Orphan designation procedure

1. Sponsor notifies the Agency of intent to file.
2. Pre-submission meeting/ teleconference.
3. Submission of application; validation by the Agency (day 1).
4. Assessment/COMP meeting/possible hearing/ COMP opinion adopted (by day 60 or 90).
5. Opinion sent to the European Commission.
6. Commission decision granted (within 30 days).
7. Publication in EU Register on the Commission's website; publication of public summary of opinion on the Agency's website.

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Further information

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Orphan medicinal product designation

An agency of the European Union

What does the European Medicines Agency do?

The Agency, through its Committee for Orphan Medicinal Products (COMP) is responsible for reviewing designation applications from persons or companies ('sponsors') who intend to develop medicines for rare diseases, so-called 'orphans'. The Agency helps sponsors to prepare orphan designation applications through free pre-submission meetings. The Agency also provides advice on the development of orphan medicinal products after designation (protocol assistance).

What are orphan products?

'Orphan' medicinal products are for diagnosing, preventing or treating life-threatening or very serious conditions that are rare and affect not more than 5 in 10,000 persons in the European Union (EU). Pharmaceutical companies are unwilling to develop such medicinal products under normal market conditions, as the cost of bringing them to the market would not be recovered by the expected sales of the medicinal products without incentives.

How is orphan development stimulated?

In the EU, the legislation provides incentives for sponsors/pharmaceutical industry to develop orphan medicinal products. To be eligible for incentives, products should be designated through the procedure for orphan designation.

What are the incentives?

- Market exclusivity

For 10 years after the granting of a marketing authorisation (approval for sale), orphan medicinal products benefit from market exclusivity in the EU. During that period, directly competitive similar products cannot normally be placed on the market.

- Protocol assistance

The Agency can provide scientific advice to optimise development and guidance on preparing a dossier that will meet European regulatory requirements. This helps applicants to maximise the chances of their marketing-authorisation application being successful.

- Fee reductions

A special fund from the European Commission, agreed annually by the European Parliament, is used by the Agency to grant fee reductions. Reduction of fees will be considered for all types of centralised activities, including applications for marketing authorisation, inspections, variations and protocol assistance. For small and medium-sized enterprises (SMEs), additional fee reductions are applicable.

- EU-funded research

Sponsors developing orphan medicinal products may be eligible for grants from the EU and Member States' programmes and initiatives supporting research and development, including the European Commission framework programme.

What is orphan designation?

Orphan medicinal product designation is based on the criteria laid down in Regulation (EC) No 141/2000. Orphan designation may be obtained at any stage of development, provided proper scientific justification of the intended use is submitted. The designation procedure is free of charge. Designation as an orphan medicinal product does not indicate that the product has already satisfied the efficacy, safety and quality criteria necessary for the granting of a marketing authorisation. As for any medicinal product, these criteria can only be assessed once the application for marketing authorisation has been submitted.

More information

Guidance documents, COMP information, public summaries of opinions for designated products and more:

http://www.ema.europa.eu/htms/human/orphans/intro.htm

Register of Orphan Medicinal Products:

http://ec.europa.eu/enterprise/sectors/pharmaceuticals/documents/community-register/html/index_en.htm

DG Health & Consumers, Rare Diseases:

http://ec.europa.eu/health/rare_diseases/policy/index_en.htm