

AGENDA - DAY ONE

7 to 8:30 am	Breakfast		
8:30 to 9 am	Welcome		
	Connected ribbons	Laura Rossano, FOP Community Advocate	
	Opening remarks	Betsy Bogard, International FOP Association	
9 to 10:30 am	FOP Talks 1: Updates on Current FOP Clinical Research (12-minute talks, except as noted)		
	Palovarotene Phase 2 Trial: Top line results (20 minutes)	Fred Kaplan, M.D., University of Pennsylvania and Donna Grogan, M.D., Clementia Pharmaceuticals	
	Expanding our knowledge of FOP: The Clementia FOP Natural History Study	Ed Hsiao, M.D., Ph.D., University of California, San Francisco	
	FOP Connection Registry: A cornerstone in FOP research	Neal Mantick, International FOP Association	
	Questions & answers		
	Facilitated discussion: Gaps and next steps	Chris Scott, M.D., Red Cross War Memorial Children's Hospital, Cape Town, South Afric	
0:30 to 11 am	Coffee Break		
11 am to 12:45 pm	FOP Talks 2: Informing Trial Design: Learnings from Observ	vational and Preclinical Data (12-minute talks)	
	Progress toward biomarkers of FOP	Robert Pignolo, M.D., Ph.D., Mayo Clinic	
	Can 18F-NaF PET/CT scan quantitate development of HO lesions in FOP?	Marelise Eekhoff, M.D., Ph.D. and Coen Netelenbos, M.D., Ph.D., VU University Medica Center, Amsterdam, Netherlands	
	Visualization of heterotopic bone formation in FOP mice	Jeff Hunter, Alexion Pharmaceuticals	
	New insights from a genetically accurate and physiologically relevant mouse model of FOP	Aris Economides, Ph.D., Regeneron Pharmaceuticals	
	FOP lesion development: Opportunities for intervention	Eileen Shore, Ph.D., University of Pennsylvania	
	Questions & answers		
	Facilitated discussion: Gaps and next steps	Chris Scott, M.D., Red Cross War Memorial Children's Hospital, Cape Town, South Afric	
12:45 to 1:45 pm	Lunch		
1:45 to 4 pm	FOP Talks 3: ACVR1/ALK2 Pathway Inhibition in FOP (12-minute talks)		
	Clinical translation of novel discoveries of activin A in FOP mice	Xiaobing Qian, M.D., Ph.D., Regeneron Pharmaceuticals	
	Repurposing saracatinib for FOP treatment	Alex Bullock, Ph.D., University of Oxford, UK	
	Small molecule Alk inhibitors to reduce heterotopic ossification in the ALK2 $^{\rm Q207D}$ mouse model: March toward the clinic	Dan Perrien, Ph.D., Vanderbilt University Medical Center	
	Discovery of ALK2 inhibitors via nanocyclix chemistry and their use in FOP	Josselin Caradec, Ph.D., Oncodesign, Dijon, France	
	Characterization of highly selective ALK2 inhibitors for the treatment of FOP	Andrew Garner, Ph.D., Blueprint Medicines	
	A tool desired for FOP	Takenobu Katagiri, Ph.D., Saitama Medical University, Saitama, Japan	
	Questions & answers		
	Facilitated discussion: Gaps and next steps	Jay Groppe, Ph.D., Texas A&M University College of Dentistry	
1 to 4:30 pm	Coffee Break		
4:30 to 5 pm	Enabling Access to Therapies		
	FOP Patient Narrative Project	Stéphanie Hoffmann, Clementia Pharmaceuticals	
	Questions & answers		
5 to 6 pm	FOP Patient Panel I: Perspectives on FOP Drug Development		
	Panelists: Monica Anderson, Erin Danzer & Lori Danzer, Shannon Halford & Raina Halford, Brian Harwell, Tim Hazlett & Debbie	Moderator: Michelle Davis, International FOP Association	
	Hazlett, Laura Rossano		



AGENDA - DAY TWO

7 to 8:30 am	Breakfast		
8:30 to 8:45 am	Welcome Back		
	Opening remarks	Betsy Bogard, International FOP Association	
8:45 to 10:45am	FOP Talks 4: New Therapeutic Approaches, Part I (12-minute talks)		
	Cellular response to hypoxia fuels FOP flare-ups	Fred Kaplan, M.D., University of Pennsylvania	
	The immunological contribution to heterotopic ossification in FOP	Michael Convente, B.A., University of Pennsylvania	
	Targeting hypoxic signaling to prevent traumatic and genetic heterotopic ossification	Benjamin Levi, M.D., University of Michigan	
	Mast cell inhibition as a therapeutic approach in FOP	Robert Pignolo, M.D., Ph.D., Mayo Clinic	
	Two tissue-resident progenitor populations regulate distinct phenotypes of FOP	Paul Yu, M.D., Ph.D., Brigham and Women's Hospital and Harvard Medical School	
	Cells and signals that regulate heterotopic ossification in FOP	David Goldhamer, Ph.D., University of Connecticut Stem Cell Institute	
	Questions & answers		
	Facilitated discussion: Gaps and next steps	Roberto Ravazzolo, Ph.D., University of Genova, Italy	
10:45 to 11:15 am	Coffee Break		
11:15 am to 1 pm	FOP Talks 5: New Therapeutic Approaches, Part II (12-minute talks)		
	Application of disease-specific iPS cells for FOP research	Junya Toguchida, M.D., Ph.D., Center for iPS Cell Research and Application, Kyoto University, Kyoto, Japan	
	The use of electronic health record-linked DNA database in drug development for FOP	Charles Hong, M.D., Ph.D., Vanderbilt University Medical Center	
	Involvement of TGF-beta Activating Kinase 1 (TAK1) during heterotopic ossification	Yuji Mishina, M.D., University of Michigan School of Dentistry	
	Act 2 for Act A: The human story	Haitao Wang, Ph.D., University of Pennsylvania	
	The impact of voluntary exercise on joint ossification in the ACVR1[R206H] knock-in mouse model of FOP	Paul Yu, M.D., Ph.D., Brigham and Women's Hospital and Harvard Medical School	
	Questions & answers		
	Facilitated discussion: Gaps and next steps	Roberto Ravazzolo, Ph.D., University of Genova, Italy	
1 to 2 pm	Lunch		
2 to 2:15 pm	IFOPA Talk		
	IFOPA: The voice of the community	Michelle Davis, International FOP Association	
2:15 to 3:15 pm	FOP Patient Panel II: A Broad View of FOP		
	Panelists: Andrew Davis, Sophia Forshtay & Connie Green & David Forshtay, Jaxon Hamilton & Lara Theoret, Justin Henke & Wendy Henke, Sarah Steele (via phone)	Moderator: Michelle Davis, International FOP Association	
	Questions & answers		
3:15 to 4 pm	Closing		
	Meeting outcomes and closing remarks	Betsy Bogard, International FOP Association	
	How did we get here?	Aarti Madan, FOP Community Advocate	
	Adjourn		