



Pharm 785

**PHARMACEUTICAL COMMITTEE**  
**17 December 2019**

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**Subject:** Evaluation of the Orphan and Paediatric Regulations

**Agenda item 3**

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**1. BACKGROUND**

The Commission is currently finalising its evaluation on the Orphan and Paediatric Regulations under the Commission's Better Regulation principles.<sup>1</sup> We expect to publish the Staff Working Document in spring 2020.

This evaluation will give an assessment about the strengths and weaknesses of the two Regulations both separately and combined. It will provide amongst others insight into how the various incentives that are related to the legislation have been used, and the financial consequences this has resulted in (overall cost-benefit analysis and per stakeholder).

It will be based on data already collected with the report on the 10 years of the paediatric Regulation<sup>2</sup> and the 2018 study about the impact of the pharmaceutical incentives.<sup>3</sup> The findings of a study on the functioning of the Orphan Regulation have also been used and the replies to the public and targeted consultations held. The contractor presented preliminary findings from the study on the Orphan Regulation at the Pharmaceutical Committee of 1 April 2019.

In the meeting of December 17, we would like to provide you with high-level outcomes of the evaluation and start a discussion on specific aspects of the two Regulations. In order to have a fruitful discussion, we would you to reflect beforehand about your national experience with both Regulations (see three questions below).

**2. DISCUSSION**

- i) Although there has been a gradual increase in medicines for patients with rare diseases and children since the introduction of the Regulations, **there is still a very large unmet need**. This can be partly explained by the long development timelines for medicines and hence the delayed onset of the effect of the Regulation. However, as many paediatric developments are linked to an adult medicine the primary driver are

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<sup>1</sup>[https://ec.europa.eu/info/law/law-making-process/planning-and-proposing-law/better-regulation-why-and-how\\_en](https://ec.europa.eu/info/law/law-making-process/planning-and-proposing-law/better-regulation-why-and-how_en)

<sup>2</sup>[https://ec.europa.eu/health/sites/health/files/files/paediatrics/docs/2017\\_childrensmedicines\\_report\\_en.pdf](https://ec.europa.eu/health/sites/health/files/files/paediatrics/docs/2017_childrensmedicines_report_en.pdf)

<sup>3</sup>[https://ec.europa.eu/health/sites/health/files/human-use/docs/pharmaceuticals\\_incentives\\_study\\_en.pdf](https://ec.europa.eu/health/sites/health/files/human-use/docs/pharmaceuticals_incentives_study_en.pdf)

adults' needs rather than the specific needs of children. For rare diseases, the huge number of rare diseases is a limiting factor, 95% of patients still has no treatment options. The impact of **incentives** and rewards in terms of making this market segment more attractive is clearly recognized, but the current design has limitations in terms of redirecting investments in areas of unmet need.

*We would like the Committee to reflect whether there are ways to improve the use of incentives to redirect investments in areas of unmet need compared to areas where the market offers opportunities for return on investment.*

- ii) The legal mechanisms introduced by the two regulations have been constructed around the **concept of 'disease'**. For example, the orphan designation is linked to the prevalence of a particular disease and the obligation to conduct paediatric studies is dependant on whether the adult disease for which the product is developed exists also in children. However, scientific developments may lead to an increase in therapies with a relatively low prevalence, e.g. due to the increased use of biomarkers (personalised medicine) or for products based on genome editing. In addition, in some therapeutic fields, for example oncology, tissue agnostic therapies may become more relevant, which may make it difficult to define the disease in legal terms. Consequently, the current criteria used to classify a medicinal product as a treatment for a rare disease (such as prevalence) may fail to accurately capture rare diseases. Moreover, in the case of paediatric medicines, it may lead to certain adult products that could work in children (due to the mechanism of action) being excluded from the scope of the actual obligation.

*We would like the Committee to reflect on the limitations of the legal criteria used in the two regulations to identify products that may receive orphan designations or are subject to the obligations to perform paediatric studies and whether there are ways to improve those criteria.*

- iii) While both Regulations have increased the number of authorised products for children and for rare diseases, this does not automatically translate into the immediate accessibility and availability of those products for all patients in the EU. There is still a huge difference depending on where patients live. However, this is an issue, which applies to medicines in general, and not only to medicines that have been developed with the help of the two Regulations. Moreover, some of the reasons for this problem lie outside the scope of what could be addressed through this legislation. At the same time, it is recognised that the Paediatric Regulation includes some provisions to stimulate access and availability, e.g. the provision that makes the reward dependant on an authorisation in all Member States – Article 36(3) or the provision that request companies that benefitted from the reward and that lose commercial interest in the product to transfer the marketing authorisation to another company – Article 35).

*We would like the Committee to reflect on mechanisms within the scope of the two Regulations that may contribute to improving access. This may include measures linked to the supply of those medicines or linked to the incentives/rewards provided?*