



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

**138<sup>th</sup> MEETING**

**June 10 - 11, 2014**

**Bethesda, MD**

**Building 35, Conference Room 620/630**

**AGENDA**

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***Day Two***

***Wednesday, June 11, 2014***

8:30 AM

**Call to Order and Opening Remarks**

**Donald Kohn, M.D., RAC Chair**

Tab 2790 For Your Information  
Notice of Meeting  
Conflict of Interest Guidance

8:35 AM

**Minutes**

Tab 2791 Minutes of the March 12, 2014, RAC Meeting

RAC Reviewers: Hans Peter Kiem, M.D., Ph.D.  
William Curry, M.D.

8:45 AM

**Discussion on Human Gene Transfer Protocol #1404-1306 titled: *An Open-label Dose Escalation Study of an Adeno-associated Virus Vector (scAAV2-P1ND4v2) for Gene Therapy of Leber's Hereditary Optic Neuropathy (LHON) caused by the G11778A mutation in mitochondrial DNA***

PI: John Guy, M.D., University of Miami Bascom Palmer Eye Institute

RAC Reviewers: Saswati Chatterjee, Ph.D.  
Rebecca Dresser, J.D.  
Marcella Sarzotti-Kelsoe, Ph.D.

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Tab 2792 Protocol

Tab 2793 OBA Summary  
OBA Letter to PI on In-Depth RAC Review and  
Public Discussion  
Reviews from Drs. Chatterjee, Whitley, and Professor  
Dresser  
PI's Response

10:10 AM **Public Comments**

10:15 AM **Break**

10:30 AM **Gene Transfer Safety Assessment Board Report**

RAC Reviewers: Michael Atkins, M.D.  
William Curry, M.D.  
Donald Kohn, M.D.  
Hans-Peter Kiem, M.D., Ph.D.  
Joseph Pilewski, M.D.  
Michel Sadelain, M.D., Ph.D.  
Marshall Strome, M.D., M.S.  
Richard Whitley, M.D.

Tab 2798 Responses to M-I-C-1  
Protocol List  
Protocols Not Selected for In-Depth Review and  
Public Discussion

10:45 AM **Discussion on Human Gene Transfer Protocol #1404-1307 titled: *A Phase I Clinical Trial of Cyclophosphamide Followed by Intravenous and Intraperitoneal Infusion of Autologous T cells Genetically Engineered to Secrete IL-12 and to Target the MUC16ecto Antigen in Patients with Recurrent MUC16ecto+ Epithelial Ovarian, Fallopian Tube or Primary Peritoneal Cancer***

PIs: Roisin O’Cearbhaill, M.B., B.Ch., B.A.O., Memorial Sloan-Kettering Cancer Center, New York, NY

Renier J. Brentjens, M.D., Ph.D. Memorial Sloan-Kettering Cancer Center, New York, NY

RAC Reviewers: Michael Atkins, M.D.  
Norman Fost, M.D., M.P.H  
Donald Kohn, M.D.

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Tab 2795 OBA Summary  
OBA Letter to PI on In-Depth RAC Review and  
Public Discussion  
Reviews from Drs. Atkins, Fost, and Kohn  
PI's Response

12:00 PM **Public Comments**

12:10 PM **Discussion of the IOM Report: *Oversight and Review of Clinical Gene Transfer Protocols Assessing the Role of the Recombinant DNA Advisory Committee***

Jacqueline Corrigan-Curay, J.D., M.D., Office of Biotechnology  
Activities, NIH

12:40 PM **Public Comments**

12:45 PM **Certificates of Appreciation to Retiring RAC Members**

Amy Patterson, M.D.

1:00 PM **Lunch**

1:45 PM **Housing of Non-Human Primates in BL4 Laboratories under the *NIH Guidelines for Research Involving Recombinant or Synthetic Nucleic Acid Molecules***

Jacqueline Corrigan-Curay, J.D., M.D.

Dee Zimmerman, B.A., Environmental Health and Safety,  
University of Texas Medical Branch

3:20 PM **Public Comment**

3:30 PM **Adjourn**