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 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.

ABSTRACT SUBMISSION

- Selected posters may be displayed at both the ASBMR and the Rare Bone Disease Alliance meeting.
- Presenters may only present an oral presentation at the ASBMR meeting OR the Rare Bone Disease Alliance meeting.
Meeting Agenda

Wednesday, September 26

8:00am - 9:55am: **Session 1: Diagnostic Approach to Rare Skeletal Diseases**
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

10:00am - 10:15am: Break

**Session 2: Preclinical Models and Pathogenesis (Bones, Cartilage & Craniofacial)**
10:20am - 12:25pm: **Session 2A**
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: Anna Teti, PhD (University of L'Aquila) on Osteopetrosis

12:30pm - 1:30pm: Lunch & Poster Session 1

1:35pm - 3:15pm: **Session 2B**
Speaker 5: Maurizio Pacifici, PhD (Children’s Hospital of Philadelphia) on chondrodysplasias
Speaker 6: Andrew Wilkie, FRS, FMedSci, FRCP (University of Oxford) on craniosynostosis
Speaker 7: Ophir Klein, MD, PhD (University of San Francisco) on Hypo-oligodontia and tooth stem cells
Speaker 8: Peter J. Brown, PhD, (University of Toronto), on Chemical Biology and Drug Discovery

3:15pm - 3:30pm: Break

3:35pm - 5:15pm: **Session 3: Therapies on the Horizon and New Disease Targets**
Speaker 1: Yang Chai, DDS, PhD (University of Southern California) on dental stem cell therapies
Speaker 2: Cecelia Gotherstrom, PhD (Karolina Institute) on Stem Cells for Treatment of OI and the BOOSTB4 Trial (sponsored by the ICCBH)
Speaker 3: Denise Adams, MD (Harvard) on Gorham’s Disease
Speaker 4: Timothy Bhattachyrra, MD (National Institutes of Health) on Melorheostosis

5:15 pm - 7:00pm: Evening Reception/Poster Session
Thursday, September 27

8:00am - 9:55am: Session 4: Targeting Signaling Pathways (Clinical)
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 anti-sclerostin therapy in OI
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

10:00am - 10:15am: Break

10:20am - 12:15pm: Session 5: Advances in Endpoints and Assessments (Preclinical & Clinical)
Speaker 1: Steven Boyd, PhD (University of Calgary) on HRPqCT 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

12:15pm - 1:45pm: Lunch and Poster Session 3

2:00pm - 3:30pm: Session 6: Current Industry Clinical Trials and Approach to Pivotal Phase 3 and Post-Approval Studies
Presentations from Regeneron, Clementia, Ultragenyx, Mereo, Alexion, 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.