**

3:35 PM **Break**

3:45 PM **Discussion of Human Gene Transfer Protocol #1304-1224 titled:**
*E10A (Endostatin Adenovirus) for the Treatment of Recurrent/Metastatic
Squamous Cell Carcinoma of the Head and Neck*

PI: George Yoo, M.D., Karmanos Cancer Center, Wayne State
University, Detroit, MI

Sponsor: Marsala Biotech, Inc.
[Slide Presentation](#)

RAC Reviewers: Walter Koch, Ph.D.
Marshall Strome, M.D., M.S.
Rebecca Dresser, J.D.

TAB 2750 Protocol

TAB 2751 OBA Summary
OBA Letter to PI In-Depth RAC Review and
Public Discussion
Reviews from Drs. Koch, Strome and Professor
Dresser
PI's Response

5:20 PM **Public Comments**

5:30 PM **Adjourn**

Wednesday, June 12, 2013

8:00 AM **Call to Order and Opening Remarks**

8:05 AM **Update on Stem Cell Gene Transfer for the Wiskott Aldrich Syndrome**

Presenter: Christoph Klein, M.D., Ph.D., Dr. von Haunersches
Kinderspital, University of Munich Medical Center,
Munich, Germany
[Slide Presentation](#)

8:30 AM **Certificates of Appreciation to Retiring RAC Members**

Presenter: Amy Patterson, M.D., Office of the Director, NIH,
Bethesda, MD

8:45 AM **Discussion of Human Gene Transfer Protocol #1304-1223 titled: *A Phase I/II, Non-Randomized, Multicenter, Open-Label Study of GIXCGD (Lentiviral Vector Transduced CD34+ Cells) in Patients with X-Linked Chronic Granulomatous Disease***

PI: Donald Kohn, M.D., University of California, Los Angeles,
Los Angeles, CA
[Slide Presentation](#)

RAC Reviewers: Hans-Peter Kiem, M.D., Ph.D.
Susan Ross, Ph.D.
Rebecca Dresser, J.D.

TAB 2752 Protocol

TAB 2753 OBA Summary
OBA Letter to PI In-Depth RAC Review and
Public Discussion
Reviews from Drs. Kiem, Ross and Professor Dresser
PI's Response

10:20 AM **Public Comments**

10:30 AM **Discussion of Human Gene Transfer Protocol #1304-1231 titled: *A Phase I Study of Intrathecal Administration of scAAV9/JeT-GAN for the Treatment of Giant Axonal Neuropathy***

PI: Carsten Bonnemann, M.D., National Institute of
Neurological Disorders and Stroke, NIH, Bethesda, MD

Sponsor: Hannah's Hope Fund
[Slide Presentation](#)

Wednesday, June 12, 2013 (continued)

RAC Reviewers: Saswati Chatterjee, Ph.D.
E. Antonio Chiocca, M.D., Ph.D.
Norman Fost, M.D., M.P.H.

TAB 2754 Protocol

TAB 2755 OBA Summary
OBA Letter to PI In-Depth RAC Review and
Public Discussion
Reviews from Drs. Chatterjee, Chiocca and Fost
PI's Response

12:10 PM **Public Comments**

12:15 PM **Lunch**

1:00 PM **Discussion of Human Gene Transfer Protocol #1303-1214 titled: A Phase I Randomized, Double-Blind, Placebo-Controlled Study of a Multi-Antigen DNA Vaccine Prime Delivered by In Vivo Electroporation, rVSV Booster Vaccine in HIV-Infected Patients Who Began Antiretroviral Therapy During Acute/Early Infection**

PI: Michael C. Sneller, M.D., National Institute of Allergy and Infectious Diseases, NIH, Bethesda, MD
[Slide Presentation](#)

RAC Reviewers: Andrew Badley, M.D.
Paula Cannon, Ph.D.
Laurie Zoloth, Ph.D.

TAB 2756 Protocol

TAB 2757 OBA Summary
OBA Letter to PI In-Depth RAC Review and
Public Discussion
Reviews from Drs. Badley, Cannon and Zoloth
PI's Response

3:00 PM **Public Comments**

3:15 PM **Adjourn**