



RECOMBINANT DNA ADVISORY COMMITTEE MEETING

Office of Science Policy - National Institutes of Health
Porter Neuroscience Research Center (Building 35)
NIH Campus, Bethesda, MD
145th RAC Meeting
AGENDA – March 8-9, 2016



AGENDA – MARCH 8, 2016

9:00 AM Call to Order and Opening Remarks

Hans-Peter Kiem, MD, PhD – RAC Chair

Tab 2861: For Your Information
Notice of Meeting
Conflict of Interest Guidance

9:10 AM December 4, 2015 Meeting Minutes

RAC Reviewers: Paula Cannon, PhD
Laurie Zoloth, PhD

Tab 2862: Minutes of the December 4, 2015 RAC Meeting

9:15 AM Discussion on Human Gene Transfer Protocol #1601-1495 titled: *A Phase 1 Dose-Escalation Study of the Safety of AAV8-VRC07 (VRC-HIV AA V070-00-GT) Recombinant AAV Vector Expressing VRC07 HIV-1 Neutralizing Antibody in Antiretroviral-Treated, HIV-1 Infected Adults with Controlled Viremia*

Presenters: Julie Ledgerwood, DO
Jason Gall, PhD
Barney Graham, MD, PhD

RAC Reviewers: Patrick Hearing, PhD
Hans-Peter Kiem, MD, PhD
Lainie Ross, MD, PhD

Tab 2863: Protocol

Tab 2864: NIH Summary
NIH Letter to PI on In-depth RAC Review and Public Discussion
Reviews from Drs. Hearing, Kiem, and Ross
PI's Response

10:45 AM Public Comments

10:50 AM BREAK

11:10 AM **Discussion on Human Gene Transfer Protocol #1601-1494 titled: *Efficacy of Local Delivery of MicroRNAs in Promoting Osteogenesis and Modulating Local Inflammation: A Pilot Clinical Trial Using the Tooth Socket Model***

Presenters: Gustavo Avila-Ortiz, DDS, PhD
 Brad Amendt, PhD
 Liu Hong, MD, PhD

RAC Reviewers: Kevin Donahue, MD
 Richard Whitley, MD
 Mildred Cho, PhD

Ad hoc Reviewer: Ilias Alevizos, DMD, MMSc

Tab 2865: Protocol

Tab 2866: NIH Summary
 NIH Letter to PI on In-depth RAC Review and Public Discussion
 Reviews from Drs. Donahue, Whitley, Cho, and Alevizos
 PI's Response

12:40 PM **Public Comments**

12:45 PM **LUNCH**

1:45 PM **Discussion Regarding the Deliberate Transfer of Chloramphenicol Resistance to *Rickettsia felis*, *R. rickettsii*, and *R. typhi***

Co-Chairs: Richard Whitley, MD
 Dawn Wooley, PhD

Presentation by Dawn Wooley, PhD

Tab 2867: Background Materials

3:15 PM **Public Comments**

3:20 PM **BREAK**

3:40 PM **Gene Transfer Safety Assessment Board Update**

Chair: Hans-Peter Kiem, MD, PhD

Tab 2868: Responses to M-I-C-1
 Protocol List
 Protocols Not Selected for In-Depth Review and Public
 Discussion

4:15 PM **ADJOURN**

AGENDA – MARCH 9, 2016

9:00 AM Call to Order and Opening Remarks

Hans-Peter Kiem, MD, PhD – RAC Chair

9:05 AM Discussion on Human Gene Transfer Protocol #1601-1492 titled: *A Phase I Dose Escalation Study Evaluating Safety and Feasibility of BPX-701 in Patients with Relapsed or Refractory Myeloid Neoplasms*

Presenters: Rachel Cook, MD

Sponsor: Bellicum Pharmaceuticals

RAC Reviewers: Michael Atkins, MD
Angelica Hardison, MBA
Dawn Wooley, PhD

Tab 2869: Protocol

Tab 2870: NIH Summary
NIH Letter to PI on In-depth RAC Review and Public Discussion
Reviews from Drs. Atkins, Wooley, and Ms. Hardison

PI's Response

10:35 AM Public Comments

10:40 AM BREAK

10:50 AM Discussion on Human Gene Transfer Protocol #1601-1493 titled: *A Feasibility and Safety Study of PSCA-Specific Chimeric Antigen Receptor Engineered T Cells (BPX-601) in Subjects with Non-Resectable Pancreatic Cancer*

Presenters: Carlos Becerra, MD

RAC Reviewers: Michael Atkins, MD
Howard Kaufman, MD
Laurie Zoloth, PhD

Tab 2871: Protocol

Tab 2872: NIH Summary
NIH Letter to PI on In-depth RAC Review and Public Discussion
Reviews from Drs. Atkins, Kaufman, and Zoloth

PI's Response

12:20 PM Public Comments

12:25 PM LUNCH

**1:00 PM FDA Presentation: Pilot Project to Develop a Clinical and CMC (Chemical
Manufacturing and Controls) Database to Examine Safety in Trials Using CAR (Chimeric
Antigen Receptor) T cells**

2:45 PM Public Comments

3:00 PM ADJOURN