May
11-12
2017

TRAINING COURSE

« Orphan drug and rare disease - Accelerating access to therapeutic innovation » 4th edition

On the 11th and 12th of May 2017, the EUDIPHARM Claude Bernard University, the F-CRIN labeled service platform Orphandev and the Training & Education Unit of F-CRIN's Coordination are co-organizing the 4th edition of the "Orphan drug & rare disease - Accelerating access to therapeutic innovation " training course. While the methodology remains the main focus of the program, this year will also be the opportunity to address more specifically the EMA tools in drug development but also the issues around personalized medicine.

Who ?

This training course is aimed at clinical research professionals, project promoters but also patient associations, involved in rare diseases and in the development of orphan drugs.

Why ?

This training seminar aims at raising awareness among clinical research actors on drug development specificities for rare diseases:

- **Identify** tools for drug development
- **Consolidate** practices
- **Apprehend** the stakes

How ?

Participants will discuss and deepen the topics proposed through:

- Knowledge capitalization
- Feedbacks
- Projection of practices
- Interactions and exchanges with clinical research actors

Training stakeholders

Speakers

European Medecines Agency
ANSM
Academic experts
Experts from the private sector
Patient associations
[…]

Organisation committee

Eudipharm : Behrouz Kassaï & Catherine Cornu
Orphandev : Olivier Blin & Cécile Colomban
F-CRIN : Vincent Diebolt & Allan Wilsdorf

Partners

PharmaTrain MASTERING MEDICINES DEVELOPMENT
Lyon 1
Aix*Marseille université
Eurobiomed
Hospices Civils de Lyon
Assistance Publique Hôpitaux de Marseille

Highlights

- Participatory teaching methods
- Intervention of experts in the field
- Approach to tools and methodology
- Participation of the leading players of the clinical research

Date: 11th and 12th May 2017
Location : Marseille – Hôpital La Timone
Training course in english
Online registration: link
Contact: leila.bachir@inserm.fr

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PROGRAMME

« Orphan drug and rare disease - Accelerating access to therapeutic innovation » 4th edition

Day 1 – Thursday 11th May 2017

Opening - 13:30

- **Welcome coffee**
- **Opening statement**
  Pr Behrouz Kassaï, Lyon University Hospital

Drug development : the EMA tools

- **Orphan designation and protocol assistance: incentives for the development of therapies for rare diseases**
  Annie Lorence, ANSM (Agence Nationale de Sécurité du médicament et des produits de santé - National agency for drugs and health products safety), member of the Committee for Orphan Medicinal Products (COMP) at the EMA (European Medicines Agency)
- **Academic experience of therapeutic development: Stromal Vascular Fraction of Adipose tissue for Scleroderma Therapy**
  Dr Jérémy Magalon, Cell Culture and Therapy Laboratory, Conception Hospital, AP-HM (Marseille University Hospitals)

Discussions

- **Paediatric Investigation PLan**
  Sylvie Benchetrit, ANSM (Agence Nationale de Sécurité du médicament et des produits de santé - National agency for drugs and health products safety)
- **Feedback experience : development of Macitentan for the treatment of pulmonary arterial hypertension in children**
  Catherine Lesage, Senior Director, Clinical Science Pediatrics Program Head, Actelion

Discussions

Newsflash - PedCRIN: Capacity building project for multinational paediatric clinical trials

Salma Malik, PedCRIN

End of day 1 - 17:30

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PROGRAMME

« Orphan drug and rare disease - Accelerating access to therapeutic innovation » 4th edition

Day 2 – Friday 12th May 2017

Opening - 08:30

- **Welcome coffee**

Newsflash - Health research priorities setting

Pr Behrouz Kassaï, Lyon University Hospital

Methodology

- **Presentation and feedback: example of a size-1 trial and its meta-analysis in Wilson's disease**
  Pr Stéphane Thobois and Pr Behrouz Kassaï, Lyon University Hospital

Drug repositionning

- **Drug repositioning: new indications for old drugs**
  Olivier Blin, Head of Clinical Pharmacology Department, Timone Hospital, AP-HM (Marseille University Hospitals), OrphanDev coordinator
- **Feedback: Repositioning of Rimeporide in Duchene muscular dystrophy**
  Florence Porte Thomé, R&D Director, EspeRare Foundation

Personalised medicine [1/2]

- **Participative workshop [1/2]**
  Leïla Bachir, F-CRIN

12:30 – Lunch break – 13h30

Personalised medicine [2/2]

- **Personnalized medicine for cystic fibrosis**
  Dr François Vermeulen, Catholic Univeristy of Leuven
- **Drug development in progeria**
  Pr Nicolas Levy, Head of medical genetics department, Timone Hospital, AP-HM (Marseille University Hospitals)
- **Participative workshop [2/2]**
  Leïla Bachir, F-CRIN
- **Table ronde**
  Academic and industry professionals as well as patients, expert children and national authorities' representatives

End of day 2 - 15:30

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INFORMATION

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Online registrations

F-CRIN infrastructure's website : **registration form**

Fees

Academic/institution
first registration for a structure: 300€ (Day2 only) / 450€ (Day1+Day2)
additional registration for the same structure: 250€ (Day2 only) / 400€ (Day1+Day2)

Student
Single price : 50€ (Day2 only) / 80€ (Day1+Day2)

Industry
first registration for a structure: 450€ (Day2 only) / 650€ (J1+J2)
additional registration for the same structure: 400€ (Day2 only) / 600€ (Day1+Day2)

Patient associations
Single price : 50€ (Day2 only) / 80€ (Day1+Day2)

Coffee breaks & lunches are included.

Site access

Address
Amphithéâtre HA2
Hôpital La Timone
264 Rue Saint-Pierre - 13005 Marseille

GPS coordinates
43.291712, 5.402719

Phone contact
Your contact on site
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