



# 2018 International Chordoma Research Workshop

Boston Marriott Cambridge Hotel | 50 Broadway, Cambridge, MA

Wednesday, March 21   Salon Foyer	
6:00 PM	Registration opens
6:30 – 8:30 PM	Welcome reception

Thursday, March 22   Salons 1-3	
7:00 AM	Registration and continental breakfast
8:00 AM	<b>Welcome</b> Josh Sommer, Chordoma Foundation Shreyaskumar Patel, The University of Texas MD Anderson Cancer Center
8:10 – 9:55 AM	<b>Session I: State of the art clinical management</b> Moderator: Shreyaskumar Patel, The University of Texas MD Anderson Cancer Center  Setting the stage for the workshop, presentations in this opening session will define the current state of chordoma clinical management, highlighting key knowledge gaps, clinical needs and opportunities to improve care. A panel discussion will aim to identify areas of further research needed to optimize radiotherapy for chordoma.
8:10 AM	<b>Mobile spine and sacral chordoma</b> Joe Schwab, Massachusetts General Hospital
8:30 AM	<b>Skull base chordoma</b> Shaan Raza, The University of Texas MD Anderson Cancer Center
8:50 AM	<b>Advanced disease</b> Anthony Conley, The University of Texas MD Anderson Cancer Center
9:15 AM	<b>Panel discussion: The role of radiotherapy in the management of chordoma</b> Yen-lin Chen, Massachusetts General Hospital Tom DeLaney, Massachusetts General Hospital Rosaria Fiore, CNAO Peter Rose, Mayo Clinic Franklin Sim, Mayo Clinic Josh Yamada, Memorial Sloan Kettering Cancer Center
9:55 AM	Break

<b>10:15 – 11:35 AM</b>	<p><b>Session II: Disease biology and target discovery</b>  Moderator: Deric Park, National Cancer Institute</p> <p>Presentations in this session will share unpublished results revealing novel tumor dependencies and potential therapeutic targets. Discussion will focus on ideas and possibilities for further target discovery and validation.</p>
<b>10:15 AM</b>	<p><b>Epigenetic control of brachyury and metabolic stress response: novel therapeutic targets for chordoma</b>  Lucia Cottone, University College London</p>
<b>10:35 AM</b>	<p><b>Systematic discovery of novel vulnerabilities in chordoma</b>  Tanaz Sharifnia, Broad Institute</p>
<b>10:55 AM</b>	<p><b>Rationale for the Advancement of PTEN/AKT Pathway Inhibitors and Combinations for Personalized Chordoma Therapy</b>  Chad Brenner, University of Michigan</p>
<b>11:15 AM</b>	<p><b>Panel discussion</b></p>
<b>11:35 AM</b>	<p><b>The role of philanthropy in transforming cancer research</b>  Michele Cleary, The Mark Foundation for Cancer Research</p>
<b>11:50 – 12:45 PM</b>	<p><b>Lunch</b>  Location: Salon 4</p>
<b>12:45 – 3:15 PM</b>	<p><b>Breakout session 1A: Brachyury biology and therapeutic discovery</b>  Moderator: David Drewry, University of North Carolina  Location: Salons 1-3</p> <p>Recognizing brachyury’s central role in chordoma pathogenesis and survival, the purpose of this session is to identify potential approaches to target brachyury, and to define the requisite biology, reagents, and experiments needed to advance development of corresponding therapies.</p>
	<p><b>Brachyury in chordoma and carcinomas: biology and potential targeting approaches</b>  Claudia Palena, National Cancer Institute</p> <p><b>Charting brachyury-mediated developmental pathways during early mouse embryogenesis</b>  Zhe Liu, Howard Hughes Medical Institute’s Janelia Research Campus</p> <p><b>Crystal structures of brachyury: a prelude to drug discovery</b>  Opher Gileadi, University of Oxford</p> <p><b>A brachyury transcriptional reporter to guide drug discovery</b>  Slim Sassi, Massachusetts General Hospital</p> <p><b>Investigating brachyury gene regulation to identify therapeutic targets in chordoma</b>  Hadley Sheppard, Baylor College of Medicine</p> <p><b>Delineating and targeting the brachyury-YAP regulatory axis in cancer</b>  Sagar Shah, Mayo Clinic Jacksonville</p> <p><b>Discussion</b></p>

