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.