The European Commission has opened a consultation on the legislation on medicines for children and rare diseases

On 12 October 2018, the European Commission opened a public consultation on the Orphan and Paediatric Regulations. The joint evaluation aims at assessing the efficiency and effectiveness of the Regulations, particularly in the light of the latest pharmaceutical developments, including the impact of the incentives for research, development and marketing of paediatric and orphan medicines.

The Paediatric Regulation came into force on 26 January 2007, with the purpose of improving the health of paediatric populations in Europe by facilitating the development and availability of medicines for children aged 0 to 17 years. It aims at ensuring that medicines for children are of high quality, ethically researched and appropriately

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1 Available at the following LINK.
authorised, without subjecting children to unnecessary trials. The Regulation established the Paediatric Committee (PDCO), responsible for coordinating the European Medicines Agency’s (EMA) work on medicines for children, determining the studies that companies must implement as part of a paediatric investigation plans (PIPs)\(^3\).

The Orphan Regulation was introduced in 1999, and aims at facilitating the development and authorisation of medicines for rare diseases, stimulating research and bringing to the market appropriate medications so that patients suffering from rare conditions have the same quality of treatment as other patients. 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 products intended for only very small numbers of patients.

The Regulation established 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\(^4\), scientific assistance for marketing authorisations and the possibility of an EU marketing authorisation with a 10-year market exclusivity period.

Applications for orphan designations 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. To qualify for orphan designation, a medicine has to meet certain specific criteria: (i) it must be intended for the treatment, prevention or diagnosis of a disease that is life-threatening or chronically debilitating; (ii) the prevalence of the condition in the EU must be of no more than 5 in 10,000 patients or it must be unlikely that the marketing of the medicine would generate sufficient returns to justify the investment needed for its development; (iii) no satisfactory method of diagnosis, prevention or treatment of the condition concerned is in existence, or, if such a method exists, the medicine must be of significant benefit to the patients affected by the condition.

It must be noted that a considerable number of paediatric diseases also qualify as a rare disease. About 60% of designated orphan medicines are intended for paediatric use. Medicines authorised across the EU resting on the results of studies from a paediatric investigation plan are eligible for an extension of their supplementary protection certificate (SPC) of a further six months. For designated orphan medicines, the incentive is an additional two years of market exclusivity.

The consultation concerns both medicines for rare diseases and paediatric diseases that qualify as rare. Other medicines treating diseases that do not qualify as ‘rare’ remain out of the scope of the consultation.

The feedback period for the consultation will end on 4 January 2019. The consultation is addressed both to individual citizens and healthcare professionals treating patients with rare diseases (including pharmacists and ‘carers’), who are invited to share their experiences and perspectives on access to orphan medicines and on the role that

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\(^3\) A paediatric investigation plan (PIP) is a development plan aimed at ensuring that the necessary data are obtained through studies in children, to support the authorisation of a medicine for children. All applications for marketing authorisation for new medicines have to include the results of studies as described in an agreed PIP, unless the medicine is exempt because of a deferral or waiver.

\(^4\) 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.
the Orphan Regulation plays in the development of new orphan medicines.

The European Commission has commissioned the consultancies Technopolis Group and Ecorys BV to collect data and investigate the views of stakeholders on the efficiency, effectiveness and relevance of the Orphan Regulation, as well as its coherence with other regulations. The survey will be open until the 21 November 2018\textsuperscript{5}. 

\textsuperscript{5} See the following \textbf{LINK}.