12:45 – 3:15 PM	<p><b>Breakout session 1B: Patient outcomes and clinical endpoints</b>  Moderator: Chris Heery, Bavarian Nordic  Location: Concept Room</p> <p>Given chordoma’s slow growth rate, low cellularity and long natural history, conventional measures of tumor response such as RECIST are inadequate indicators of clinical benefit. In this session we will discuss response evaluation criteria that could be more relevant to chordoma therapeutic development, and will seek to identify potential strategies for validating these criteria.</p>
	<p><b>T1-Weighted dynamic contrast-enhanced MR perfusion imaging characterizes tumor response to radiation therapy in chordoma</b>  Patricia Santos, University of Pennsylvania</p> <p><b>PET imaging for chordoma</b>  Silvia Stacchiotti, Istituto dei Tumori, Milan</p> <p><b>Developing endpoints and outcome measures for NF1: Response Evaluation in Neurofibromatosis and Schwannomatosis (REiNS) working group</b>  Brigitte Widemann, National Cancer Institute</p> <p><b>Developing functional outcome measures for chordoma</b>  Joe Schwab, Massachusetts General Hospital</p> <p><b>Quality of life insights from AO Spine knowledge forum</b>  Charles Fisher, University of British Columbia</p> <p><b>Quality of life endpoints in the SACRO study</b>  Stefano Radaelli, Istituto dei Tumor, Milan</p> <p><b>Development of a patient-centered, disease-specific quality-of-life assessment tool for clival chordomas</b>  Raj Mukherjee, University of Pittsburgh</p> <p><b>Skull base quality of life measures</b>  Erin McKean, University of Michigan</p> <p><b>Methodological considerations for patient reported outcomes in chordoma</b>  Antonia Bennett, University of North Carolina</p> <p><b>Regulatory perspective on efficacy endpoints in studies of rare tumors</b>  Ashley Ward, Food and Drug Administration</p> <p><b>Discussion</b></p>
3:15 PM	<b>Break</b>

<b>3:30 – 5:30 PM</b>	<p><b>Breakout session 2A: Immunology and immunotherapy</b>  Moderator: Jim Hodge, National Cancer Institute  Location: Salons 1-3</p> <p>This session will seek to identify key unanswered questions about the chordoma-immune interaction and identify the most promising opportunities for applying advances in immunotherapy to chordoma.</p>
	<p><b>Overview of chordoma immunology</b>  Jim Hodge, National Cancer Institute</p> <p><b>CAR T cell-based immunotherapy of chordoma</b>  Soldano Ferrone, Massachusetts General Hospital (to be confirmed)</p> <p><b>Immune correlatives and their application to chordoma</b>  Chris Heery, Bavarian Nordic</p> <p><b>Investigation of in-vivo synergistic effect of checkpoint blockade and radiation therapy against chordomas in a humanized mouse model</b>  Sheng-fu Larry Lo, Johns Hopkins University</p> <p><b>The NANT Chordoma NK Brachyury Vaccine</b>  Patrick Soon Shiong, NantWorks</p> <p><b>Discussion</b></p>
<b>3:30 – 5:30 PM</b>	<p><b>Breakout session 2B: Pediatric chordoma</b>  Moderator: Brigitte Widemann, National Cancer Institute  Location: Concept Room</p> <p>The goal of this session is to outline a research roadmap for improving outcomes among pediatric chordoma patients. Discussion will focus on defining the greatest clinical needs for this population, identifying critical unanswered questions, and proposing key research objectives.</p>

	<p><b>Overview of pediatric chordoma</b> Katherine Thornton, Dana Farber Cancer Institute</p> <p><b>Outcomes of pediatric chordoma patients treated with proton therapy</b> Norbert Liebsch, Massachusetts General Hospital</p> <p><b>Clinicopathologic characteristics of poorly differentiated chordoma</b> Yen-lin Chen, Massachusetts General Hospital</p> <p><b>A series of 62 chordomas in children and adolescent patients: clinical characters, surgery treatments and outcomes</b> Jiwei Bai, Beijing Tiantan Hospital</p> <p><b>UPMC pediatric chordoma experience</b> Paul Gardner, University of Pittsburgh Medical Center</p> <p><b>Optimizing precision medicine therapies for pediatric brain tumors</b> Carl Koschmann, University of Michigan</p> <p><b>Discussion</b></p>
<b>5:30 – 7:30 PM</b>	<p><b>Reception and poster session</b> Hors d'oeuvres and cash bar Location: Salons 5-7</p>

<b>Friday, March 23   Salons 1-3</b>	
<b>7:00 AM</b>	<b>Continental breakfast</b>
<b>8:00 – 9:40 AM</b>	<p><b>Session III: Breakout session reports</b> Moderator: Rob Schoelkopf, Yale</p> <p>Reports on key outcomes and recommendations from each breakout session will be followed by group a brainstorm about opportunities to pursue newly identified research priorities.</p>
<b>8:00 AM</b>	<p><b>Brachyury biology and therapeutic discovery</b> David Drewry, University of North Carolina</p>
<b>8:20 AM</b>	<p><b>Immunology and immunotherapy</b> Jim Hodge, National Cancer Institute</p>
<b>8:40 AM</b>	<p><b>NCI Rare Tumor Initiative and Rare Tumor Patient Engagement Network</b> Brigitte Widemann, National Cancer Institute</p>
<b>9:00 AM</b>	<p><b>Patient outcomes and quality of life</b> Chris Heery, Bavarian Nordic</p>
<b>9:20 AM</b>	<b>Discussion</b>
<b>9:40 AM</b>	<b>Break</b>

