



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Press office

## Press release

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# FDA and EMA Agree to Accept a Single Orphan Drug Designation Annual Report

The U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) are moving forward in their collaboration on orphan designations and administrative simplification.

In recognition of World Rare Disease Day (the last day of February), the FDA and the EMA announce that they have agreed to accept the submission of a single annual report from sponsors of orphan products (drugs and biologics) designated for both the US and the EU.

Both regulatory agencies require the submission of an annual report for orphan designated products. These reports provide information on the status of the development of orphan medical products, including a review and status of ongoing clinical studies, a description of the investigation plan for the coming year, any anticipated or current problems in the process, difficulties in testing, and any potential changes that may impact the product's designation as an orphan product.

This one annual report submission to both regulatory agencies is voluntary, and will only be applicable to sponsors who have obtained an orphan designation status for their product both in the EU and US. Starting on 28 February 2010, sponsors may send the single Orphan Drug Designation Annual Report to each Agency.

"This process provides benefits for both agencies," said Timothy Coté, M.D., MPH, director of FDA's Office of Orphan Products Development. "Additionally, it reduces the duplication involved for sponsors in reporting to two separate regulatory agencies."

Both regulatory authorities will exchange the annual reports electronically through a secured portal. Both agencies have agreed to accept the reports on World Rare Disease Day, or, if the sponsor chooses, on their normal annual reporting date.

"We are very pleased with this collaboration on regulatory requirements and about sharing data that will help us understand the viability of the products," said Jordi Llinares, MD, MSc, Head of Orphan Medicines at the EMA. Professor Kerstin Westermark, Chair of the EMA Committee for Orphan Medicinal Products declared: "This new step in our collaboration provides each of our agencies with information in real time on any challenges arising during the development of products for rare diseases and will help identifying and acting on bottlenecks."



Presently, U.S. regulations require that orphan product sponsors submit an annual report within 14 months after the date of orphan designation and annually thereafter until market approval is obtained.

In the European Union, the Regulation on orphan medicinal products requests annual updates on the status of product development after designation.

Currently, even if an orphan product was granted designation on the exact same day in both the USA and European Union, companies submit different reports to their respective regulatory agency.

This optional process for submission will not introduce any new regulatory requirements. Each regulatory body will conduct their own review and assessment of the annual report to assure the information meets all the legal and scientific requirements of each agency. The use of one annual report will benefit sponsors by eliminating the duplication of efforts, and by simplifying the process with a single document submission that meets the requirements of both the FDA and EMA. The use of a single annual report will also help regulators better identify and share information throughout the development process of an orphan product.

### **Contact our press officers**

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