



PHARMACEUTICAL COMMITTEE
23 October 2018

Subject: Update on the state-of-play of the evaluation of the Orphan and Paediatric Regulations

Agenda item 3i

The Commission published a report on paediatrics in 2017 and announced in this report that it will evaluate both the EU Regulation on Orphan medicinal products and the Paediatric Regulation. The roadmap for this evaluation was published in December 2017 (https://ec.europa.eu/info/law/better-regulation/initiatives/ares-2017-6059807_en)

The purpose of the evaluation is twofold.

1. It will give an assessment about the strengths and weaknesses of the two legal instruments separately and combined, thereby focusing on the outputs/results in products catering for a real unmet medical need taking into account the way pharmaceuticals are developed, scientific advances and changing business models.
2. It will shed light on how the various incentives that are related to the legislation have been used, and the financial consequences this has resulted in (in general and per stakeholder).

To back-up the evaluation with the necessary data, an external contractor (Technopolis Group) is currently conducting a study on the functioning of the EU Regulation on Orphan medicinal products. It will focus on its relevance, effectiveness, efficiency, coherence and EU added value.

The targeted consultations launched in September 2018 focus on national public authorities, sponsors of orphan medicines, developers of generic or biosimilar orphan medicinal products, academic experts and patient & consumer. A public consultation aiming at citizens and healthcare professionals opened this month. The final report of the study is planned for March/April 2019.

The evaluation shall be completed in the 3rd quarter of 2019 the latest (in the form of a Commission Staff Working Document).

Action to be taken:

For information