<b>10:00 – 10:40 AM</b>	<b>Keynote lecture</b>  <b>Base editing: chemistry on a target nucleotide in the genome of living cells</b> David Liu, Broad Institute
<b>10:45 AM – 12:25 PM</b>	<b>Session IV: Opportunities to accelerate chordoma research</b> Moderator: David Sandak, Accelerate Brain Cancer Cures  This session will showcase programs, initiatives and bold ideas that could be leveraged to accelerate key chordoma research priorities.
<b>10:45 AM</b>	<b>NCI Rare Tumor Initiative and Rare Tumor Patient Engagement Network</b> Brigitte Widemann, National Cancer Institute
<b>11:05 AM</b>	<b>AO Spine Knowledge Forum</b> Charles Fisher, University of British Columbia
<b>11:25 AM</b>	<b>Children’s Brain Tumor Tissue Consortium</b> Adam Resnick, Children’s Hospital of Philadelphia
<b>11:45 AM</b>	<b>Activating the First Response of Cancer immunotherapy in Natural Killer (NK) Cells</b> Patrick Soon Shiong, NantWorks
<b>12:05 PM</b>	<b>Discussion</b>
<b>12:25 - 1:30 PM</b>	<b>Lunch + group photo</b> Location: Salon 4
<b>1:30 – 3:05 PM</b>	<b>Session V: Understanding and modeling tumor heterogeneity</b> Moderator: Adrienne Flanagan, University College London  Talks in this session will highlight relevant molecular and genetic differences both between and within chordoma tumors and will discuss how to ensure that preclinical models adequately reflect these differences.
<b>1:30 PM</b>	<b>Nivolumab with or without stereotactic radiosurgery in treating patients with recurrent, advanced, or metastatic chordoma</b> Michael Lim, Johns Hopkins University
<b>1:45 PM</b>	<b>Clonal chromosomal changes associated with patient outcome</b> Paul Gardner, University of Pittsburgh Medical Center
<b>2:00 PM</b>	<b>Single cell RNAseq to investigate chordoma cell heterogeneity</b> Slim Sassi, Massachusetts General Hospital
<b>2:15 PM</b>	<b>Cell culture models of tumor diversity in chordoma</b> Kevin Mellert, University of Ulm
<b>2:30 PM</b>	<b>Molecular characterization of available preclinical models</b> Joan Levy, Chordoma Foundation
<b>2:45 PM</b>	<b>Discussion</b>
<b>3:05 PM</b>	<b>Break</b>

<b>3:20 – 5:10 PM</b>	<p><b>Session VI: Clinical trials</b>  Moderator: Shreyaskumar Patel, The University of Texas MD Anderson Cancer Center</p> <p>This session will provide updates on ongoing and planned clinical trials, highlighting lessons learned in the process of designing, initiating, and running chordoma trials.</p>
<b>3:20 PM</b>	<p><b>A randomized, double-blind, phase 2 trial of GI-6301 (yeast-brachyury vaccine) versus placebo in combination with standard of care definitive radiotherapy</b>  Deric Park, National Cancer Institute</p>
<b>3:35 PM</b>	<p><b>BN-brachyury vaccine trial</b>  Chris Heery, Bavarian Nordic</p>
<b>3:50 PM</b>	<p><b>Afatinib in locally advanced and metastatic chordoma</b>  Hans Gelderblom, University of Leiden</p>
<b>4:05 PM</b>	<p><b>CDK4/6 Inhibition in locally advanced/metastatic chordoma</b>  Stefan Frohling, University of Heidelberg</p>
<b>4:20 PM</b>	<p><b>Sacral chordoma: a randomized &amp; observational study on surgery versus definitive radiation therapy in primary localized disease (SACRO)</b>  Stefano Radaelli, Istituto dei Tumor, Milan</p>
<b>4:35 PM</b>	<p><b>A phase II, multicenter study of the EZH2 inhibitor tazemetostat in adult subjects with INI1-negative tumors</b>  Silvia Stacchiotti, Istituto dei Tumor, Milan</p>
<b>4:50 PM</b>	<p><b>Discussion</b></p>
<b>5:10 – 5:15 PM</b>	<p><b>Conclusion</b>  Josh Sommer, Chordoma Foundation</p>
<b>5:15 – 6:30 PM</b>	<p><b>Break</b></p>
<b>6:30 – 9:00 PM</b>	<p><b>Reception and dinner with chordoma community</b>  Location: Salon 4</p> <p>Join chordoma patients and family members for a special evening honoring those in our community who have made a truly uncommon impact. Dinner will be followed by live music from the Ipswich Jazz Ensemble featuring Dr. Tom DeLaney.</p>