Definition of orphan drug by the EMA

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28/03/23

About 30 million people living in the European Union (EU) suffer from a rare disease. The European Medicines Agency (EMA) plays a central role in facilitating the development and authorisation of medicines for rare diseases, which are termed 'orphan medicines' in the medical world.

This is an overview of the European regulation and how the European medicine agency define and assess the orphan medicinal products.

The orphan regulation established

- criteria for defining the orphan medicinal products
- a committee (COMP) who is entitled to evaluate these criteria
- the procedures
- the incentives without which actually is very difficult to develop medicine for rare disease and this was one of the main actions of the European commission with regard rare diseases

Designation criteria are mainly based on three pillars

- Rarity of the condition, in Europe has been established as a 5 people affected by the condition in 10000 in the Community.
- Seriousness of the medical condition which is defined as a life treated condition or a chronically debilitating condition.
- Alternative methods authorised; if satisfactory method exist the sponsor should establish that the product will be of a significant benefit.

Incentives for orphan drugs

- Access to the protocol assistance and community marketing authorization (all the orphan drugs are authorized centrally)
- 10 years marketing exclusivity
- Fee reduction

Designation process

Pre submission meeting → the applicants submitted the dossier → the dossier is validated.

After that it's started to count the days:

- At day 30 there is an information meeting in which all the dossier is submitted to the comp and the COMP and the comp coordinator evaluated the dossier

ERICA has received funding from the European Union's Horizon 2020 research and innovation programme under grant agreement No 964908

- Usually at day 60 there is a discussion around the aspect of the procedures about criteria that mentioned before. If committee, consider the data are sufficient to fulfil all the criteria then it can directly go to the comp opinion which is transmitted to the European commission.
- In case there are lacking element or doubts or other questions, the comp could raise a list of questions and the sponsor should provide to explain the data missing.
- At day 90 the comp will finally decide on the application and will send its opinion to the European commission

What is assessed at designation

- Condition applied for
- The chronically debilitating and life-threatening nature of the condition
- The intention to treat the condition
- The prevalence of the condition
- The significant benefit (clinically relevant advantage, a major contribution to patient care)

What is assessed at Marketing Authorization

The sponsor is requested to submit a report on the maintenance of ODD criteria.
COMP re-evaluates the fulfilment of the criteria in parallel with the MA assessment, if doubt the sponsor will be invited for an oral hearing.

Opinion by COMP if the product should be removed or not from the Community Register.

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