

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