OrphanDev

F-CRIN-labelled platform

Drug development accelerator for rare diseases

Based in a dynamic region in the rare diseases area (with 11 reference centres, Eurobiomed, a cluster focused on rare diseases, two rare diseases networks, university-hospital teams involved), **OrphanDev** is a public platform attached to Aix Marseille Université (AMU) and the Institut de Neurosciences de la Timone (INT). It is part of the national infrastructure F-CRIN (French Clinical Research Infrastructure Network) and works in collaboration with all the national structures active in the area of rare diseases.

OrphanDev is specialised in supporting researchers, clinicians and the health industry actors in the development of drugs for rare diseases. This multidisciplinary team supports all projects from private or public sector, throughout the crucial stages of drug development. It provides its scientific and regulatory expertise in Orphan Designation and Protocol Assistance Applications; its logistical and methodological support for clinical trials in rare diseases with a specificity in the patient's recruitment strategy; and its experience in national and European calls for projects. This set of services and tools allows the acceleration of the development of therapies for rare diseases.

OrphanDev is an academic platform in the heart of rare diseases, close to the research teams, clinicians, industry professionals and patient organizations. It proposes its scientific, regulatory and methodological expertise in the development of drugs for rare diseases. OrphanDev assists its partners, particularly for Orphan Designation applications, the recruitment of patients for clinical trials, and European projects.

int institut de neurosciences de la timone

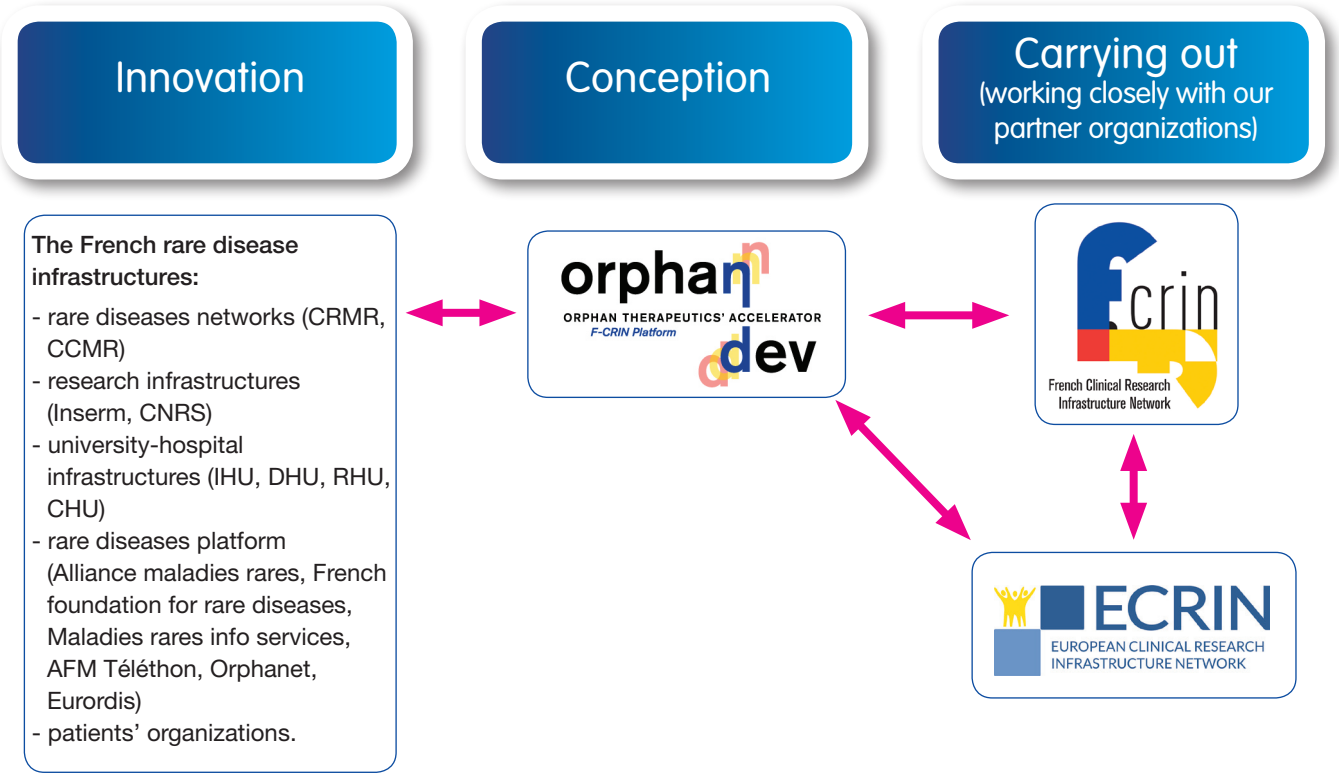
Aix*Marseille université

fcrin French Clinical Research Infrastructure Network

orphan dev ORPHAN THERAPEUTICS' ACCELERATOR F-CRIN Platform

Our partners

OrphanDev is one of the links in the chain of a national system with a European opening for the research and the development in rare diseases in France.



