Orphan Drug Designation (US)

Food and Drug Administration (Orphan Drug Act 1983)

In order to be eligible for Orphan Drug Designation (ODD), an active substance must be intended for the prevention, diagnosis, or treatment of a disease or condition affecting fewer than 200,000 persons in the United States (US). Alternatively, designation can also be requested if the cost of developing the drug and making it available in the US for such diseases or conditions will exceed any potential profits from its sale within the first 7 years of marketing.

ODD may be requested for a new, previously unapproved drug before the Sponsor submits a New Drug Application (NDA) or Biologics License Application (BLA) for the drug for the same rare disease or condition. A Sponsor may also seek ODD at any time for an already approved drug for a new, unapproved use. Where a drug is the same as an already approved orphan drug, ODD may be requested if the new treatment is clinically superior.

The application itself consists of a short (maximum of 30 pages) document which gives sufficient information to show the designation criteria have been met. There is no formal timeline for review, but most applications are reviewed within 90 days.

Incentives for orphan designated drugs include a 50% tax credit for qualified clinical testing, waiving of NDA and BLA user fees, an opportunity for 7 years of market exclusivity following marketing approval, annual grant funding and a waiver from paediatric requirements (apart from oncology indications).

At a glance

Agency

FDA U.S. FOOD & DRUG ADMINISTRATION

Criteria

- Rare disease or condition affecting less than 200,000 persons in the US
  OR
- Affects more than 200,000 persons in the US with no reasonable expectation that research and development costs will be recovered from first 7 years of sales in the US
  AND
- Clinical superiority over current approved “same drug(s)” with orphan status.

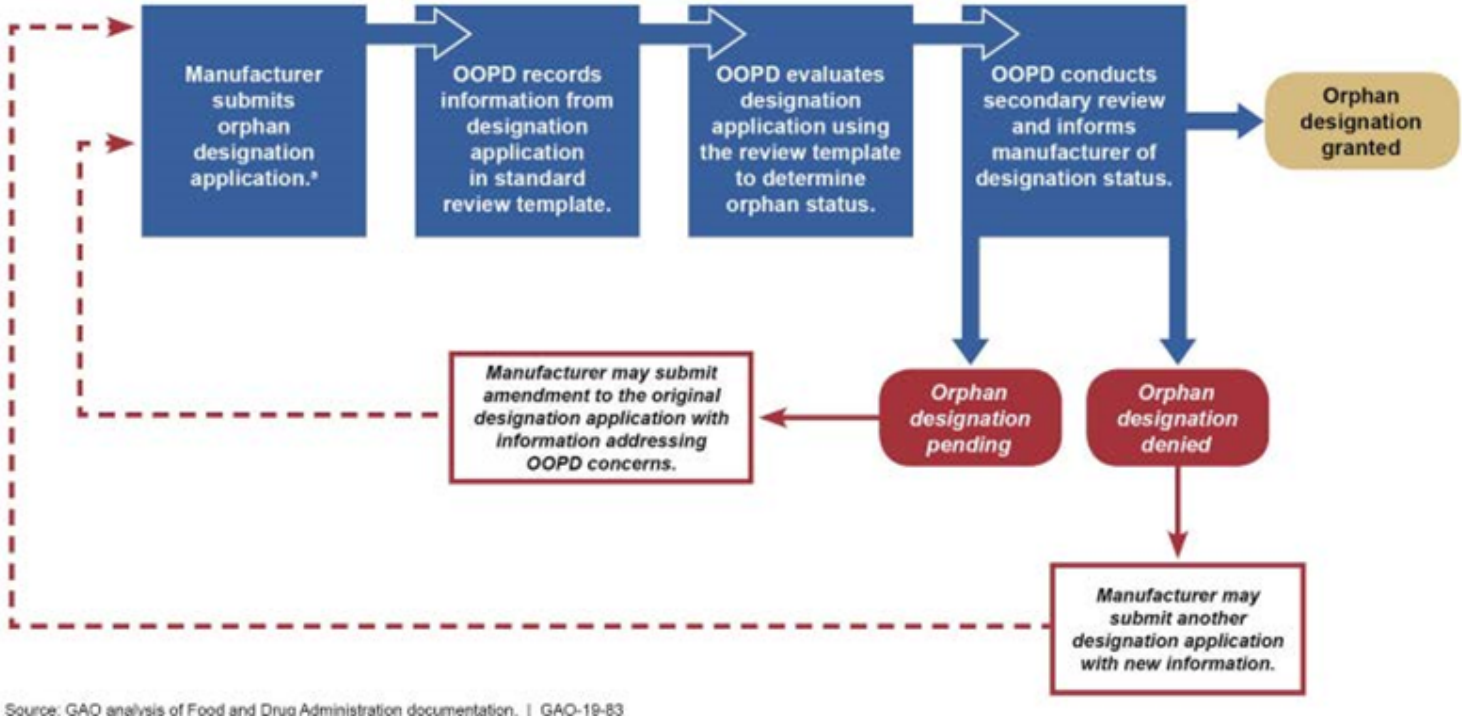
Timings

- For new drugs any time before NDA/BLA submission
- FDA usually reviews within 90 days but there is no fixed timetable

Orphan Drug Designation (US)

Schematic

Office of Orphan Products Development's (OOPD) Orphan Designation Process



Source: GAO analysis of Food and Drug Administration documentation. | GAO-19-83