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The European Medicines Agency has launched an online portal for orphan designation applications

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In June 2018 the European Medicines Agency (EMA) launched 'IRIS'[1], a new secure online portal for sponsors to submit their orphan designation applications.

Orphan medicinal products are used to treat rare diseases and are so called because the pharmaceutical industry would, in the absence of a friendly regulatory environment, have little financial interest in developing and marketing medicines intended for only very small numbers of patients. Regulation (EC) No 141/2000[2] on orphan medicinal products was introduced with the aim of stimulating research and development and bringing to the market appropriate medications so that patients suffering from rare conditions would benefit from the same quality of treatment as other patients. The Regulation establishes a centralised procedure at EMA level for the designation of orphan medicines and puts in place incentives for their research, development and commercialization. Pharmaceutical companies can benefit from such incentives as fee reductions for regulatory activities[3], scientific assistance for marketing authorisations and the possibility of an EU marketing authorisation with a 10-year market exclusivity period.

In order to obtain the orphan designation, the medicine must fulfill certain requirements:

- it must be intended for the treatment, prevention or diagnosis of a disease that is life-threatening or chronically debilitating;

- the condition must be rare, affecting a population of not more than 5 in 10,000 in the European Union, or it must be unlikely that marketing of the medicine would generate sufficient returns to justify the investment needed for its development;

- no satisfactory method of diagnosis, prevention or treatment of the condition concerned exists, or, if such a method exists, the medicine must be of significant benefit to those affected by the condition.

Applications for orphan designation are examined by the EMA Committee for Orphan Medicinal Products (COMP). The EMA then sends the COMP opinion to the European Commission, which has competence for granting the designation.

In order to modernise and improve the orphan designation process, the new online portal provides a single virtual space where applicants can submit and manage the information and documents related to their applications and where they are able to manage post-designation activities. EMA expects the online

[1] See the following LINK.
[2] Regulation (EC) No 141/2000 of the European Parliament and of the Council of 16 December 1999 on orphan medicinal products, OJEU L 18 of 22.01.2000.
[3] This includes reduced fees for protocol assistance, marketing-authorisation applications, inspections before authorisation, applications for changes to marketing authorisations made after approval, and reduced annual fees.

portal to reduce the time needed to prepare and submit applications. During the review process, applicants can check the status of their applications from any device and receive automatic notifications when the status of the application changes.

The online portal is part of an EMA-wide programme to improve the way it records and manages master data in pharmaceutical regulatory processes (SPOR)[4] and interacts with external stakeholders, facilitating the handling of product-related applications.

Applicants will still be able to use the existing submission process until 19 September 2018. To help applicants with the transition, EMA has developed guidance documents explaining how to use the new system and what has changed with its introduction[5].

[4] EMA is implementing the standards in a phased programme based on the four domains of master data in pharmaceutical regulatory processes: substance, product, organisation and referential (SPOR) master data. These standards are developed by the International Organization for Standardization (ISO) for the identification of medicinal products (IDMP). Their purpose is to facilitate the reliable exchange of medicinal product information in a robust and consistent manner, by providing a common product 'language' for stakeholders to use in their interactions and therefore ensuring wide interoperability across global regulatory and healthcare communities. The use of these standards is a regulatory requirement as Articles 25 and 26 of the Commission Implementing Regulation (EU) No 520/2012 of 19 June 2012 on the performance of pharmacovigilance activities provided for in Regulation (EC) No 726/2004 of the European Parliament and of the Council and Directive 2001/83/EC of the European Parliament and of the Council (OJEU L 159 of 20.06.2012) oblige EU Member States, marketing authorisation holders and EMA to make use of the ISO IDMP standards.

[5] See: IRIS Quick guide to registration (available at the following LINK); Procedure for orphan medicinal product designation - Guidance for sponsors submitting an application via the current existing submission process until 19 Sept 2018 (available at the following LINK); Procedure for orphan medicinal product designation - Guidance for sponsors submitting an application via IRIS secure online portal (available at the following LINK).