



RECOMBINANT DNA ADVISORY COMMITTEE MEETING

Office of Science Policy - National Institutes of Health
Porter Neuroscience Research Center (Building 35)
NIH Campus, Bethesda, MD
147th RAC Meeting
FINAL AGENDA – December 14, 2016



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- 1:00 PM** **Call to Order and Opening Remarks**
Richard Whitley, MD – RAC Chair
- Tab 2887: For Your Information
Notice of Meeting
Conflict of Interest Guidance
- 1:10 PM** **June 21-22, 2016 Meeting Minutes**
- RAC Reviewers: Patrick Hearing, PhD
Joseph Pilewski, MD
- Tab 2888: Minutes of the June 21-22, 2016 RAC Meeting
- 1:20 PM** **Director's updates**
Jessica Tucker, PhD
Director, Biosafety, Biosecurity and Emerging Biotechnology Policy Division
Office of Science Policy
- Tab 2897: Protocol List
Protocols Not Selected for In-Depth Review and Public
Discussion
- 1:35 PM** **Gene Transfer Safety Assessment Board Update**
- Presenter: Richard Whitley, MD
- Tab 2889

2:00 PM **Presentation of Human Gene Transfer Protocol #1610-1547 titled:** *Phase I, open label dose-escalation study to evaluate the safety, expansion, persistence and clinical activity of a single dose of UCART123 (allogeneic engineered T-cells expressing anti-CD123 chimeric antigen receptor), administered in patients with Blastic Plasmacytoid Dendritic Cell Neoplasm (BPDCN).*

Tab 2890: Protocol

Tab 2891: NIH Summary
NIH Letter to PI on In-depth RAC Review and Public Discussion

Presenters: David Sourdive, PhD – for CMC section (Cellectis)
Julianne Smith, PhD – for TALEN® technology, genetic stability studies and non-clinical development (Cellectis)
Naveen Pemmaraju, MD – for UCART123 BPDCN (MD Anderson Cancer Center)

Presentation of Human Gene Transfer Protocol #1610-1548 titled: *Phase I, open label dose-escalation and expansion study to evaluate the safety, proliferation, persistence and clinical activity of a single dose of UCART123 (allogeneic engineered T-cells expressing anti-CD123 chimeric antigen receptor), administered in patients with Relapsed/Refractory Acute Myeloid Leukemia, and patients with high-risk newly diagnosed Acute Myeloid Leukemia*

Tab 2892: Protocol

Tab 2893: NIH Summary
NIH Letter to PI on In-depth RAC Review and Public Discussion

Presenters: David Sourdive, PhD – for CMC section (Cellectis)
Julianne Smith, PhD – for TALEN® technology, genetic stability studies and non-clinical development (Cellectis)
Gail Roboz, MD – for UCART123 AML clinical trial (Weill Cornell)

Presentation of Human Gene Transfer Protocol #1610-1549 titled: *Phase I, open label, dose-escalation study to evaluate the safety, expansion and persistence of a single dose of UCART19 (allogeneic engineered T-cells expressing anti-CD19 chimeric antigen receptor), administered intravenously in patients with relapsed or refractory CD19 positive B-cell acute lymphoblastic leukemia (B-ALL)*

Tab 2894: Protocol

Tab 2895: NIH Summary
NIH Letter to PI on In-depth RAC Review and Public Discussion

Presenters: David Sourdive, PhD – for CMC section (Collectis)
Julianne Smith, PhD – for TALEN® technology, genetic stability studies and non-clinical development (Collectis)
Nitin Jain, MD – for UCART19 (MD Anderson Cancer Center)

3:30 PM Break

3:45 PM Discussion of Protocols

RAC Reviewers: Michael Atkins, MD
Mildred Cho, PhD
David DiGiusto, PhD
Kevin Donahue, MD
Matthew Porteus, MD, PhD

Tab 2896A: Dr. Cho's reviews for 1610-1547, 1548, and 1549

Tab 2896B: Dr. DiGiusto's review for 1610-1547, 1548, and 1549

Tab 2896C: Dr. Porteus' review for 1610-1547, 1548, and 1549

Tab 2896D: Dr. Donahue's reviews for 1610-1547, and 1548

Tab 2896E: Dr. Atkins' review for 1610-1549

Tab 2896F: Sponsor/PI Responses

5:30 PM Public Comments

6:00 PM ADJOURN