FDA Regulatory Considerations for Orphan Drug Designation
Tissue Agnostic Therapies in Oncology Public Workshop
May 9, 2018
FDA White Oak Campus
10903 New Hampshire Ave, Building 31, Room 1503, Section B and C
Silver Spring, Maryland 20993

9:00 AM – 9:30 AM
Registration

9:30 AM – 9:40 AM
Opening Remarks
Speaker: Scott Gottlieb, MD, Commissioner, FDA

9:40 AM – 9:50 AM
Meeting Overview and Goals
Speaker: Debra Lewis, OD, MBA, OOPD, OMPT, FDA

9:50 AM – 10:10 AM
Presentation – Scientific Background of Tissue Agnostic Drug Development
Speaker: David Hong, MD, MD Anderson
Objective: To highlight the scientific issues and clinical trial designs pertaining to the development of oncology drugs agnostic of tumor type.

10:10 AM – 10:30 AM
Presentation - Office of Hematology and Oncology Products (OHOP) Perspective
Speaker: Steven Lemery, MD, MHS, OHOP, CDER, FDA
Objective: To highlight the OHOP regulatory perspective on organ specific disease versus tissue agnostic indications using pembrolizumab/MSI-H as an example.

10:30 AM – 10:50 AM
Presentation – Office of Orphan Products Development (OOPD) Perspective
Speaker: Debra Lewis, OD, MBA, OOPD, OMPT, FDA
Objective: To provide an overview of the orphan drug designation and exclusivity framework and current approach.

10:50 AM - 11:00 AM
Break

11:00 AM – 12:30 PM
Panel Discussion with Audience Q&A
Co-moderators: Debra Lewis, OD, MBA, OOPD, OMPT, FDA and Joohee Sul, MD, OHOP, CDER, FDA

Panelists:
Henry Startzman, MD, OOPD, OMPT, FDA
Steven Lemery, MD, MHS, OHOP, CDER, FDA
David Hong, MD, MD Anderson
Shivaani Kummar, MD, Stanford University School of Medicine
Dung Le, MD, Johns Hopkins Sidney Kimmel Comprehensive Cancer Center
Eric Rubin, MD, Merck & Co., Inc.
Scott Mellis, MD, PhD, Regeneron Pharmaceuticals, Inc.
Richard Moscicki, MD, PhRMA
Mark Stewart, PhD, Friends of Cancer Research
Pamela Gavin, MBA, National Organization for Rare Disorders
Kurt Karst, JD, Association for Accessible Medicines
Rachel Sher, JD, MPH, Association for Accessible Medicines

12:30 PM – 1:30PM
Lunch

1:30 PM – 2:15 PM
Continued Panel Discussion with Audience Q&A

2:15 PM – 2:30 PM
Closing Remarks
Speaker: Debra Lewis, OD, MBA, OOPD, OMPT, FDA

The statements and opinions of the presenters and panelists external to the Food and Drug Administration (FDA) do not reflect the view of the FDA.