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.

During the last decades a big leap has been made forward with the mapping of the human genome and thus identification of many rare diseases genetic and molecular basis. This scientific progress has opened perspective for treatment discovery. Meanwhile, international and national regulations have provided legal framework for healthcare improvement in the field of rare diseases and fostered research.

But this new therapeutic era for rare diseases with an increasing number of promising proofs of concept is characterized by the young and non mature drugs development culture. It appears as an emergency to now fill the gap in translational research for rare diseases.

Pre clinical issues, early phase clinical trials' design, conception and conduction of clinical trials, patients' selection and recruitment, multinational cooperation are thus new challenges to uptake in order to accelerate and improve availability and access of therapeutic innovations.

This Eudipharm training seminar aims at raising awareness among clinical research actors on drug development specificities for rare diseases. It will contribute to provide tools and solutions for clinical research professional and project carriers thanks to the participation of clinical research experts.

Organisation Committee

Eudipharm: Behrouz Kassai, Catherine Cornu, Kent Neal
OrphanDev: Joelle Micallef, Yolande Adjibi, Marine Berro
F-CRIN: Olivier Rascol, Vincent Diebolt, Allan Wilsdorf

With the support of the **French Foundation for rare diseases**

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.

Place

Aix Marseille Université
Amphithéâtre Gastaud
Jardin du Pharo
57 Boulevard Charles Livon
13007 – Marseille

For more information:

Website:
www.eudipharm.net/claroline141/RARE/
www.fcrin.org/nos-actualites/actualites/accelerating-access-therapeutic-innovation
www.orphan-dev.org

Scientific Secretariat: Yolande Adjibi, Marine Berro
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Orphan Drug & Rare disease Seminar

-Accelerating Access to Therapeutic Innovation-

-Marseille-

-17 & 18 october 2013 -

Training Seminar
organised by

Thursday October 17th 2013

08:45-9:00 - INTRODUCTION TO THE SEMINAR
Eudipharm, OrphanDev, F-CRIN

09:00-10:00 - CONFERENCES:
Rare diseases and organization for rare diseases
Prof. Nicolas Levy, Director of the French Foundation for Rare Diseases, France
Why reinforcing clinical development transnational process and organization is a necessity-Experience reporting
Prof. Evelyne Jacqz-Aigrain, National coordinator of paeditratics' investigating centers, Robert Debré Hospital, France

10:00-10:30 Coffee break
10:30-12:30 - ROUND TABLE:
Methodology of clinical trial in rare diseases
Moderator: Prof. Behrouz Kassai, Hospices Civils de Lyon, France
Design of clinical trials for small populations
Prof. Corinne Alberti, Robert Debré hospital, France
The Use of Modelling and simulating to design new trials,exemple of Dravet syndrome
Dr. Catherine Chiron & Dr. Polina Kurbatova, Necker hospital, France
Outcomes for rare diseases clinical trials
Prof. Shahram Attarian, Timone hospital, France

12:30-14:00 Lunch Break
14:00-16:00 - CONFERENCES: Improving clinical trials for rare diseases a priority for the future
Partnering in rare disease: a case study in accelerating the development for a therapy
Dr Jeremy Springhorn, Alexion Pharmaceuticals, US
Strengthening capacities for clinical trial
Prof. Joaquim Ferreira, University of Lisbon, Portugal
Patient's recruitment in clinical trials for rare diseases, a strategy to anticipate
Dr. Joelle Micallef, OrphanDev, France
Relevance of registries for clinical trials in rare diseases, how to construct them to match clinical trial needs
Prof. Paul Landais, France

16:00-16:30 Coffee break
16:30-18:00 - ROUND TABLE:
Patients' organizations and orphan drug development
Moderator: Anne Sophie Lapointe, Vaincre les Maladies Lysosomales, France
Serge Braun, French Association against Neuromuscular Diseases (AFM), France, Maria Mavris, Eurordis, Prof. Marc Nicolino, pediatric endocrinology, France, Khazal Paradis, Genzyme , Amsterdam.

20:00 Gala Diner

Friday October 18th 2013

09:00-10:00 - CONFERENCES:
From Pre-clinic to Clinic: what bridges?
Translating proof of concept in Cell model into clinical evaluation
Marc Peschanski, i-Stem, Paris, France
Translating Body of evidence into clinical evaluation
Dr Bernard Landwehrmeyer, Ulm University, Germany

10:00-10:30 Coffee Break
10:30-12:00 - CONFERENCES:
Orphan drug's development process
The Orphan Drug designation
Jordi Llinares, EMA, UK
Drugs Evaluation and development in Paediatrics,
Prof. Gerard Pons, EMA Paediatrics' Committee, France
One orphan drug development experience
Dr Luc-André Granier , Advicenne Pharma, France

12:00-13:30 Lunch Break
13:30-15:30 - ROUND TABLE:
Multinational collaboration for clinical development in rare diseases
Moderator: Prof. Jacques Demotes, ECRIN, France
How to get into a European project?
Celine Damon, Head of European Research Programs Unit Aix-Marseille University, Health NCP, H2020, France
How to initiate a European clinical trial?
Dr. Nicolas André, paedaitric oncology, Timone Hospital, Marseille
The voluntary harmonization procedure
Prof. Jacques Demotes, ECRIN, France
One Start-Up experience
Eric Halioua, Promethera, Belgique

15:30 - CONCLUSION

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REGISTRATION
Register on –line: www.atout-org.com/orphandrugseminar
by E-mail to: orphandrugseminar2013@atout-org.com
by Fax : +33 4 96 15 12 51

- Please register before September 17, 2013 -

FEES BEFORE / FEES AFTER **31 JULY 2013**
V.A.T. included - coffee breaks & lunches included

Academic, institution	**€360**/ €450
Industry	**€960 /** €1200
Student	**€55** / €70

Conférence dinner on Thursday ,17 october 2013 **€55**