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 (Karolina Institute) 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-naturietic 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

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