WORKSHOP: Translational research on bone impairment in rare diseases

Faculty of Medicine of Lyon, 9 &10 June 2022

SCIENTIFIC PROGRAM

THURSDAY, 9TH JUNE 2022

10:00 – 10:30: Welcome participants

SESSION 1: How to conduct clinical trials on rare diseases (Dr A. Portefaix, Dr F. Schaefer)

- 10:30: Clinical trials in orphan diseases : methodological perspectives – Dr Sisovsky (State Institute for Drug Control, Slovakia)
- 11:05: Clinical research in bone disease : the interest of Patients' Related Outcomes – Dr Appelmans- Dijkstra (Leiden University Medical Center, NL)
- 11:40: Challenges to define clinical trial outcomes in bone diseases – Pr Chapurlat (Inserm UMR 1033-HCL- University Lyon 1, France)
- 12:15: Future of Dental research in rare bone diseases – Pr Chaussain (University Paris Descartes , France)

13:00 – 14:30: Lunch break

SESSION 2: Pathophysiological models (Dr O. Peyruchaud, Pr A. Linglart)

- 14:30: Craniofacial development – Dr Mayor (UCL- London -UK)
- 15:05: The interest of zebrafish models in evaluating rare bone diseases - Dr Dambroise (Institut Imagine Paris, France)

15:45 – 16:00 : Coffee Break

- 16:00: The interest of murine models in evaluating rare bone diseases – Dr Riminucci (Sapienza University of Rome, Italy)

- 16:35: The interest of « omics » models in evaluating rare bone diseases – Pr Hesse (Hospital of the Ludwig-Maximilians-University Munich, Germany)
- 17:15: Discussion

FRIDAY, 10TH JUNE 2022

08:00 – 09:00 : SESSION 3 - MEET THE EXPERTS

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

- 09:00: Cystinosis model – Dr Alioli (Inserm UMR 1033 University Lyon 1, France, France)
- 09:30: Craniosynostosis and altered molecular signaling in the calvarial stem cell niche – Dr Lattanzi (Università Cattolica del Sacro Cuore Rome, Italy)
- 10:00: Example of researcher/clinician collaboration – Dr Machuca- Gayet / Pr Bacchetta (Inserm UMR 1033-HCL- University Lyon 1, France)
- 10:15: Discussion

10 :15 – 10:30 : Coffee break

SESSION 5 : Therapeutics (Pr J. Bacchetta, Dr L. Sangiorgi)

- 10:30: New therapies of genetic bone disorders: CRISPR/Cas9 gene editing and cell therapy in the treatment of osteogenesis imperfecta and other rare bone diseases - where are we now? – *Dr Van Dijk (Northwick Park Hospital London, UK) (to be confirmed)*

- 11:30: RUDY registry to improve therapeutic management : pros and cons of patient direct implication in registries – Dr Javaid (University of Oxford, UK)
- 12:00: Therapeutic potentials of vitamin D receptor ligands – Dr Laverny (IGBMC-Strasbourg, France)
- 12:35: Closing remarks