Despite attractive legal incentives, and an increasing number of promising orphan designations, orphan drugs development for rare diseases remains a challenge, therapeutic evaluation being the main barrier to overcome.

**OrphanDev** is an academic platform in the heart of rare diseases, close to the research teams, clinicians, industry professionals and patient organizations. 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 Drug 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**.

### RARE DISEASES’ SPECIFICITIES

| Low or ultra low prevalence | How many patients? |
| Few knowledge on natural evolution | Which patients? Which outcomes? |
| Heterogeneous presentations | Times of evaluation? |
| Multiplicity of clinical symptoms | |
| Chronicity and low evolving diseases | |
| Inexistence of guidelines, diseases orphan of clinical trials | |

### DIFFICULTIES FOR PROTOCOL CONCEPTION

**ORPHANDEV, OUR “KNOW HOW” TO OPTIMIZE CLINICAL TRIALS FOR RARE DISEASES**

**Protocol conception:**
How to translate pre clinical findings into therapeutic evaluation? [2][3]
The pharmacological principles for success [4]

**From 3 pilars to 4 cornerstones**

- **Free Drug**
  - Dose determination
  - Route of administration
  - Evaluation Criteria

- **Target delivery**
  - Dose/ Concentration related

- **Translatability to clinical efficacy**

- **Mechanistic / Pathophysiological approaches**

- **Regulatory approaches**
  - Clinical Efficacy Read out, Biomarkers, Risks

**Patient’s recruitment strategy:**

OrphanDev proposes a patient’s recruitment strategy in line with the clinical trial’s constraints and goals. We set-up and manage communication tools, and pre-screening tools in collaboration with all the investigator sites.

**An example of call center contribution in the recruitment of patients**

- **Call center**
  - 41%
- **Active list of patients**
  - 59%

**REFERENCES**


**ORPHANDEV, OUR ACTIVITIES (2011-2015)**

- Study protocol conception
- Logistical support in clinical trials
- Orphan designation
- Protocol assistance
- Drug development plan
- Medical redaction
- Counseling Activities

**ORPHANDEV, OUR TRAINING COURSES**

« Orphan Drug & Rare Disease Seminar »
European meeting between the key players in the area of rare diseases (researchers, clinicians, industry, competent authorities), which aims to encourage a common reflection on the challenges and questions concerning the assessment of orphan drugs.

« 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 Apuepir’ (FAA) organizations, and with the counseling of F-CRIN. The aim is to sensibilise patients to therapeutic evaluation by allowing them to better understand drugs development process and clinical trials.

**ORPHANDEV, OUR COMMUNICATION TOOLS**

Website, newsletters, orphandocs, social networks...