

Stimulating the development of new medicines for children

The pharmaceutical industry is committed to developing new medicines for children, fully integrating paediatric research in its medicine development programs, and working with other stakeholders to address the Commission's recommendations.

We were at a very different place 15 years ago, when talking about availability of licensed paediatric medicines in Europe and the rest of the world. Over the last decade we have seen exciting new treatments that were enabled through scientific breakthroughs allowing better management and even cure of deadly diseases such as HIV, Hepatitis C or certain cancers that are also applicable to paediatric populations.

In the case of vaccines, paediatric development has always been the core activity in research and development given that the prevention of most infectious diseases occurs primarily during childhood. Vaccines have had a large positive impact on children's health in Europe and worldwide.

The Paediatric Regulation has contributed and will continue to contribute to ensuring that scientific breakthroughs are translated into new treatments for children. We believe that its implementation can be optimized to improve the efficiency and speed of paediatric development and foster a more collaborative research environment.



The Paediatric Regulation in force since 2007 has been successful by stimulating the delivery of over **260 new medicines and indications**¹ for treating children, for example in areas like HIV, cancer and rheumatoid arthritis.



New formulations **suitable for children** have been developed to enable children to take their medicines more easily, and research continues in this innovative area.



The number of paediatric development programmes is increasing and many **paediatric clinical trials** addressing unmet medical needs are ongoing. The proportion of paediatric clinical trials has increased by **50% in 2007-2016** (from 8.25% to 12.4% of all trials conducted in the EU)².



The landscape of paediatric clinical research is evolving with **growing infrastructure** and overall **more clinical trials** to support regulatory submissions. Because of the limited number of paediatric patients that can be included in studies, developing medicines for children is necessarily a **global exercise**.

We believe that:



The implementation of the Paediatric Regulation can be further **improved through pragmatic measures**. We do not favour a re-opening of the legislation but propose non-legislative changes that would have a faster impact, as well as more collaborative research programmes.

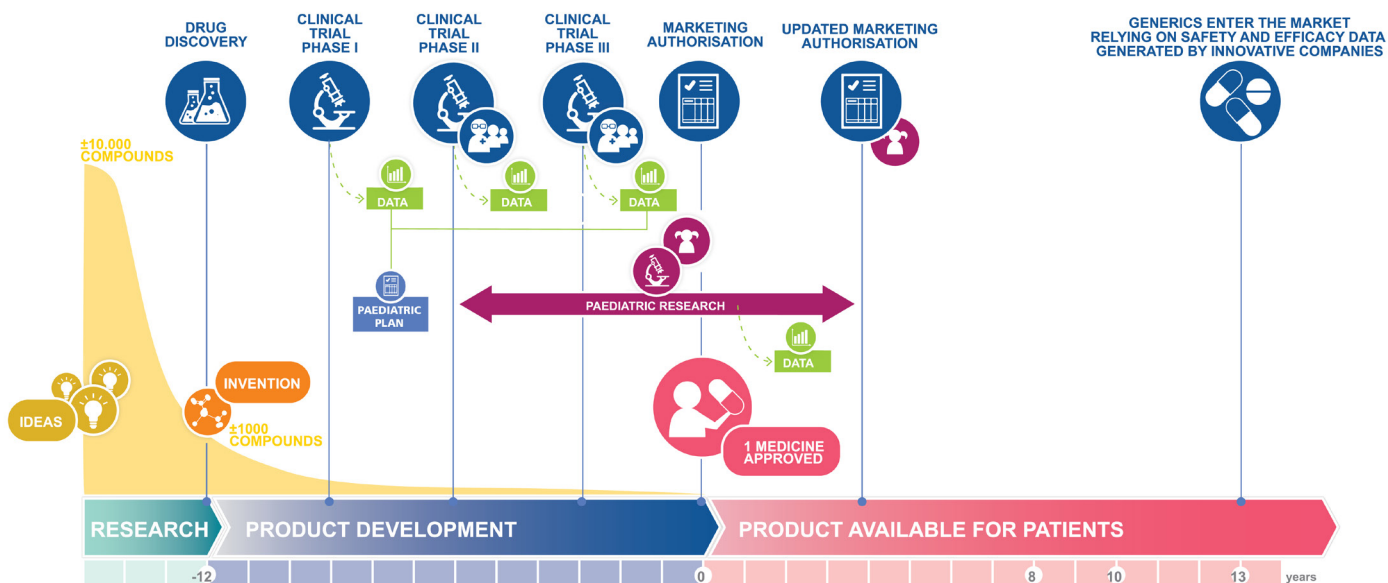


More can be done to increase the number of medicines for children given that important unmet needs remain for the treatment of childhood conditions. Industry is fully committed to this task and stands ready to increase collaboration with the paediatric community in research and development through programmes such as IMI.

Improving development speed and efficiency within the current framework

We are working on a number of pragmatic steps and measures that can be taken to optimise implementation of the Regulation in the short term that will ultimately improve the efficiency and speed of paediatric development:

- * **An inventory of disease-based unmet paediatric needs, based on the existing requirements of Article 43 of the Paediatric Regulation**, to provide a common basis for strategic decision making on paediatric medicine development. The inventory should indicate clearly for each need if there is research on-going and what type of research, ensuring transparency for all stakeholders of areas where research is most needed and to avoid that the paediatric population is subjected to unnecessary or unfeasible trials. Multiple stakeholders (industry, regulators, epidemiologists, patient groups, paediatric networks) should be involved in this assessment.
- * **Improving the efficiency of paediatric investigation plans (PIPs) through better integrated scientific and regulatory dialogue, leading to a model where the PIP develops with the evolution of scientific knowledge.** The improved PIP process will lead to agreement of development plans that fit better within the global drug development process. It is expected to improve the scientific credibility of the PIP, remove the need for long deferrals for study starting dates and reduce the need for multiple modifications, offering greater certainty to all that the agreed PIPs can be effectively completed.
- * **Improving the international collaboration and references framing the discussion for potential paediatric development plans to ensure a clear and predictable outcome, particularly in the field of oncology.** The US efforts led by FDA on molecular targets for oncology can be leveraged to facilitate global convergence.



¹ State of Paediatric Medicines in the EU: 10 years of the EU Paediatric Regulation, Report from the European Commission to the European Parliament and the Council, available at: https://ec.europa.eu/health/sites/health/files/files/paediatrics/docs/2017_childrenewmedicines_report_en.pdf

² ibid.