

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

128TH MEETING

NIH/BUILDING 31C, 6TH FLOOR
CONFERENCE ROOM 6

MARCH 7-8, 2012

AGENDA

Wednesday, March 7, 2012

- 3:30 PM **Call to Order and Opening Remarks**
- Yuman Fong, M.D., RAC Chair
- Tab 2694 For Your Information
 Notice of Meeting
 Conflict of Interest Guidance
- 3:35 PM **Discussion of Human Gene Protocol #1201-1144** titled: *AAV8-mediated Low Density Lipoprotein Receptor Gene Replacement in Subjects with Homozygous Familial Hypercholesterolemia*
- PI: Patrick Moriarty, M.D., The University of Kansas
 Medical Center, Kansas City, KS
- Sponsor: Daniel J. Rader, M.D., University of Pennsylvania,
 Philadelphia, PA
 Slide Presentation
- RAC Reviewers: Saswati Chatterjee, Ph.D.
 Hans-Peter Kiem, M.D., Ph.D.
 Anna Mastroianni, J.D., M.P.H.
- Tab 2696 Protocol
- Tab 2697 OBA Summary
 OBA Letter to PI on In-Depth RAC Review and
 Public Discussion
 Reviews from Drs. Chatterjee, Kiem and
 Professor Mastroianni
 PI's Response

(continued) Wednesday, March 7, 2012

4:35 PM **Break**

4:50 PM **Public Comment**

5:05 PM **Minutes**

Tab 2695 Minutes of RAC Meeting, December 13-14, 2011

RAC Reviewers: Andrew Badley, M.D.
Susan Ross, Ph.D.

5:10 PM **Gene Transfer Safety Assessment Board Report**

Tab 2698 Responses to M-1-C-1
Protocol List
Protocols Not Selected for In-Depth Review and Public
Discussion

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.
Marshall Strome, M.D., M.S.
James Yankaskas, M.D., M.S.
Slide Presentation

5:30 PM **ADJOURNMENT**

Thursday, March 8, 2012

8:30 AM **Welcome and Introductions**

8:35 AM **Discussion of Results from Protocol # 977** titled: *Direct CNS
Administration of a Replication Deficient Adeno-Associated Virus Gene
Transfer Vector Serotype rh.10 Expressing the Human CLN2 cDNA to
Children with Late Infantile Neuronal Ceroid Lipofuscinosis*

Presenter: Ronald Crystal, M.D., Weill Cornell Medical College,
New York Presbyterian Hospital, New York, NY
Slide Presentation

9:10 AM **Public Comments**

(continued) Thursday, March 8, 2012

RAC Reviewers: Norman Fost, M.D., M.P.H.
Marie-Louise Hammarskjöld, M.D., Ph.D.
Donald Kohn, M.D.

Tab 2701 Protocol

Tab 2702 OBA Summary
OBA Letter to PI on In-Depth RAC Review and
Public Discussion
Review from Drs. Berns, Fost, Hammarskjöld and Kohn
PI's Response

12:30 PM **Public Comment**

12:45 PM **Lunch**

1:30 PM **Update and Discussion on Regarding IBC Review of Low Risk Protocols**

Presenter: Jacqueline Corrigan-Curay, J.D., M.D.
Office of Biotechnology Activities, National Institutes of
Health, Bethesda, MD
Slide Presentation

2:10 PM **Public Comment**

2:30 PM **Discussion of Results on OBA Protocol # 0704-849** titled: *A Phase I Study
Evaluating the Use of Allodepleted T Cells Transduced with Inducible
Caspase 9 Suicide Gene After Haploidentical Stem Cell Transplantation*

Presenters: Helen Heslop, M.D., Baylor College of Medicine,
Houston, TX
and
Cliona Rooney, Ph.D., Baylor College of Medicine,
Houston, TX
Slide Presentation 1 -- Slide Presentation 2

Tab 2703 Background Material

3:15 PM **Public Comment**

3:30 PM **ADJOURNMENT**