Orphan medicine

An orphan medicine is a medicine that has been developed specifically to treat a rare condition (an ‘orphan disease’). Orphan medicines generally follow the same regulatory development path as any other medicine, however, some incentives are provided to encourage a manufacturer to invest in developing them. 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.

The incentives for developing orphan medicines include specific scientific advice, and 10 year market exclusivity. Market exclusivity means that no other medicine for the same condition will be granted market authorisation during this period. Reduced fees for applications for services from the EMA may also be available.