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Orphan drugs and rare diseases at a glance

1. Orphan drugs at a glance

A medicinal product is designated as an orphan medicinal product if:

- it is intended for the diagnosis, prevention or treatment of a life-threatening or chronically debilitating condition affecting no more than five in 10,000 persons in the European Union at the time of submission of the designation application (prevalence criterion), or;
- 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 expected sales of the medicinal product would cover the investment in its development, and;
- no satisfactory method of diagnosis, prevention or treatment of the condition concerned is authorised, or, if such method exists, the medicinal product will be of significant benefit to those affected by the condition.

Companies with an orphan designation for a medicinal product benefit from incentives such as:

- protocol assistance (scientific advice during the product-development phase);
- marketing authorisation (10-year marketing exclusivity);
- financial incentives (fee reductions or exemptions);
- national incentives detailed in an inventory made available by the European Commission.

Since 1 January 2007, orphan medicinal products are eligible for the following level of fee reductions:

- 100% reduction for protocol assistance and follow-up;
- 100% reduction for pre-authorisation inspections;
- 50% reduction for new applications for marketing authorisation;
- 50% reduction for post-authorisation activities, including annual fees (applies only to small and medium-sized enterprises), in the first year after granting of a marketing authorisation.

The funds made available by the Community for fee exemptions for orphan medicinal products amount to €6,000,000 in 2007.

2. Rare diseases at a glance

- Rare diseases are life-threatening or chronically debilitating conditions affecting no more than five in 10,000 people in European Union. While this number may seem small, it translates into approximately 246,000 persons in the 27 EU Member States. Most of the people represented by these statistics suffer from less frequently occurring diseases affecting one in 100,000 people or fewer.
- It is estimated that between 5,000 and 8,000 distinct rare diseases exist today, affecting between 6% and 8% of the population in total in other words, between 27 million and 36 million people in the European Union. Five new diseases are described every week in the medical literature.
- Symptoms of some rare diseases may appear at birth or in childhood, including infantile spinal muscular atrophy, lysosomal storage disorders, patent ductus arteriosus (PDA), familial adenomatous polyposis (FAP) and cystic fibrosis. However, more than 50% of rare diseases appear during adulthood, such as renal cell carcinoma, glioma and acute myeloid leukaemia.
- 80% of rare diseases have identified genetic origins. They concern between 3% and 4% of births. Other rare diseases are the result of infections (bacterial or viral) and allergies, or are due to degenerative and proliferative causes.
- Medical and scientific knowledge about rare diseases is lacking. While the number of scientific publications about rare diseases continues to increase particularly those identifying new syndromes fewer than 1,000 diseases essentially those that occur most frequently benefit from a minimum of scientific knowledge.
- The European Union's Seventh Framework Programme for Research and Technological Development (FP7, 2007-2013) will boost research into rare diseases. Its first phase focuses on innovative and multidisciplinary projects investigating (on an EU-wide scale) the natural course and pathophysiology of non-infectious, non-malignant rare diseases in the areas of: endocrine, immune and metabolic diseases; genito-urinary tract diseases; diseases affecting the digestive and respiratory system. The acquired knowledge will provide the basis for future development of diagnostic, therapeutic and potentially preventive approaches. The FP7 will also include research into rare Mendelian phenotypes (human phenotypes) of common diseases.

Information sources

European Commission Directorate-General for Health and Consumer Protection: http://ec.europa.eu/health/ph threats/non com/rare diseases en.htm

European Organisation for Rare Diseases (Eurordis):

http://www.eurordis.org

Orphanet:

http://www.orpha.net/

Community Register of orphan medicinal products for human use (Provides access to European Commission decisions on designation of orphan medicinal products.):

http://ec.europa.eu/enterprise/pharmaceuticals/register/index.htm

Seventh Framework Programme for Research and Technological Development (FP7): http://ec.europa.eu/research/fp7/index_en.cfm

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