



## RECOMBINANT DNA ADVISORY COMMITTEE MEETING

Office of Science Policy - National Institutes of Health  
Porter Neuroscience Research Center (Building 35)  
NIH Campus, Bethesda, MD  
146<sup>th</sup> RAC Meeting  
AGENDA – June 21-22, 2016



### AGENDA – June 21, 2016

**8:30 AM**      **Call to Order and Opening Remarks**  
Richard Whitley, MD – Acting RAC Chair

Tab 2873:      For Your Information  
Notice of Meeting  
Conflict of Interest Guidance

**8:40 AM**      **March 8-9 2016 Meeting Minutes**  
RAC Reviewers: Patrick Hearing, PhD  
Douglas McCarty, PhD

Tab 2874: Minutes of the March 8-9, 2016 RAC Meeting

**8:45 AM**      **Discussion on Human Gene Transfer Protocol #1601-1502 titled: *A Phase I Safety Study of Adoptive Cellular Therapy Using Autologous T Cells Transduced with Lentivirus to Express a CD33 Specific Chimeric Antigen Receptor in Patients with Relapsed or Refractory CD33-Positive Acute Myeloid Leukemia***

Presenters:      William Wierda, MD  
Mike Rytting, MD (Via Phone)

RAC Reviewers:      Joseph Pilewski, MD  
Lainie Ross, MD, PhD  
Richard Whitley, MD

Tab 2875:      Protocol

Tab 2876:      NIH Summary  
NIH Letter to PI on In-depth RAC Review and Public Discussion  
Reviews from Drs. Pilewski, Ross, and Whitley

PI's Response

**10:15 AM**      **Public Comments**

**10:20 AM**      **BREAK**

**10:40 AM**      **Discussion on Human Gene Transfer Protocol #1604-1524 titled: *Phase I Trial of Autologous T Cells Engineered to Express NYESO-1 TCR and Gene Edited to Eliminate Endogenous TCR and PD-1***

Presenters: Edward Stadtmauer, MD  
Carl June, MD  
J. Joseph Melenhorst, PhD

Participant: Barbara Vance, PhD

RAC Reviewers: Michael Atkins, MD  
Paula Cannon, PhD  
Mildred Cho, PhD

Tab 2877: Protocol

Tab 2878: NIH Summary  
NIH Letter to PI on In-depth RAC Review and Public Discussion  
Reviews from Drs. Atkins, Cannon, and Cho

PI's Response

**12:10 PM Public Comments**

**12:30 PM LUNCH**

**1:30 PM Updates in manufacturing of CTL019**

Presenter: Carl June, MD  
Participant: J. Joseph Melenhorst, PhD

Tab 2879: Background Materials

**3:00 PM Public Comments**

**3:05 PM BREAK**

**3:15 PM Acknowledgment of Service of Departing RAC Members**

Francis S. Collins, MD, PhD, NIH Director

**3:35 PM Discussion on Human Gene Transfer Protocol #1604-1520 titled: *A Phase I/II, Open-Label Safety and Dose-Finding Study of Adeno-Associated Virus (AAV) Serotype 8 (AAV8)-Mediated Gene Transfer of Human Ornithine Transcarbamylase (OTC) in Adults with Late-Onset OTC Deficiency***

Presenter: Cary O. Harding, MD

Ad Hoc Expert: Ada Hamosh, MD, MPH

RAC Reviewers: Lainie Ross, MD, PhD  
Richard Whitley, MD

Dawn Wooley, PhD

Tab 2880: Protocol

Tab 2881: NIH Summary  
NIH Letter to PI on In-depth RAC Review and Public Discussion  
Reviews from Drs. Ross, Whitley, and Wooley

PI's Response

**4:55 PM Public Comments**

**5:00 PM ADJOURN**

**AGENDA – June 22, 2016**

**8:30 AM Call to Order and Opening Remarks**  
Richard Whitley, MD– Acting RAC Chair

**8:35 AM Gene Transfer Safety Assessment Board Update**

Chair: Richard Whitley, MD

Tab 2882: Responses to M-I-C-1  
Protocol List  
Protocols Not Selected for In-Depth Review and Public  
Discussion

**9:15 AM Discussion on Human Gene Transfer Protocol #1603-1512 titled: *Phase I Study of Oncolytic Polio/Rhinovirus Recombinant against Recurrent Malignant Glioma in Children***

Presenter: Matthias Gromeier, MD

Acting Chair: William Curry, MD

RAC Reviewers: Mildred Cho, PhD  
William Curry, MD  
Howard Kaufman, MD

Tab 2883: Protocol

Tab 2884: NIH Summary  
NIH Letter to PI on In-depth RAC Review and Public Discussion  
Reviews from Drs. Cho, Curry, and Kaufman

PI's Response

**10:45 AM Public Comments**

**10:50 AM BREAK**

**11:05 AM Discussion on Human Gene Transfer Protocol #1603-1509 titled: *Pediatric and Young Adult Leukemia Adoptive Therapy (PLAT)-03: A Pilot Feasibility and Safety Study of CD19t T-APC Vaccination Following CAR T Cell Immunotherapy for CD19+ Leukemia***

Presenters: Colleen Annesley, MD  
Rebecca Gardner, MD  
Michael Jensen, MD

Acting Chair: William Curry, MD

RAC Reviewers: Kevin Donahue, MD  
Laurie Zoloth, PhD

Ad Hoc Reviewer: Dean Lee, MD, PhD

Tab 2885: Protocol

Tab 2886: NIH Summary  
NIH Letter to PI on In-depth RAC Review and Public Discussion  
Reviews from Drs. Donahue, Zoloth and Lee

PI's Response

**12:40 PM Public Comments**

**12:45 PM ADJOURN**