OrphanDev

F-CRIN-labeled platform

OrphanDoc:
The Orphan Drug Designation

Orphan drug designation aims at conferring an orphan status to drugs used for the diagnosis, prevention or treatment of a disease causing a life-threatening or a chronically debilitating condition affecting not more than 5 for 10 000 persons in the European community.

It is granted by a regulatory authority: the European Medicines Agency (EMA) in Europe, the Food and Drug Administration (FDA) in the United States, the ministry of Health, Labour and Welfare of Japan, the Therapeutic Good Administration in Australia.

The orphan drug designation allows to benefit from specific incentive measures to develop new drugs for rare diseases. Obtaining an orphan drug designation does not guarantee the marketing authorization but improves the chances to get it.

OrphanDev is an academic platform in the heart of rare diseases, close to the research teams, clinicians, industry professionals and patients. It proposes its scientific, regulatory and methodological expertise specific in the development of drugs for rare diseases. OrphanDev supports its partners, particularly for Orphan Drug Designation applications, the recruitment of patients for clinical trials and European projects. This set of services and tools allows the acceleration of the development of therapies for rare diseases.

OrphanDev - F-CRIN Platform

PIICI - INT

Secteur de Pharmacologie Intégrée et Interface Clinique et Industrielle - Institut des Neurosciences de la Timone (Aix Marseille Université et UMR 7289 CNRS)

27 Bd Jean Moulin
13005 Marseille
Tel: 04 91 32 41 62

contacts@orphan-dev.org

www.orphan-dev.org

Our team:
Pr Olivier Blin, coordinator
Marine Berro, project manager
Cécile Colomban, project manager

The benefits

Designation

Protocol assistance. EMA and FDA can provide scientific advice to guide the applicant in its choices during the drug development, in order to meet the European regulatory requirements and to prepare its application for marketing authorization. This assistance increases the chances to obtain the future marketing authorization.

Fee reductions, that concern every type of centralized action (marketing authorization application, pre-authorization inspections, protocol assistance, annual fees…). Additional reductions are applied for small and medium enterprises (SMEs). For example, when ODD was obtained, fees for protocol assistance are reduced by 75%, and by 100% for SMEs. Sponsors benefit from 50%* reduction on the marketing authorization application fees (100% for SMEs*).

* Percentages are variables vary from one year to another.

Research funding, through access to call for projects dedicated to rare diseases and restricted to ODD holders (EU framework programme for Research and Innovation).

Market

An accelerated procedure for marketing authorization application. Requested by the sponsor and under certain conditions, the evaluation of the marketing authorization application by the EMA can be accelerated (150 days instead of 210 days).

Commercial exclusivity of 10 years in the EU and 7 years in the US after obtaining marketing authorization, regardless of the patent. During this period, no other similar and directly competitive drug can be authorized on the market.

The procedure in Europe

Notification to COMP
(Committee for Orphan Medicinal Products) of intention to submit an application.
By email, at least 2 months before submission.

Pre-submission meeting with EMA
which allows to prepare the application.
Optional but strongly recommended.

Submission of the application, validated by EMA (day 1)
Written in English, it contains a detailed description of the disease, its prevalence, the standard care of patients, the proposed drug, the proof of concept and the development stage.

Evaluation of the application by the COMP
which gives its positive or negative opinion (within *60 to 90 days*).

Evaluation of the application by the European commission which takes the final decision (*in 30 days*).

Publication
- of the decisions of the European commission on the community register of orphan medicinal products: http://ec.europa.eu/health/documents/community-register/html/alforphreg.htm
- of the summary of the COMP opinion: http://www.ema.europa.eu/ema/index.jsp?curl=pages/medicines/landing/orphan_search.jsp&mid=WC0b01ac058001d12b

Other implicit benefits

- allows to be positioned on a given pathology
- Brings credibility to the project for its development and the search of funding

When to ask for it?

It is recommended to apply for the orphan drug designation as soon as possible. A body of evidence showing the interest of a molecule or a treatment for a rare disease (*in vitro* results, pre-clinical results, literature, etc.) is sufficient to constitute an application file for orphan drug designation.

Review after 15 years of existence in Europe :

From January 1st 2000 to January 1st 2015 :
2127 application of orphan drug designation submitted
1406 designations granted by the European commission
97 AMM obtained