# Orphan Drug & Rare disease Seminar

*- Accelerating Access to Therapeutic Innovation -*

## Eudipharm

Eudipahrm is a Master degree in pharmaceutical medicine offered by Université Claude Bernard in Lyon, France. In 2012, it was awarded Center of Excellence by PharmaTrain, a European consortium of Master's degrees in pharmaceutical medicine. This elective module is part of the PharmaTrain program. It aims at providing continuing professional development to those wishing to update their skills or acquire new expertise. More info: www.eudipharm.net

## OrphanDev

Created in 2009, OrphanDev is the French national F-CRIN platform which supports project carriers in Clinical trials in rare diseases. OrphanDev is a multidisciplinary team gathering skills in various fields (academic research, industry research, clinical pharmacology, Public Health, R&D, production, orphan drug development, clinical development…). Since the attachment of OrphanDev to FCRIN in 2013, the network participates to European project with ECRIN and other international projects. OrphanDev supplies services adapted to rare diseases clinical trial projects and to drug development projects in rare diseases. This platform is aimed at institutions, academics, as the drug companies. More info: www.orphan-dev.org

## F-CRIN

F-CRIN is the French component of the European infrastructure ECRIN-Eric (European Clinical Research Infrastructure Network). F-CRIN has been created to reinforce the French clinical research competitiveness in targeted high scientific added-value fields by supporting investigators and sponsors for every stage of clinical development but especially for proof-of-concept studies, transnational clinical trials and projects submitted to European calls. Its objective is to set up a platform and a portal open to sponsors and investigators for more skills and more ease for the setup/realization of projects in three operational fields: Large-scale academic clinical trials; European projects coordinated by French teams and Early-stage industrial trials for innovative therapeutics (Biotech; Pharma). F-CRIN is also greatly involved in the training of French professionals with the organization of topical training courses and the setup of an online tool, the "Training Course Advisor (TCA)". More info: www.fcrin.org

## Background

In Europe, rare diseases affect more than 30 millions persons. A rare disease is defined as being a life threatening or chronically debilitating disease which affects less than 1 person per 2000. There are between 6000 and 8000 identified rare diseases. Most of them have genetic origins (80%) and have no causal treatment.

Since the mapping of the human genome, **rare diseases have entered therapeutic era**. Policies have accompanied scientific progress, giving hope and making treatments available for some of these diseases.

**Orphan Drug's business model** appears now as an attractive one for the research community. Indeed, in this stringent economic context, orphan drug development offers huge potential to treat patients in a personalized way and to grant therapeutic innovation to pharmaceutical companies.

However, present economical context brings us to rethink our social model, questioning our fundamental values. Amongst the rare disease community, voices are raising: **Is Orphan Drug Model sustainable**? Answers need to be found in order to sustain the huge achievement of the rare diseases' community for patients so far.

This Eudipharm training seminar aims at raising awareness among clinical research actors on drug development specificities for rare diseases. This edition will offer a first day on reglementary issues and the second day will focus on drug repositionning and personalised medicine, thanks to the participation of clinical research experts and authorities.

## Location

**Marseille (event to be confirmed)**

## For more information:

**Scientific Secretariat:** Cécile Colomban, OrphanDev, cecile.colomban@univ-amu.fr
**Seminar Organisation:** Leïla Bachir, F-CRIN, leila.bachir@inserm.fr

# PROGRAMME – 17-18 November

## Organisation Committee

**Eudipharm**: Behrouz Kassaï and Catherine Cornu

**OrphanDev**: Olivier Blin and Cécile Colomban

**F-CRIN**: Vincent Diebolt, Allan Wilsdorf and Leïla Bachir

## 17 NOVEMBER

### AFTERNOON SESSIONS

13:30 – Welcome coffee

14:00 – Opening statement
Eudipharm, OrphanDev and F-CRIN

– Conferences: EMA tools in drug development (part 1)
- EMA presentation of the orphan drug designation
- EMA presentation of the protocole assistance
- Academic feedback on drug development

– Coffee break

– Conferences: EMA tools in drug development (part 2)
- Presentation of the pediatric investigation plan
- EMA presentation on advanced therapy
- Feedback on pediatric investigation plan

17:30 – End of sessions

## 18 NOVEMBER

### MORNING SESSIONS

08:30 – Welcome coffee

09:00 – Latest news: focus on a publication

– Conferences
- Methodology in rare diseases: overview and specificities
- Drug repositionning

– Coffee break

– Feedbacks
- Methodology
- Drug repositionning

– Workshop "Challenges of personalised medicine"

12:30 – Lunch break

### AFTERNOON SESSIONS

13:30 – Conference: Personalised medicine feedback in cystic fibrosis

– Workshop's feedback

– Roundtable "Challenges of personalised medicine"

– Closing statement

15:30 – End of sessions

*Provisional programme subject to changes in terms of workshop configurations and sequence of teaching units (the number of hours and the pedagogical content of the session will remain unchainged)*