

## WORKSHOP: Translational research on bone impairment in rare diseases

Faculty of Medicine of Lyon, 9 & 10 June 2022

### SCIENTIFIC PROGRAM

#### THURSDAY, 9TH JUNE 2022

10h00 – 10h30 Welcome participants

#### SESSION 1: How to conduct clinical trials on rare diseases (Dr A. Portefaix, Pr J. Bacchetta)

- 10h30 Clinical trials in orphan diseases: methodological perspectives – P. Sisovsky, MSc (State Institute for Drug Control, Slovakia)
- 11h05 Clinical research in bone disease: the interest of Patients' Related Outcomes – Dr Appelman- Dijkstra (Leiden University Medical Center, NL)
- 11h40 Challenges to define clinical trial outcomes in bone diseases – Pr Chapurlat (Inserm UMR 1033-HCL- University Lyon 1, France)
- 12h15 Future of Dental research in rare bone diseases – Pr Chaussain (University Paris Descartes, France)

13h00 – 14h30 Lunch Break

#### SESSION 2: Pathophysiological models (Dr I. Machuca- Gayet, Pr A. Linglart)

- 14h30 Craniofacial development – Dr Mayor (UCL- London -UK)
- 15h05 The interest of zebrafish models in evaluating rare bone diseases - Dr Dambroise (Institut Imagine Paris, France)

15h45 – 16h00 Coffee Break

- 16h00 The interest of murine models in evaluating rare bone diseases – Dr Riminucci (Sapienza University of Rome, Italy)
- 16h35 The interest of « omics » models in evaluating rare bone diseases – Pr Hesse (Hospital of the Ludwig-Maximilians-University Munich, Germany)
- 17h15 Discussion

## FRIDAY, 10TH JUNE 2022

### 08h30 – 09h30 SESSION 3 – MEET THE EXPERTS

#### SESSION 4: How to adapt a clinical issue to an experimental model (Pr F. Di Rocco, Dr O. Peyruchaud)

- 09h30 Cystinosis model – Dr Alioli (Inserm UMR 1033 University Lyon 1, France, France)
- 10h00 Craniosynostosis and altered molecular signaling in the calvarial stem cell niche – Dr Lattanzi (Università Cattolica del Sacro Cuore Rome, Italy)
- 10h30 Example of researcher/clinician collaboration – Dr Machuca- Gayet / Pr Bacchetta (Inserm UMR 1033-HCL- University Lyon 1, France)
- 10h50 Discussion

### 11h00 – 11h15 Coffee break

### SESSION 5: Therapeutics (Pr J. Bacchetta, Pr. R. Chapurlat)

- 11h15 RUDY registry to improve therapeutic management: pros and cons of patient direct implication in registries – Dr Javaid (University of Oxford, UK)
- 11h45 Therapeutic potentials of vitamin D receptor ligands – Dr Laverny (IGBMC-Strasbourg, France)
- 12h15 Closing remarks

