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 (Cellectis)  
Julianne Smith, PhD – for TALEN® technology, genetic stability  
    studies and non-clinical development (Cellectis)  
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