AGENDA

The committee will discuss biologics license application (BLA) 125646 for tisagenlecleucel suspension for intravenous use. The application was submitted by Novartis Pharmaceuticals Corp. The proposed indication (use) for this product is for the treatment of pediatric and young adult patients 3 to 25 years of age with relapsed/refractory B-cell acute lymphoblastic leukemia (ALL).

8:00 a.m. Call to Order and Introduction of Committee  
Bruce J. Roth, MD  
Chairperson, ODAC

8:05 a.m. Conflict of Interest Statement  
Jennifer Shepherd, RPh  
Acting Designated Federal Officer, ODAC

8:10 a.m. Introductory Remarks  
Wilson W. Bryan, MD  
Director  
Office of Tissues and Advanced Therapies (OTAT)  
Center for Biologics Evaluation and Research (CBER)  
FDA

8:15 a.m. APPLICANT PRESENTATIONS  
Novartis Pharmaceuticals Corp.

Introduction  
Samit Hirawat, MD  
Head, Oncology Global Development Unit  
Novartis Pharmaceuticals Corp.

Unmet Need  
Stephen P. Hunger, MD  
Children’s Hospital of Philadelphia

Manufacturing  
Spencer Fisk, BSc  
Head, Cell & Gene Technical Development & Manufacturing  
Novartis Pharmaceuticals Corp.

Lentiviral Vector  
James Miskin, PhD  
Chief Technical Officer  
Oxford Biomedica (UK) Ltd.

Correlations Between Product Attributes and Clinical Outcomes  
David Lebwohl, MD  
CAR-T Franchise Global Program Head  
Novartis Pharmaceuticals Corp.
AGENDA (cont.)

9:00 a.m.  FDA PRESENTATION
Tisagenlecleucel CMC presentation       Xiaobin Victor Lu, PhD
Chemistry, Manufacturing & Controls (CMC) Reviewer
Gene Therapies Branch (GTB)
Division of Cellular & Gene Therapies (DCGT)
OTAT, CBER, FDA

10:00 a.m. Clarifying Questions to the Presenters

10:15 a.m. BREAK

10:30 a.m. OPEN PUBLIC HEARING

11:00 a.m. Questions to the Committee/Committee Discussion

12:00 p.m. LUNCH

1:00 p.m. APPLICANT PRESENTATIONS
Novartis Pharmaceuticals Corp.

Efficacy                  Samit Hirawat, MD

Safety                   David Lebwohl, MD

Clinical Perspective    Stephan Grupp, MD, PhD
Children’s Hospital of Philadelphia

Conclusion               David Lebwohl, MD

1:45 p.m. FDA PRESENTATION
BLA 125646 Tisagenlecleucel       Maura O’Leary, MD
Medical Officer, Team Leader
Clinical Hematology Branch (CHB)
Division of Clinical Evaluation & Pharmacology/Toxicology (DCEPT)
OTAT, CBER, FDA

2:30 p.m. Clarifying Questions to the Presenters

2:45 p.m. BREAK

3:00 p.m. OPEN PUBLIC HEARING

3:30 p.m. Questions to the Committee/Committee Discussion

5:00 p.m. ADJOURNMENT