Medicinal products for paediatric use

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After a first report published in 2013, the Commission presented a **second report** on the impact of the paediatric Regulation (Regulation (EC) No 1901/2006) ten years after its adoption.

The report provides an account of its achievements, both in public health and economic terms and an analysis on the extent to which its objectives have been met. It builds on a 10-year report prepared by the European Medicines Agency (EMA), a public consultation and discussions with Member States, the European Parliament and interested parties.

As a reminder, the Regulation is structured around **three main objectives**:

- to encourage and enable high-quality research into the development of medicines for children;
- to ensure, over time, that most medicines used by children are specifically authorised for such use with age-appropriate forms and formulations; and
- to increase the availability of high-quality information about medicines used by children.

The main findings of the report are as follows:

More medicines for children: the figures show that the Regulation has had a **significant impact** on the development of paediatric medicines in the Union. Pharmaceutical companies now view this development as integral to the overall development of medicinal products.

Between 2007 and 2016, **more than 260 new paediatric medicines were authorised**. In addition, the number of agreed paediatric investigation plans (PIPs) has increased significantly. This result, which would not have been achieved without specific legislation, underlines that the Regulation remains relevant. In addition, the measures taken to improve its application have gradually increased its effectiveness.

Better medicines: the last 10 years have seen some considerable progress in the **availability** of medicines for children in certain therapeutic fields because of the Regulation. Rheumatology or infectious diseases are often referred to as prime examples.

The increase in paediatric research and the number of new products with specific paediatric indications is encouraging. Those positive results do however not evenly spread among all therapeutic areas, but concentrate in some, often linked to research priorities in adults rather than children. **This shows that the Regulation works best in areas where the needs of adult and paediatric patients overlap**.

The report notes that especially, in diseases that are **rare and/or unique to children** and which in many cases are equally supported through the orphan legislation, major therapeutic advances often failed to materialise.

It seems difficult to understand why companies refrain from taking advantage of the Orphan Drug Regulation for pediatric cancers as they do for adult cancers. A huge number of new adult cancer products are thriving thanks to the Orphan Drug Regulation, but this is not the case for childhood cancer, although all are considered rare within the meaning of the Regulation.

Therefore and before proposing any amendments, the Commission intends to **take a closer look at the combined effects of the Orphan and Paediatric Regulation** through a joined evaluation of those two legal instruments aimed at supporting medicine development in subpopulations of particular need.

Reward system: the Regulation places an additional burden on pharmaceutical companies by asking them to carry out paediatric research, which they might not have undertaken otherwise. The Regulation however, links this obligation with a reward system in order to allow companies to recuperate the additional upfront costs incurred as a result of it through prolonged protection period.

Still the use of rewards was limited to **55 % of the completed PIPs and there are instances of over- or under compensation** pointing to certain limitations of the current system. Additionally, the paediatric use marketing authorisation (**PUMA**) concept with its specific reward has failed to deliver.

Next steps: this report marks not the end, but an essential intermediate step in the debate on a joint vision about the future parameters for paediatric and orphan medicines. The further evaluation supporting this process aims at providing results **by 2019** so to allow the next Commission to take informed decision about possible policy options.

In the meantime, the Commission is committed to a **positive agenda of concrete actions** in order to streamline the current application and implementation together with EMA wherever needed. This includes:

- providing additional **transparency** of new products authorised with paediatric indications;
- analysing the experience with use of deferrals and consider changes in practice to ensure **speedier completion of PIPs**;
- revisiting processes and expectations in the context of handling of applications for PIPs and if necessary adapt the corresponding Commission guideline;
- exploring opportunities to discuss **paediatric needs** in an open and transparent dialogue involving all relevant stakeholders like academia, health care providers, patients/care givers, paediatric clinical trial networks, industry and regulators; delivering **regular updates** about development and trends of the paediatric medicines landscape in the EU;
- fostering international cooperation and harmonisation.

Additionally, it will further support high-quality healthcare and research for children through projects such as the **European Reference Networks**, which connect health care providers and centres of expertise. Those networks have the potential of significantly improving access to diagnosis and treatment in the short term and to make a difference in terms of child health.