European Commission | European Medicines Agency | US Food and Drug Administration

London, 26 November 2007
Doc. Ref. EMEA/557391/2007

The European Union and the FDA working together to create Common Application for Orphan Designation for Medicines

The European Commission, the European Medicines Agency (EMEA) and the United States (US) Food and Drug Administration (FDA) have adopted a common application form for sponsors seeking orphan designation of medicines in the European Union (EU) and US. This initiative is aimed at simplifying the process of obtaining orphan status for medicines intended for rare diseases in both jurisdictions.

About 30 million people living in the EU and 25 million Americans suffer from more than 6,000 rare diseases. Rare diseases are defined as those affecting fewer than five in 10,000 people in the EU and fewer than 200,000 people in the US. Due to the small number of patients, sponsors of medicines for rare diseases may expect relatively low profit from sales and, in some cases, a financial loss, when the costs of research and development of these drugs are taken into account.

Realising that some potentially promising orphan medicinal products for rare diseases would not be developed, the Orphan Drug Act was enacted in the US in 1983 and the European Union adopted its Regulation on Orphan Medicinal Products in 1999. These legal frameworks aim to provide regulatory and financial incentives, such as protocol assistance, and marketing exclusivity, to sponsors to develop and market orphan medicinal products.

To be eligible for receiving orphan incentives, sponsors of orphan medicines have had to submit separate applications for orphan designation to the EMEA and to the FDA using different submission formats to satisfy the respective regulatory requirements. These different formats have imposed an additional burden on sponsors. Hence, the parties have agreed to harmonise the application form to simplify part of the orphan medicines designation process.

This common application format will now allow sponsors to apply to both jurisdictions at the same time with one application. A common format will also establish a favourable environment for the EMEA and FDA to share common experiences and gain an understanding of the similarities and differences of the process of obtaining orphan designation in the two regulatory systems.

The common application form contains a section for common information required by both the EMEA and the FDA. It also has sections for requirements unique to each agency. A sponsor who wants to submit an orphan designation application to EMEA alone may also use this form. The EMEA and the FDA will still conduct independent reviews of such submissions to assure the data submitted meet the legal and scientific requirements of their respective jurisdictions.

For more information see:

COMMON EMEA/FDA APPLICATION FORM FOR ORPHAN MEDICINAL PRODUCT DESIGNATION

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