



T Cell Immunotherapy – Optimizing Trial Design
Office of Biotechnology Activities
Office of Science Policy
National Institutes of Health (NIH)
Bethesda, MD
September 10-11, 2013



8:30 AM **Welcome and Introductory Remarks**

Co-Chairs: Donald Kohn, M.D., University of California, Los Angeles
Hans-Peter Kiem, M.D., Ph.D. Fred Hutchinson Cancer
Research Center

*Session I: Current Status of Cancer Immunotherapy:
Trials, Results, and Challenges*

8:40 AM **Update on Current Approaches and Trials**
Slide Presentations:

[Introductory Briefing](#) - Donald Kohn, M.D.

[Renier Brentjens, M.D., Ph.D.](#)

*Memorial Sloan-Kettering Cancer
Center*

[Michael Jensen, M.D.](#)

University of Washington

[Laurence Cooper, M.D., Ph.D.](#)

MD Anderson Cancer Center

[Stephen Forman, M.D.](#)

City of Hope

[Antoni Ribas, M.D., Ph.D.](#)

University of California, Los Angeles

[Carl June, M.D.](#)

University of Pennsylvania

[Helen Heslop, M.D.](#)

Baylor College of Medicine

[Daniel Powell, Ph.D.](#)

University of Pennsylvania

[Stephen Gottschalk, M.D.](#)

Baylor College of Medicine

[Richard Junghans, M.D., Ph.D.](#)

Roger Williams Medical Center

[Philip Greenberg, M.D.](#)

*Fred Hutchinson Cancer Research
Center*

[Crystal Mackall, M.D.](#)

National Cancer Institute (NCI), NIH

[Brian Till, M.D.](#)

*Fred Hutchinson Cancer Research
Center*

[Steven Rosenberg, M.D., Ph.D.](#)

NCI, NIH

Session II: Review of Strategies to Promote the Persistence of Cells

11:20 AM **BREAK**

11:30 AM **Host Preparation**

Steven Rosenberg, M.D., Ph.D. – [Slide Presentation](#)

Discussion Session

1. What lymphodepletion agents are being used?
2. Are there data to compare across protocols?
3. Is it always needed?

12:15 PM LUNCH (On Your Own)

1:15 PM **Design of Chimeric Antigen Receptors**

Michel Sadelain, M.D., Ph.D. – [Slide Presentation](#)
Memorial Sloan-Kettering Cancer Center

Design of T Cell Receptors

Antoni Ribas, M.D., Ph.D. – [Slide Presentation](#)

1:45 PM **Discussion Session**

1. What data do we have comparing co-stimulatory domains in second and third generation CARs?
2. Cytokine support – What is the optimum approach?
3. How can combinatorial design be used to test relative efficacy and avoid antigen escape?

2:30 PM **Product Related Factors:**

Gene Delivery Considerations

Laurence Cooper, M.D., Ph.D. – [Slide Presentation](#)

Optimizing the T Cell Product

Michael Jensen, M.D. – [Slide Presentation](#)

Potential Use of Stem Cells

David Baltimore, Ph.D. – [Slide Presentation](#)
California Institute of Technology

3:15 PM

Discussion Session

1. How much variation is there across protocols in active product?
2. Is there an optimal protocol for T cell expansion?
3. Does the mix of T cells make a difference – CD4/CD8 vs. T central memory cells etc?
4. What approaches might use human stem cells as a target?
5. For integrating vectors, do we have data comparing lentiviral vectors, murine retroviral vectors, transposons regarding transduction efficiency?

3:45 PM

BREAK

Session III: Dilemmas and Challenges: Approaches and Assessments

4:00 PM

Target Selection

Steven Rosenberg, M.D., Ph.D. – [Slide Presentation](#)

Carl June, M.D. – [Slide Presentation](#)

1. What type of screening needs to be done for new target antigens?
2. Are murine models valuable/necessary?
3. Are large animal models needed for novel targets?

4:45 PM

Dosing Strategies – Goals and Options

Renier Brentjens, M.D., Ph.D. – [Slide Presentation](#)

1. Starting doses is there a usual range?
2. Single versus Split – How is it being done and do we have data on whether this improves safety?
3. Dose escalation – single subject cohorts versus traditional 3 subject cohorts.

5:30 PM

Adjourn

Day 2 – Wednesday, September 11, 2013

Session IV: Scientific and Commercial Challenges

- 8:15 AM** **Opening Remarks**
[Summary of Sessions I – III](#) - Donald Kohn, M.D.
- 8:30 AM** **Managing the Unexpected – SIRS and other Adverse Reactions**
[OBA Summary Data on SAEs](#) - Hans-Peter Kiem, M.D., Ph.D.
- Discussion Session**
1. What cytokine measurements are helpful and what have we learned?
 2. What is the role of steroids, and IL-6 antagonists?
 3. Pre-screening of subjects, what chronic conditions should be excluded from early trials and how is screening done?
- 9:30 AM** **Suicide Genes - Potential Role in Avoiding Acute and Long-term Toxicities**
- Helen Heslop, M.D. – [Slide Presentation](#)
Baylor College of Medicine
- 9:45 AM** **BREAK**
- 10:00 AM** **Moving the Field Forward to Licensed Products for Commercialization**
1. What are the key scientific questions to be addressed?
 2. Strategies to enhance communication, data sharing and management
 3. Resource needs and sources
 4. Path to licensing – will it be different than for other therapies?
 5. Are there alternative models for early trials to facilitate rapid testing of new targets?
 6. What are the key issues to prepare for commercialization of these products?
- 12:00 PM** **Final Comments**
- 12:15 PM** **Adjourn**