Regulatory Factsheet

Orphan Drug Designation (EU & UK)

EU - Commission Regulation (EC) No. 141/2000

In order to be eligible for Orphan Drug Designation (ODD), a product must be intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating disease which affects less than 5 per 10,000 persons in the European Community, or it must be unlikely that marketing of the medicine would generate sufficient return to justify the investment needed for development.

Applicants must also show that there are no satisfactory methods for diagnosis, prevention or treatment of the condition in the EU, or that the new treatment is anticipated to be of significant benefit over existing methods.

Applications must be made before submission of the Marketing Authorisation (MA) Application (MAA) and in accordance with published deadlines. An opinion is reached by the Committee for Orphan Medicinal Products within 90 days and this is communicated to the European Commission for adoption as a legally binding Decision.

ODD enables access to a number of incentives in the EU including, but not limited to, fee-reduced centralised scientific advice via the EMA, and a 10 year period of orphan market exclusivity, during which time a MAA for a similar medicinal product for the same indication cannot be accepted for assessment unless certain derogations – including, potentially, the need to demonstrate "clinical superiority" - apply (see schematic).

At a glance

Agency

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Criteria

- Life threatening or chronically debilitating disease affecting less than 5 per 10,000 persons in the Community
  Or
- Unlikely to generate sufficient return to justify investment
  AND
- No satisfactory diagnosis, prevention or treatment method authorised for the condition
  Or
- The new treatment has potential to offer significant benefit to patients (greater efficacy, safety, or major contribution to patient care)

Timings

- Any time before MAA submission
- 5-6 months (submission to Commission Decision)

UK (MHRA)

- Request designation at time of MAA submission
- Same criteria as EU
- Active EU ODD: apply for Great Britain orphan MAA
- No active EU ODD: apply for United Kingdom orphan MAA

Schematic:

Considerations for ODD at the time of EU MAA