



EUROPEAN COMMISSION  
HEALTH AND FOOD SAFETY DIRECTORATE-GENERAL

Health systems and products  
Medicinal products – authorisations, European Medicines Agency

PHARM 684

**PHARMACEUTICAL COMMITTEE**  
**17 March 2015**

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**Subject: Forthcoming activities on orphan medicinal products**

**Agenda item 2f**

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Orphan medicinal products are intended for the diagnosis, prevention or treatment of life-threatening or very serious conditions that affect no more than 5 in 10,000 people in the European Union. The orphan legislation has been very successful to attract a lot of research in the field of rare diseases.

To date, the European Commission has already authorised 112 orphan medicines for the benefit of patients suffering from rare diseases. The sponsors responsible for these medicines benefit from incentives such as fee waivers for the regulatory procedures or a 10 year market exclusivity.

Equally important, the European Commission has designated 1156 products as orphan medicinal products. The sponsors developing these products benefit from incentives such as protocol assistance. This assistance should facilitate the development and authorisation of innovative medicines for the benefit of the patients.

Nevertheless, the European Commission has been made aware of some weaknesses and challenges in the field of orphans which affect the reputation and the credibility of the system.

Consequently, the European Commission has decided to take actions in this field in particular:

**1 . Streamlining the regulatory framework by reviewing certain guidelines**

The European Commission would like to clarify the requirements to facilitate entry of innovative products with a significant benefit over existing products. To this end, we need to clarify what type of data the sponsors need to show to demonstrate the significant benefit over authorised medicines.

The 2003 Communication<sup>1</sup> from the Commission on Regulation (EC) n° 141/2000 on orphan medicinal products provides interpretations of the legislation including the criterion of significant benefit or market exclusivity. The 2003 Communication will be reviewed to streamline the regulatory framework and to be adapted to technical progress. The European Commission plans to adopt the revised Communication for the 1<sup>st</sup> quarter 2016.

Moreover, once an orphan is authorised, it is not possible to authorise a similar product during ten years. We would like to ensure that the definition of 'similar' is suitable. In light of the experience to date, the European Commission will re-examine the definition of 'similar products' and will assess the need to review this definition.

In order to take an informed decision, the Commission would be interested in learning whether Member States wish to share concerns on the interpretation of the orphan legislation laid down in the 2003 Communication as regards:

- The criteria for orphan designation e.g. criterion of significant benefit;
- Procedure for designation, re-evaluation and removal of the orphan designation from the Commission registry;
- Orphan Community marketing authorisation;
- Market exclusivity and similarity.

These concerns should be communicated to [sante-pharmaceuticals-d5@ec.europa.eu](mailto:sante-pharmaceuticals-d5@ec.europa.eu) by 15 April 2015.

## **2. Updating the inventory of all incentives made available by the EU and the Member States to support research and availability of orphans.**

Article 9(3) of Regulation (EC) No 141/2000 on orphan medicinal products requires that the Commission publishes, on a regular basis, a detailed inventory of all incentives made available by the Community and the Member States to support research into and the development and availability of orphan medicinal products. That inventory shall be updated regularly. The first inventory was published in January 2001 and then updated in 2002 and in 2005. In the coming weeks, the European Commission will send a letter to all members of the Pharmaceutical Committee to collect information on national incentives for orphan medicinal products. The European Commission plans to publish the updated inventory in the 4<sup>th</sup> quarter of 2015.

In order to prepare this report on time, the European Commission would like the support of the Member States in replying to the forthcoming EC letter.

### **Action to be taken:**

For follow-up

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<sup>1</sup> <http://eur-lex.europa.eu/LexUriServ/LexUriServ.do?uri=OJ:C:2003:178:0002:0008:en:PDF>