Fostering the development of medicines for children in the EU

efpia

Since 2007, the Paediatric Regulation has contributed to translating scientific breakthroughs into new treatments for children.

296 new paediatric **medicines approved** between 2007 and 2019[1].

50% increase in proportion of clinical trials including children (2006–2016)[2].

Paediatric medicine development has **become an integral part** of the overall development of medicines[3].

Major advances in paediatric treatment:

More than **80%** of children diagnosed with cancer now survive 5 years or more.[4]

Formulations to treat children with HIV. Treatments to prevent mother-to-child transmission of HIV.

First treatment for Hepatitis C.

Obligations and rewards under the provisions of the Paediatric Regulation

A pharmaceutical company develops a medicine **targeting a specific condition** in adults.

Childhood diseases have specificities which **need to be taken into account** during drug development. In line with the Paediatric Regulation, the company needs to develop the **same medicine for children**.
A Paediatric Investigation Plan (PIP) is agreed with the European Medicines Agency (EMA) for every medicine in development, **unless a waiver is granted**. The PIP describes the company development strategy i.e. how and by when data will be generated for use of the medicinal product in children.

Developing specific medicines for children requires a **great deal of effort** from the companies. This is why the legislators have designed a **set of rewards** to compensate for the additional effort incurred.

PIP Waiver:
-medicine is **not safe or effective for children**;
-medicine is **not expected to be of use** in children;
-condition targeted by the medicine in development **does not exist in children.**

A 6-month extension to the supplementary protection certificate (SPC) for **the product**.

or

If the product is an orphan medicinal product, the **10 years of market exclusivity** provided by the Orphan Regulation can be **extended by a further 2 years** in the **specific orphan indication**.

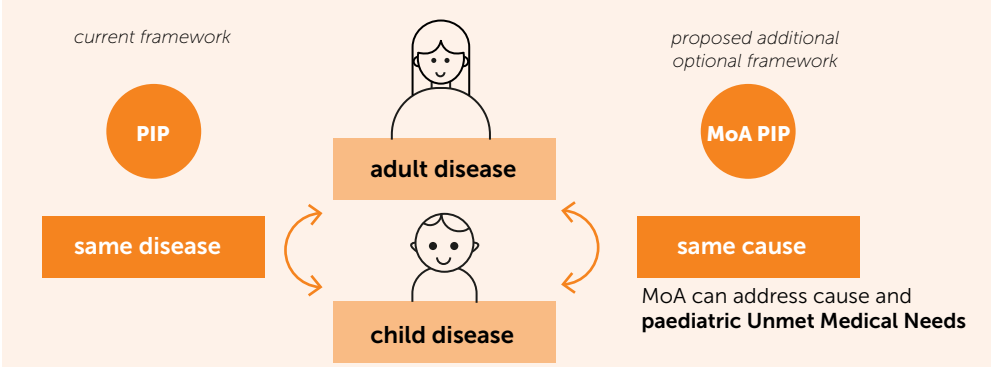
Towards a 'child-centric' approach
The EU Paediatric Regulation has successfully **stimulated the development of new medicines** for children. Existing incentives and rewards remain essential to stimulate innovation, but we know that **more can be done to foster research** in disease areas affecting exclusively children, specially where an unmet medical need exists.

Creating an innovation ecosystem to foster research into medicines for children

The upcoming review of the Paediatric Regulation provides an opportunity to evolve from an 'adult-centric' approach to a 'child-centric' approach. EFPIA proposes to:

Address paediatric Unmet Medical Needs (UMN) via Mechanism of Action (MoA) PIPs

EFPIA is proposing a framework for the "MoA PIP", based not on the adult indication, but on a paediatric unmet medical need. In other words, the PIP will be designed having in mind how the product works (its mechanism of action) and what unmet need in children it might be able to address. This would mean that a medicine developed for an adult disease may also be studied in a different childhood disease because both diseases have the same cause, and the treatment may work for both. This child-centric PIP approach implies significant efforts and scientific challenges. An additional reward for developers would be fair and appropriate.



This concept requires a revision to how a condition is defined.

Definition of Condition based on current scientific knowledge

Thanks to advances in science, we are now able to **understand the pivotal causes of many diseases**. This is a game changer to discovering new treatments: many medicines are now developed to **target the cause of a given disease**.
The mechanism of action (MoA) of such a medicine can make it suitable for use in more than one condition if each condition can be shown to be associated to the same pivotal cause.
We therefore propose to **adjust how a disease (or a condition) is defined**, to include the cause of the disease, where this link is scientifically proven **based on the most up to date evidence**.

Better integrate paediatric development discussions into the overall regulatory development dialogue with regulators

Discussions with regulators should also include the **data generation needs for paediatric patients**. Usually, these discussions also need **input from other regulators** such as the FDA to **achieve alignment** on a global paediatric programme.

With **600 new paediatric medicines in development**, we won't stop pushing the boundaries of what we thought was impossible. We are looking forward to working with all partners to create a regulatory environment **centred around children's needs and scientific progress**.

1 https://www.frontiersin.org/articles/10.3389/fmed.2021.593281/full#F1
2 Commission report – implementation of the paediatric regulation, 2017, available at: https://ec.europa.eu/health/system/files/2017-11/2017_childrensmedicines_report_en_0.pdf
3 Ibid.
4 https://worldspanmedia.s3-eu-west-1.amazonaws.com/media/siope/PDF/the-siope-strategic-plan.pdf

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