**WHAT IS AN ORPHAN DRUG?**

**• 450 orphan drugs designated since 2000**
**• 37 orphan drugs with marketing authorisation since 2000**
**• 2 million EU citizens potentially benefiting from these drugs**

**Sources of information**
**Eurordis**
www.eurordis.org – Monthly electronic newsletter

Full list of designated orphan drugs available at:
http://ec.europa.eu/enterprise/pharmaceuticals/register/alforphreg.htm

European Medicines Agency (EMEA)
http://emea.europa.eu

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Eurordis - Rare Diseases Europe
www.eurordis.org - eurordis@eurordis.org

Rare Diseases Europe
Pharmaceutical companies are unwilling to develop such drugs under normal market conditions, as the cost of bringing them to the market would not be recovered by the expected sales of drugs without providing incentives.

**WHAT IS AN ORPHAN DRUG?**

Orphan drugs are medicinal products intended for the diagnosis, prevention or treatment of rare diseases, which are diseases affecting less than 1 in 2,000 persons or a maximum of 250,000 citizens in the European Union.

**INCENTIVES PROVIDED BY THE EU REGULATION**

- **Market exclusivity in the EU**
  Similar competitive products cannot be placed on the market for 10 years after the granting of marketing authorisation and 12 years if paediatric studies are performed.

- **Protocol assistance**
  Scientific advice is provided to pharmaceutical companies by the EMEA (European Medicines Agency, based in London) to optimise drug clinical development meeting European regulatory requirements.

- **Access to the centralised procedure**
  Orphan drugs have direct access to the EMEA centralised procedure for the application for marketing authorisation.

- **Fee reductions**
  Fee waiver for orphan designation and reduced fees for marketing authorisation, inspections, variations and protocol assistance.

- **EU-funded research**
  Pharmaceutical companies developing orphan drugs may be eligible for grants from EU and Member State programmes and initiatives supporting research and development, including the Community framework programmes.

**ORPHAN DESIGNATION**

Orphan designation may be granted at any stage of drug development, provided proper scientific justification of the intended use is submitted. Orphan designation is given by the Committee for Orphan Medicinal Products (COMP) at the EMEA (European Medicines Agency).

Orphan designation is not an endorsement for the use of the drug for the designated condition. An application for marketing authorisation must first be submitted by the pharmaceutical company. Efficacy, safety and quality criteria need to be satisfied for the granting of a marketing authorisation.

**ORPHAN DRUG LEGISLATION DEVELOPMENT**

1983 – First Orphan Drug Act in the United States
1999 – Adoption by the European Parliament of the Regulation on Orphan Medicinal Products
2000 – Creation of the Committee for Orphan Medicinal Products (COMP) at the EMEA (European Medicines Agency)