RARE BONE DISEASE HIGHLIGHTS FROM THE ASBMR 2022 RARE DISEASES SYMPOSIUM

COR2ED
THE HEART OF MEDICAL EDUCATION

ASBMR
The American Society for Bone and Mineral Research

ONGOING CLINICAL STUDIES* IN DIVERSE CONDITIONS

- MULTICENTRIC CARPOTARSAL OSTEOLYSIS SYNDROME
- JANSEN TYPE METAPHYSEAL CHONDRODYSPLASIA
- GENERALIZED ARTERIAL CALCIFICATION OF INFANCY (GACI)
- FIBRODYSPLASIA OSSIFICANS PROGRESSIVA (FOP)
- FAMILIAL HYPOPHOSPHATEMIA
- HYPOPHOSPHATASIA
- OSTEOGENESIS IMPERFECTA (OI)
- DYSOSTOSES

*SOURCE: CLINICALTRIALS.GOV

Certain non-disease specific therapies for rare bone disease may cause **adverse events** not otherwise observed in treatment of more common bone disease: appropriate **clinician follow-up** is key to **patient care.**

THE SCIENCE

Experimental therapies for achondroplasia are designed to interact with the pathogenic receptor FGFR3 or its downstream signaling pathways

THE TREATMENT

Effective therapies should address key morbidities such as stenoses and cervical cord compression

Expanding from the paradigm of pharmacological **therapy of OI**, initial safety data from a trial of umbilical cord **mesenchymal stem cell transplantation** suggests that multiple transplantations in children with type IV disease is safe.

From **scientists** to **clinical researchers** to **patient advocates**, the mutual engagement among more than 300 Rare Diseases Symposium attendees highlights the importance of ongoing **collaboration** and **communication** between these groups, to advance solutions for **rare bone disease patients**.

FGFR3: fibroblast growth factor receptor 3

This educational content was developed in partnership between COR2ED and the American Society for Bone and Mineral Research, supported by an independent medical education grant from the Rare Bone Disease Consortium, represented by Alexion, AstraZeneca Rare Disease, by Ipsen and by Kyowa Kirin, who had no input into selection of topics or speakers.

September 2022

Access the ASBMR 2022 Rare Bone Disease Highlights podcast series