

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

133<sup>RD</sup> MEETING

NIH/ROCKLEDGE II  
CONFERENCE ROOM 9100

MARCH 12, 2013

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8:30 AM	<b>Call to Order and Opening Remarks</b>
	Yuman Fong, M.D., RAC Chair
	Tab 2731                      For Your Information Notice of Meeting Conflict of Interest Guidance
8:35 AM	<b>Update on Human Gene Transfer Protocol #0510-731 titled:</b> <i>Open-Label, Dose-Escalation Study Evaluating the Safety of a Single Administration of an Adenoviral Vector Encoding Human Aquaporin-1 to One Parotid Salivary Gland in Individuals with Irradiation Induced Parotid Salivary Hypofunction</i>
	PI:                                      Bruce Baum, D.M.D., Ph.D., Warren Grant Magnuson Clinical Center, NIH, Bethesda, MD <b>Slide Presentation</b>
	Tab 2732                      Background Materials
9:05 AM	<b>Discussion of Human Gene Transfer Protocol #1301-1200 titled:</b> <i>Phase I/II Gene Transfer Clinical Trial for LGMD2D (Alpha-sarcoglycan Deficiency) Using SC rAAV8.tMCK.hSGCA</i>
	PI:                                      Jerry Mendell, M.D., The Research Institute, Nationwide Children's Hospital, Columbus, OH <b>Slide Presentation</b>
	RAC Reviewers:                      Saswati Chatterjee, Ph.D. Yuman Fong, M.D. Norman Fost, M.D., M.P.H.
	Tab 2733                      Protocol

*(continued) Tuesday, March 12, 2013*

Tab 2734                    OBA Summary  
OBA Letter to PI In-Depth RAC Review and  
Public Discussion  
Reviews from Drs. Chatterjee, Fong and Fost  
PI's Response

10:25 AM                    **Public Comment**

10:30 AM                    **Break**

10:45 AM                    **Discussion of Human Gene Transfer Protocol #1301-1202 titled:** *Phase I Study of Safety and Immunogenicity of ADU-623, a Live Attenuated Listeria monocytogenes Vaccine (Lm ΔactA/ΔinlB) Expressing EGFRvIII-NY-ESO-1, in Patients with Treated and Recurrent WHO Grade III/IV Astrocytomas*

PI:                            Marka Crittenden, M.D., Ph.D., Providence Cancer  
Center, Portland, OR  
**Slide Presentation**

RAC Reviewers:            E. Antonio Chiocca, M.D., Ph.D.  
Rebecca Dresser, J.D.  
Joseph Pilweski, M.D.  
Marshall Strome, M.D., M.S.

Tab 2735                    Protocol

Tab 2736                    OBA Summary  
OBA Letter to PI In-Depth RAC Review and  
Public Discussion  
Reviews from Drs. Chiocca, Pilewski, Strome and  
Professor Dresser  
PI's Response

12:15 PM                    **Public Comments**

12:20 PM                    **Minutes of the December 4-5, 2012, RAC Meeting**

RAC Reviewers:            Andrew Badley, M.D.  
Hans-Peter Kiem, M.D., Ph.D.

Tab 2737                    Minutes of December 4-5, 2012, RAC Meeting

12:25 PM                    **Updates from the Office of Biotechnology Activities**

Presenter:                    Jacqueline Corrigan-Curay, J.D., M.D.  
Office of Biotechnology Activities, NIH  
**Slide Presentation**

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12:30 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.

Tab 2738 Responses to M-I-C-1  
Protocol List  
Protocols Not Selected for In-Depth Review and  
Public Discussion  
Background Materials  
**Slide Presentation**

12:35 PM

**LUNCH**

1:15 PM

**Update on 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**

Presenter: Daniel Pertschuk, M.D., Tocagen, Inc.  
San Diego, CA

2:00 PM

**Public Comments**

2:10 PM

**Biosafety Considerations for Research Involving the Full-Length cDNA Cloning of Single-Strand, Non-Segmented, Positive Sense Risk Group 4 RNA Viruses (e.g. Flaviviruses)**

**Reverse Genetics of Tick-borne Flaviviruses: Glycoprotein Contributions to Pathogenesis**

Presenter: Bevan Sawatsky, Ph.D., University of Texas  
Medical Branch, Galveston, TX

**Recommendations from the Biosafety Working Group**

Presenter: Paula Cannon, Ph.D., RAC  
**Slide Presentation**

*Adhoc:* Stuart Nichol, Ph.D., Centers for Disease Control  
and Prevention, Atlanta, GA

Tab 2739 Background Materials

3:15 PM

**Public Comments**

3:20 PM

**Adjourn**