

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

131ST MEETING

NIH/BUILDING 31, 6TH FLOOR
CONFERENCE ROOM 10

DECEMBER 4–5, 2012

AGENDA

Tuesday, December 4, 2012

2:00 PM

Call to Order and Opening Remarks

Yuman Fong, M.D., RAC Chair

Tab 2716

For Your Information
Notice of Meeting
Conflict of Interest Guidance

Minutes of the September 12, 2012, RAC Meeting

RAC Reviewers: Tianxi Cai, Sc.D.
Dawn Wooley, Ph.D.

Tab 2717

Minutes of September 12, 2012, RAC Meeting

2:05 PM

Discussion of Human Gene Transfer Protocol #1210-1189: *A Phase II, Randomized, Open Label Study of Ad-RTS-hIL-12 Monotherapy or Combination with Palifosfamide-Tris in Subjects with Recurrent/Metastatic Breast Cancer and Accessible Lesions*

PI: John Nemunaitis, M.D., Mary Crowley Research Centers - Medical City, Dallas, TX
Slide Presentation

Sponsor: ZIOPHARM Oncology, Inc.

RAC Reviewers: David Ornelles, Ph.D.
Joseph Pilewski, M.D.
Marcella Sarzotti-Kelsoe, Ph.D.

Tab 2718

Protocol

(continue) Tuesday, December 4, 2012

Tab 2719 OBA Summary
OBA Letter to PI In-Depth RAC Review and
Public Discussion
Reviews from Drs. Ornelles, Pilewski and
Sarzotti-Kelsoe
PI's Response

3:10 PM **Public Comment**

3:15 PM **Discussion of Human Gene Transfer Protocol #1210-1188: Phase I
Gene Transfer Clinical Trial for Spinal Muscular Atrophy Type 1
Delivering the Survival Motor Neuron Gene by Self-complementary AAV9**

PI: Jerry Mendell, M.D., The Research Institute at
Nationwide Children's Hospital, Columbus, OH
Slide Presentation

RAC Reviewers: Saswati Chatterjee, Ph.D.
E. Antonio Chiocca, M.D., Ph.D.
Laurie Zoloth, Ph.D.

Tab 2720 Protocol

Tab 2721 OBA Summary
OBA Letter to PI In-Depth RAC Review and
Public Discussion
Reviews from Drs. Chatterjee, Chiocca and Zoloth
PI's Response

4:45 PM **Public Comment**

4:50 PM **Break**

5:00 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, Ph.D., M.D.
Donald Kohn, M.D.
Joseph Pilewski, M.D.
Marshall Strome, M.D., M.S.

Slide Presentation

Tab 2722 Responses to M-I-C-1
Protocol List
Protocols Not Selected for In-Depth Review and
Public Discussion

(continue) Tuesday, December 4, 2012

Update Discussion on Protocol #0604-776 titled: *A Phase I Study of CD19 Chimeric Receptor Expressing T Lymphocytes in B-Cell Non-Hodgkins Lymphoma and Chronic Lymphocytic Leukemia*

PI: Carlos Ramos, M.D., Baylor College of Medicine,
Houston TX
Slide Presentation

5:40 PM **Adjourn**

Wednesday, December 5, 2012

8:30 AM **Call to Order and Opening Remarks**

Yuman Fong, M.D., RAC Chair

8:35 AM **Discussion of Human Gene Transfer Protocol #1208-1180:** *Local Modulation of Immune Receptor Function to Enhance Immune Responses to Dendritic Cell Vaccination in Subjects with Triple Negative Breast Cancer*

PI: Scott K. Pruitt, M.D., Ph.D., Duke University
Medical Center, Durham, NC
Slide Presentation

RAC Reviewers: Rebecca Dresser, J.D.
Marie-Louise Hammarskjöld, M.D., Ph.D.
Hans-Peter Kiem, Ph.D., M.D.

Tab 2723 Protocol

Tab 2724 OBA Summary
OBA Letter to PI In-Depth RAC Review and
Public Discussion
Reviews from Professor Dresser and
Drs. Hammarskjöld and Kiem
PI's Response

9:40 AM **Public Comment**

(continue) Wednesday, December 5, 2012

9:45 AM **Discussion of Human Gene Transfer Protocol #1210-1192: *A Study to Infuse ROR1-Specific Autologous T Cells for Patients with CLL***

PIs: William Wierda, M.D., Ph.D., The University of Texas MD Anderson Cancer Center, Houston, TX

And

Laurence Cooper, M.D., Ph.D., The University of Texas MD Anderson Cancer Center, Houston, TX

Slide Presentation

Sponsor: MD Anderson Cancer Center

RAC Reviewers: Paula Cannon, Ph.D.
Donald Kohn, M.D.
Susan Ross, Ph.D.

Tab 2725 Protocol

Tab 2726 OBA Summary
OBA Letter to PI In-Depth RAC Review and Public Discussion
Reviews from Drs. Cannon, Kohn and Ross
PI's Response

10:50 AM **Public Comment**

11:00 AM **Discussion of Human Gene Transfer Protocol # 1210-1190: *A Phase I Open-Label Study to Assess the Safety, Tolerability and Preliminary Efficacy of LX-1101(Lymfactin,™ VEGF-C Adenoviral Vector) in the Treatment of Patients with Secondary Lymphedema Associated with the Treatment of Breast Cancer***

PI: Stanley Rockson, M.D., Stanford University School of Medicine, Stanford, CA

Sponsor: Laurantis Pharma, Ltd.
Slide Presentation

RAC Reviewers: Walter Koch, M.D.
Margaret Mallino
Marshall Strome, M.D., M.S.
Laurie Zoloth, Ph.D.

Tab 2727 Protocol

(continue) Wednesday, December 5, 2012

Tab 2728 OBA Summary
OBA Letter to PI In-Depth RAC Review and
Public Discussion
Reviews from Drs. Koch, Strome, Zoloth and
Ms. Mallino
PI's Response

12:10 PM **Public Comment**

12:20 PM **LUNCH**

1:15 PM **Discussion of Human Gene Transfer Protocol #1209-1182:** *Autologous
Activated T-Cells Transduced with a 3rd Generation GD-2 Chimeric
Antigen Receptor and iCaspase9 Safety Switch Administered to Patients
with Relapsed or Refractory Neuroblastoma (GRAIN)*

PIs: Chrystal Louis, M.D., M.P.H., Texas Children's
Cancer Center, Baylor College of Medicine, Houston,
TX

and

Malcolm Brenner, M.B., Ph.D., Center for Cell and
Gene Therapy, Baylor College of Medicine, Houston,
TX

Slide Presentation

RAC Reviewers: Andrew Badley, M.D.
Yuman Fong, M.D.
Norman Fost, M.D., M.P.H.

Tab 2729 Protocol

Tab 2730 OBA Summary
OBA Letter to PI In-Depth RAC Review and
Public Discussion
Reviews from Drs. Badley, Fong and Fost
PI's Response

2:45 PM **Public Comment**

3:00 PM **Adjourn**