

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

126th Meeting

Rockville Hilton & Executive Meeting Center

September 13–14, 2011

Tuesday, September 13, 2011

10:30 AM **Call to Order and Opening Remarks**

Yuman Fong, M.D., RAC Chair

Tab 2672 For Your Information
 Notice of Meeting
 Conflict of Interest Guidance

10:35 AM **Minutes of RAC Meeting** June 7-9, 2011

RAC Reviewers: Walter Koch, Ph.D.
 Bernard Roizman, Sc.D.

10:40 AM **Discussion of Human Gene Transfer Protocol #1107-1119:** *An Open Label Phase I Study to Evaluate the Safety and Tolerability of GI-630, a Vaccine Consisting of Whole, Heat-Killed Recombinant Saccharomyces cerevisiae (yeast) Genetically Modified to Express Brachyury Protein in Adults with Metastatic Carcinoma*

Presenter: James L. Gulley, M.D., Ph.D., F.A.C.P., National Cancer Institute, National Institutes of Health, Bethesda, MD
Slide Presentation

Sponsor: GlobeImmune, Inc.

RAC Reviewers: Norman Fost, M.D., M.P.H.
 Marcella Sarzotti-Kelsoe, Ph.D.
 Marshall Strome, M.D., M.S.

Tab 2673 Protocol

Tab 2674 OBA Summary
 OBA Letter to PI on In-Depth RAC Review and Public Discussion
 Reviews from Drs. Fost, Sarzotti-Kelsoe, and Strome
 PI's Response

12:00 PM **Public Comment**

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12:05 PM **LUNCH**

1:00 PM **Discussion of Proposed Amendment to OBA Protocol #950:**
Gene Therapy for SCID-XI using a Self Inactivating (SIN) Gammaretroviral vector

Presenter: Sung-Yun Pai, M.D., Harvard Medical School and
 Children's Hospital Boston, Boston, MA
Slide Presentation

Sponsor: David A. Williams, M.D., Harvard Medical School and
 Children's Hospital Boston, Boston, MA

Ad hocs: Rebecca H. Buckley, M.D., Duke University Medical
 Center, Durham, NC, (*via teleconference*)
Slide Presentation

and

Richard O'Reilly, M.D., Memorial-Sloan Kettering Cancer
Center, New York, NY (*via teleconference*)

2:00 PM **Public Comment**

2:15 PM **Updates to the NIH Guidelines:** *Proposed Changes to Section III-E-1 -
Experiments with Defective Viral Genomes in Tissue Culture
and
Appendix B-Classification of Human Etiologic Agents on the Basis of
Hazard*

Presenters: Susan Ross, Ph.D. – **Slide Presentation**
 Joseph Kanabrocki, Ph.D. **Slide Presentation**

Tab 2675 Background Material

3:15 PM **Break**

3:30 PM **Discussion of Human Gene Transfer Protocol #1107-1120:**
*A Phase I Ascending Dose Trial of the Safety and Tolerability of Toca 511,
a Retroviral Replicating Vector, Administered to Subjects at the Time of
Resection for Recurrent High Grade Glioma and Followed by Treatment
with Toca FC, Extended-Release 5-FC*

PI: E. Antonio Chiocca, M.D., Ph.D., Ohio State University
 Medical Center, Columbus, OH

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Presenters: Dan Pertschuk, M.D., Tocagen, Inc., San Diego, CA
and
Doug Jolly, Ph.D., Tocagen, Inc., San Diego, CA
[Slide Presentation](#)

Sponsor: Tocagen Inc.

RAC Reviewers: Donald Kohn, M.D.
Susan Ross, Ph.D.
Jim Yankaskas, M.D., M.S.

Tab 2676 Protocol

Tab 2677 OBA Summary
OBA Letter to PI on In-Depth RAC Review and
Public Discussion
Reviews from Drs. Kohn, Ross, and Yankaskas
PI's Response

4:40 PM

Public Comment

5:00 PM

Discussion of IBC Review of Human Gene Transfer Protocols

Presenter: Jacqueline Corrigan-Curay, J.D., M.D., Acting Director,
Office of Biotechnology Activities, NIH, Bethesda, MD
[Slide Presentation](#)

5:30 PM

Public Comments

5:45 PM

Adjourn

Wednesday, September 14, 2011

8:30 AM **Welcome and Introductions**

8:40 AM **Gene Transfer Safety Assessment Board Report**

RAC Reviewers: Andrew Badley, M.D.
 E. Antonio Chiocca, M.D., Ph.D.
 Yuman Fong, M.D.
 Donald Kohn, M.D.
 Marshall Strome, M.D., M.S.
 James Yankaskas, M.D., M.S.

Tab 2678 Responses to M-I-C-1
 Protocol List
 Protocols Not Selected for In-Depth Review and
 Public Discussion
 Slide Presentation

9:00 AM **Discussion of Human Gene Transfer Protocol #1107-1117: *A Phase I/II Safety, Pharmacokinetic, and Pharmacodynamic Study of APS001F with Flucytosine and Maltose for the Treatment of Advanced and/or Metastatic Solid Tumors***

PI: John J. Nemunaitis, M.D., Mary Crowley Cancer Research Center,
 Dallas, TX (*via teleconference*)

Presenter: Barry Anderson M.D., Ph.D., Theradex, Princeton, NJ
 Slide Presentation

Sponsor: Anaeropharma Science, Inc.

RAC Reviewers: Andrew Badley, M.D.
 Michael Buchmeier, Ph.D., M.S.
 Anna Mastroianni, J.D.
 David Ornelles, Ph.D.

Tab 2679 Protocol

Tab 2680 OBA Summary
 OBA Letter to PI on In-Depth RAC Review and
 Public Discussion
 Reviews from Drs. Badley, Buchmeier, Ornelles,
 and Professor Mastroianni
 PI's Response

10:50 AM **Public Comment**

11:00 AM **BREAK**

Wednesday, September 14, 2011

11:10 AM

Discussion of Results of OBA Protocol #0610-809: *A Phase 1/2 Randomized, Double-Blinded, Placebo-Controlled Dose Escalation trial of Intracoronary Administration of MYDICAR® (AAVI/SERCA2a) in Subjects with Heart Failure*

Presenter: Dr. Krisztina Zsebo, Ph.D., Chief Executive Officer and President, Celladon Corporation, La Jolla, CA
Slide Presentation

Tab 2681 Background Material

12:05 PM

Public Comment

12:15 PM

Adjourn