• The F-CRIN infrastructure (French Clinical Research Infrastructure Network)

F-CRIN's mission is to reinforce the visibility and the competitiveness of French clinical research, whether academic or industrial, particularly on the European and international level. The infrastructure has labelled clinical investigation networks centered on 8 themes and clinical research support platforms, all partner entities of the **OrphanDev platform**.

On a European level, F-CRIN is associated with ECRIN, a European structure whom F-CRIN is the French scientific partner for the set up and the management of complexe multinational clinical trials.

Our training courses

• Orphan Drug and Rare Disease Seminar

European meeting between the key players in the area of rare diseases (researchers, clinicians, industry, competent authorities), which has for objective to encourage a common reflexion on the challenges and questions concerning the assessment of orphan drugs.
Organised every 18 months in collaboration with EudiPharm and with the support of F-CRIN, this training course is intended for academics and health industry professionals.

• Explain me clinical trials

Destined for patients, in particular those suffering from rare diseases, this training course is organised in partnership with the 'Tous Chercheurs' and 'François Aupetit' (AFA) organizations, and with the counselling of F-CRIN. The aim is to sensibilise patients to therapeutic evaluation by allowing them to better understand drugs development process and clinical trials.

The **OrphanDev** team has a considerable experience in the key steps of drug development for rare diseases. It proposes specific scientific, regulatory, methodological and logistical support specific to the orphan drugs development and rare diseases clinical trials.
At **OrphanDev**, experts in the clinical pharmacology and drug development area, help industries, clinicians and researchers in their rare diseases projects.

Our specific expertise

EMA/FDA ORPHAN DESIGNATION

Advantages

- Financial advantages in the regulatory procedures at the EMA
- A ten years commercial exclusivity after the Marketing Authorisation in Europe and seven years in the United States
- Visibility and recognition of the project and its sponsor
- Eligibility for European calls for projects in the area of rare diseases

OrphanDev Services

- Dossier feasibility study
- Proposal of a timetable for the submission
- Help for drafting the scientific document
- Help for compiling the administrative file
- Drafting the prevalence section
- Assistance in the meetings with the agencies
- Submission and follow-up of the application

EMA/FDA PROTOCOL ASSISTANCE OR SCIENTIFIC ADVICE

Advantages

- Obtaining scientific and regulatory recommendations from the EMA and/or FDA
  - on the drug development plan
  - on the protocol for a clinical trial

OrphanDev Services

- Proposal of a timetable for the submission
- Help for compiling the administrative file
- Help for drafting the scientific document
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PATIENT'S RECRUITMENT IN CLINICAL TRIALS

Advantages

- Time-saving for investigators
- Time-saving for patients
- Reduced Sreen Failure
- Compliance with the inclusion deadlines

OrphanDev Services

- Proposal for a patient's recruitment strategy in line with the clinical trial's constraints and goals
- Set up and management of communication tools
- Set up and management of pre-screening tools
- Follow-up of the inclusions in collaboration with the investigator sites and networks involved in the study

Our other areas of expertise

Implementation of the clinical trial project

- Feasibility study
- Synopsis writing
- Advice on the set up of a clinical trial project
- Writing the responses to national and European calls for projects

Designing and conducting clinical trials

- Writing study documents

Regulatory submission and follow-up of clinical trials

- Submission of the application dossier to the competent authorities
- Study follow-up with the competent authorities

End of the clinical trial

- Promoting the results (medical writing)

Why OrphanDev ?

- **Benefit** from a specific expertise regarding clinical trials in rare diseases.
- **Receive** support in Orphan Designation and Protocol Assistance Applications.
- **Comply** with the goals and deadlines in terms of patient's recruitment.
- **Perform** clinical trials with optimum turnaround times.
- **Access** to a network of specialist professionals.
- **Have** an expert platform at your disposal for conducting therapeutic trials.
- **Overcome** the regulatory difficulties regarding the therapeutic assessment for a rare disease.
- **Deal** with logistical issues, particularly in the case of multicenter studies.

Our team

Prof. Olivier Blin, **Coordinator**
Marine Berro, **Project Manager**
Cécile Colomban, **Project Manager**

OrphanDev - F-CRIN Platform

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