

## Mechanistic and Therapeutic Advances in Rare Skeletal Diseases

*A meeting in affiliation with the American Society for Bone and Mineral Research (ASBMR)*

**SEPTEMBER 26-27, 2018**

Preceding the ASBMR Annual Meeting *Montreal, Canada*

*A meeting jointly organized by the [Rare Bone Disease Alliance \(RBDA\)](#) and the Brittle Bone Disorders Consortium of the NIH Rare Diseases Clinical Research Network to bring together scientists interested in rare bone disease. The program will cover state of the art topics in diagnosis, preclinical mechanisms of disease, clinical targeting of signaling pathways, and novel clinical endpoints for assessment of efficacy.*



**Program Chair**

Brendan Lee, MD, PhD

*Baylor College of Medicine*



**Program Co-Chair**

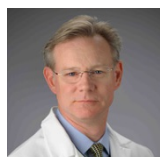
Maurizio Pacifici, PhD

*Children's Hospital of Philadelphia*

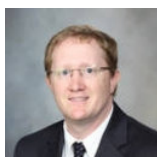
### Program Committee:



Yang Chai, DDS, PhD  
*USC*



Michael Collins, MD  
*NIDCR*



Matthew Drake, MD, PhD  
*Mayo Clinic*



Deborah Krakow, MD  
*UCLA*



Sandesh Nagamani, MD  
*Baylor College of Medicine*

### REGISTRATION (\$300)

- Registration (\$300) for this meeting will be available *only* through the [ASBMR's Annual Meeting registration](#) opening in May 2018
- Reduced Registration (\$150) for allied health professionals, students, fellows, residents, government, VA, and military attendees.

### HOTEL INFORMATION

Hotel Information is now available through the ASBMR at <http://www.asbmr.org/hotel-reservations>.

### Meeting Agenda

**Wednesday, September 26**

**8:00am - 9:40am: Session 1: Diagnostic Approach to Rare Skeletal Diseases**

**Moderator: Maurizio Pacifici**

Speaker 1: Brendan Lee, MD, PhD (Baylor College of Medicine) on next generation sequencing and multi-omic approaches for diagnosing skeletal diseases.

Speaker 2: Struan Grant, PhD (Children's Hospital of Philadelphia) on higher order chromatin structure and distal genetic interactions in the diagnosis of skeletal diseases

Speaker 3: Deborah Krakow, MD (University of California, Los Angeles) on radiographic and ultrasound imaging of skeletal diseases

Speaker 4: David Eyre, PhD (University of Washington) on past, current and future biomarkers of the skeleton

**9:40am – 9:55am: Break**

**Session 2: Preclinical Models and Pathogenesis (Bones, Cartilage & Craniofacial)**

**9:55am - 12:00am: Session 2A**

**Moderator: Deb Krakow**

Speaker 1: Michael Collins, MD (National Institute of Dental and Craniofacial Research) on Fibrous Dysplasia

Speaker 2: Aris Economides, PhD (Regeneron Pharmaceuticals) on BMP Signaling and Therapeutic Approaches

Speaker 3: Frank Rauch, MD (Shriners Hospital of Montreal) on Osteogenesis Imperfecta

Speaker 4: Yingzi Yang, PhD (Harvard School of Dental Medicine) on Progressive Heterotopic Ossification

Speaker 5: Maurizio Pacifici, PhD (Children's Hospital of Philadelphia) on Hereditary Multiple Exostoses

**12:00am - 1:30pm: Lunch & Poster Session 1**

**1:30pm - 3:10pm: Session 2B**

**Moderator: Yang Chai**

Speaker 6: Eileen Shore, PhD (University of Pennsylvania) on Fibrodysplasia Ossificans Progressiva

Speaker 7: Andrew Wilkie, FRS, FMedSci, FRCP (University of Oxford) on craniosynostosis

Speaker 8: Ophir Klein, MD, PhD (University of San Francisco) on Hypo-oligodontia and tooth stem cells

Speaker 9: Anna Teti, PhD (University of L'Aquila) on Osteopetrosis

**3:10pm - 3:30pm: Break**

**3:30pm - 5:35pm: Session 3: Therapies on the Horizon and New Disease Targets**

**Moderator: Michael Collins**

Speaker 1: Peter J. Brown, PhD, (University of Toronto), on Chemical Biology and Drug Discovery

Speaker 2: Yang Chai, DDS, PhD (University of Southern California) on dental stem cell therapies

Speaker 3: Cecelia Gotherstrom, PhD (Karolinska Institutet) on Stem Cells for Treatment of OI and the BOOSTB4 Trial (sponsored by the ICCBH)

Speaker 4: Denise Adams, MD (Harvard) on Gorham's Disease

Speaker 5: Timothy Bhattachyrra, MD (National Institutes of Health) on Melorheostosis

**5:35 pm - 7:30pm: Evening Reception/Poster Session 2**

## **Thursday, September 27**

### **8:00am - 9:40am: Session 4: Targeting Signaling Pathways (Clinical)**

**Moderator: Matthew Drake**

Speaker 1: Sandesh Nagamani, MD (Baylor College of Medicine) on the clinical trial of anti-TGFb in OI

Speaker 2: Andreas Grauer, MD (Amgen) on the role of sclerostin inhibition in bone.

Speaker 3: Tom Carpenter, MD (Yale University) on FGF23 and X-linked hypophosphatemia

Speaker 4: Julie Hoover Fong, MD, PhD (Johns Hopkins) on C-natriuretic peptide & achondroplasia

**9:45am - 10:00am: Break**

### **10:05am - 11:45am: Session 5: Advances in Endpoints and Assessments (Preclinical & Clinical)**

**Moderator: Sandesh Nagamani**

Speaker 1: Steven Boyd, PhD (University of Calgary) on HR-pQCT evaluation of bone

Speaker 2: Sharmila Majumdar, PhD (University of San Francisco) on MRI evaluation of cartilage

Speaker 3: Theresa Kehoe, MD and Gemma Kuijpers, PhD (FDA) on FDA approach to novel endpoints.

Speaker 4: William Horton, MD (Oregon Health Sciences University) on novel growth plate markers

Speaker 5: Michael Whyte, MD (Shriners Hospital, St. Louis, MO) on Hypophosphatasia: What's Next?

**11:45am - 1:45pm: Lunch and Poster Session 3**

### **1:45pm - 3:45pm: Session 6: Current Industry Clinical Trials and Approach to Pivotal Phase 3 and Post-Approval Studies – 10 minutes each formal presentation and then 40 minute panel**

**Moderator: Brendan Lee**

Presentations from Regeneron, Clementia (FOP and HME), Ultragenyx, Mereo, and other companies.

Representatives will discuss status of ongoing clinical trials in rare bone diseases.

**For additional information, contact [waldmancharlene234@gmail.com](mailto:waldmancharlene234@gmail.com)**

*The Rare Bone Disease Alliance is a program of the Osteogenesis Imperfecta Foundation.*