

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 **Minutes**
- Tab 2695** **Minutes of RAC Meeting, December 13-14, 2011**
- RAC Reviewers:** Andrew Badley, M.D.
 Susan Ross, Ph.D.
- 3:40 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, MD, University of Pennsylvania,
 Philadelphia, PA
- RAC Reviewers:** Saswati Chatterjee, Ph.D.
 Hans-Peter Kiem, M.D., Ph.D.
 Anna Mastroianni, J.D., M.P.H.
- Tab 2696** Protocol

(continued) Wednesday, March 7, 2012

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

4:40 PM **Break**

4:50 PM **Public Comment**

5:05 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.

5:30 PM **Adjourn**

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: Ron Crystal, M.D., Weill Cornell Medical College,
New York Presbyterian Hospital, NY

9:10 AM **Public Comments**

(continued) Thursday, March 8, 2012

9:20 AM

Discussion of Human Gene Protocol # 1201-1145 titled: *Phase I Study of Direct Administration of AdVEGF-All6A+, a Replication Deficient Adenovirus Vector Expressing a cDNA/Genomic Hybrid of Human Vascular Endothelia Growth Factor to the Ischemic Myocardium of Individuals with Diffuse Coronary Artery Disease via Minimally Invasive Surgery*

PI: Todd Rosengart, M.D., Stony Brook University Medical Center and Weill Cornell Medical College, NY

Sponsor: Ron Crystal, M.D.

RAC Reviewers: Rebecca Dresser, J.D.
Walter Koch, Ph.D.
David Ornelles, Ph.D.

Tab 2699 Protocol

Tab 2700 OBA Summary
OBA Letter to PI on In-Depth RAC Review and Public Discussion
Reviews from Dr. Ornelles, Koch and Professor Dresser
PI's Response

10:30 AM

Break

10:45 AM

Public Comment

11:00 AM

Discussion of Human Gene Protocol #1201-1143 titled: *A Phase 2, Randomized, Active Control, Open-Label, Multi-Center Study to Evaluate the Safety and Efficacy of EPODURE for Sustained Treatment of Anemia in Hemodialysis Patients*

PI: Geoffrey Block, M.D., Denver Nephrology Research, Denver, CO

Sponsor: Medgenics Medical Israel

Ad hoc: Jeffrey Berns, M.D., University of Pennsylvania, Philadelphia, PA

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

(continued) Thursday, March 8, 2012

	Tab 2701	Protocol
	Tab 2702	OBA Summary OBA Letter to PI on In-Depth RAC Review and Public Discussion Review from Drs. 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
2:10 PM	Public Comment	
2:30 PM	Discussion of Results on OBA Protocol # 0704-849	<i>titled: A Phase I Study Evaluating the Use of Allodepleted T Cells Transduced with Inducible Caspase 9 Suicide Gene After Haploidentical Stem Cell Transplantation</i>
	Presenter:	Helen Heslop, M.D., Baylor College of Medicine, Houston, TX <i>and</i> Cliona Rooney, Ph.D., Baylor College of Medicine, Houston, TX
	Tab 2703	Background Material
3:15 PM	Public Comment	
3:30 PM	Adjourn	

**Times and Order of Presentations Subject to Change*