

**FDA/The Osteosarcoma Institute (OSI) Workshop:
Advancing Osteosarcoma Drug Development –
Connecting Research and Regulatory Pathways for Improved Outcomes**

AGENDA

8:30 – 9:30 AM (1 hour)	Coffee and Conversation <i>In-person attendees</i>
9:30 – 9:45 AM (15 minutes)	Opening Remarks and Introduction to the Workshop <i>Lee Helman, MD (OSI)</i> <i>Martha Donoghue, MD (FDA)</i> <i>Nicole Drezner, MD (FDA)</i>
9:45 – 10:15 AM (30 minutes)	SESSION 1: Patient and Advocate Insights <i>Moderator: Mac Tichenor (OSI)</i> <i>Mikaela Naylor (MIB Agents, Junior Advisory Board)</i> <i>MacKenzie Maddry (MIB Agents, Junior Advisory Board)</i> <i>Ann Graham (MIB Agents)</i> <i>Justin Ingram, PhD (Alex’s Lemonade Stand)</i> <i>Michael Egge, Esq. (Osteosarcoma Collaborative/OSI)</i>
10:15 – 11:00 AM (45 minutes)	SESSION 2: Clinical and Regulatory Landscape <ul style="list-style-type: none"> • Summary of current treatments and ongoing clinical trials in osteosarcoma. • Overview of regulatory requirements for drug approval in the context of rare diseases. <i>Amanda Marinoff, MD (UCSF)</i> <i>Jasmine Smith, MD (FDA)</i>
11:00 – 11:20 AM (20 minutes)	SESSION 3: Targeting Osteosarcoma <ul style="list-style-type: none"> • Discussion of promising therapeutic targets and preclinical research. <i>Alejandro Sweet-Cordero, MD (UCSF)</i>
11:20 – 11:45 AM (25 minutes)	BREAK
11:45 AM – 12:45 PM (60 minutes)	SESSION 4: Trial Design and Endpoints in Osteosarcoma <ul style="list-style-type: none"> • Regulatory, clinical, and industry perspectives on endpoints and innovative trial design approaches for osteosarcoma therapies. <i>Kristin Wessel, MD (FDA)</i> <i>Katherine Janeway, MD, MMSc (DFCI)</i> <i>Melinda Merchant, MD, PhD (Normunity)</i> <i>Harpreet Singh, MD (Precision for Medicine)</i>
12:45 – 1:30 PM (45 minutes)	SESSION 5: Attendee and Panelist Question and Answer <i>Moderator: Nicole Drezner, MD (FDA)</i> <i>Panelists from Sessions 1-4</i>

1:30 – 2:15 PM (45 minutes)	LUNCH
2:15 – 3:00 PM (45 minutes)	SESSION 6: A Fireside Chat – Changing the Landscape of Pediatric Cancer with Legislation <i>Nancy Goodman, Esq. (Kids v Cancer)</i> <i>Mac Tichenor (OSI)</i>
3:00 – 4:00 PM (60 minutes)	SESSION 7: The Path Forward <ul style="list-style-type: none"> How advocacy, academic, industry, and regulatory parties can collaborate on a path forward through specific product development, trial design, endpoints, combination, and platform trials. <p><u>Moderator:</u> <i>Melinda Merchant, MD, PhD (Normunity)</i> <i>Katie Barnett, MD (FDA)</i> <i>Nicole Drezner, MD (FDA)</i> <i>Alli Murdoff (Battle Osteosarcoma/OSI)</i> <i>Brigitte Widemann, MD (NCI)</i> <i>Davy Chiodin, PharmD (Day One Biopharmaceuticals)</i> <i>Tedi Soule, PharmD (GSK)</i> <i>Fernanda Arnaldez, MD (AstraZeneca)</i> <i>Dr. Jose Ricardo Pérez-Torrealba (Exelixis)</i></p>
4:00 – 4:45 PM (45 minutes)	SESSION 8: Attendee and Panelist Question and Answer <u>Moderators:</u> <i>Lara E. Davis, MD (OHSU) and Nicole Drezner, MD (FDA)</i> <i>Panelists from Sessions 6 and 7</i>
4:45 – 5:00 PM (15 minutes)	Closing Remarks and Adjourn <i>Katherine Janeway, MD, MMSc (DFCI)</i> <i>Kristin Wessel, MD (FDA)</i>