Orphan designation

Orphan designation is a special status for a medicine used to treat a rare disease or condition. An orphan designation is adopted by the Committee for Orphan Medicinal Products (COMP) of the European Medicines Agency (EMA) and confirmed by the European Commission (EC) before the granting of marketing authorisation.

To qualify for orphan designation, a medicine must meet a number of criteria:

(1) It must be intended for the treatment, prevention or diagnosis of a disease that is life-threatening or chronically debilitating.

(2) The condition must affect no more than 5 in 10,000 people in the EU, or it must be unlikely that sales of the medicine will be sufficient to justify the investment needed for its development.

(3) No satisfactory method of diagnosis, prevention or treatment of the condition exists, or, if it does, the medicine in question must provide a significant benefit to those affected by the condition.

Developers of medicines who obtain orphan designation benefit from a number of incentives. Incentives include specific scientific advice and 10-year market exclusivity. Market exclusivity means that, during this period, no other medicine for the same condition will be granted market authorisation. Reduced fees for applications for services from the European Medicines Agency (EMA) may also be available.