FACTSHEET

Orphan Drugs

Orphan drugs *"are medicinal products intended for diagnosis, prevention or treatment of life-threatening or very serious diseases affecting less than five in 10 000 people. "*

These drugs are called "orphans" because the pharmaceutical industry has little interest, under normal market conditions, in developing and marketing products intended for only a small number of patients suffering from very rare conditions. For the drug companies, the cost of bringing a rare-disease medicinal product to the market would not be covered by the expected sales of the product. For this reason, governments and organisations representing rare disease patients have emphasised the need for economic and regulatory incentives to encourage drug companies to develop and market medicines for the many neglected and "orphaned" rare disease patients.

In 2000, the European Medicines Agency (EMA) laid down the legislation (Regulation (EC) No 847/2000) which governs designation of 'orphan' status

Applications for orphan drug status are dealt with by the EMA's Committee for Orphan Medicinal Products (COMP).

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

- It is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting no more than 5 in 10,000 people in the EU at the time of submission of the designation application;

- It is intended for the diagnosis, prevention or treatment of a life-threatening, seriously debilitating or serious and chronic condition and without incentives it is unlikely that the revenue after marketing of the medicinal product would cover the investment in its development.

In both cases, there must also be either no satisfactory method of diagnosis, prevention or treatment of the condition concerned is authorised, or, if such a method does exists, the medicine must be of significant benefit to those affected by the condition.

Should a drug be successfully granted 'orphan' status, then the pharmaceutical company will benefit from a range of incentives to maintain the economic viability of marketing the drug.

In the EU, the major incentive offered is **market exclusivity for 10 years**. According to Regulation 141/2000, Article 8 (EC/141/2000) … *"the Community and the Member States shall not, for a period of 10 years, accept another application for a marketing authorisation, or grant a marketing authorisation or accept an application to extend an existing marketing authorisation, for the same therapeutic indication, in respect of a similar medicinal product".*

In addition, the EU provides further incentives during the development of an orphan drug as well as after the approval of the product; these incentives include:-

- Protocol assistance (a form of Scientific Advice) which is free of charge (regular fees for advice on CMC, nonclinical and clinical questions add up to 76 300 EUR)

- Fee reduction and exemptions

- Pre-authorisation inspection are free of charge (regular fees for each inspection inside or outside the Community are 19 100 EUR)
- 50% fee reduction for Marketing Authorisation Application (regular fees for a single strength associated with one pharmaceutical form and one presentation: 254 100 EUR)
- Post-authorisation activities, including annual fees, during the first year after marketing authorization are free of charge (e.g. regular annual fee for each Marketing Authorisation of a medicinal product: 91 100 EUR)

The EMA maintains a register of orphan drugs. The full list can be viewed at **http://www.ema.europa.eu/ema/index.jsp?curl=pages/medicines/landing/orphan_search.jsp&mid=WC0b01ac058001d12b**

Examples of Orphan Drugs

1.Denileukin diftitox for the treatment of cutaneous T-cell lymphoma, which is estimated to affect affect approximately 0.74 in 10,000 people in the European Union (EU). This is equivalent to a total of around 28,000 people

2. Interestingly though, **not all** orphan drugs are necessarily new chemical entities. Methotrexate oral liquid , for example, is designated orphan status for acute lymphoblastic leukaemia which was considered to affect 0.6 in 10,000 persons in the European Union, which, at the time of designation, corresponded to about 30,000 persons. Methotrexate has, of course, been available for many years in tablet and injection form, but this orphan formulation was considered to be beneficial for the treatment of children as it might improve compliance.

Sources:

European Medicines Agency:
http://www.ema.europa.eu/ema/index.jsp?curl=pages/home/Home_Page.jsp&mid=

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