



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Human Medicines Development and Evaluation

Successes of the Paediatric Regulation after 5 years August 2007-December 2012

The [Paediatric Regulation](#) has three main objectives:

- To promote high quality research with children to inform on the quality, safety and efficacy of medicines that children (from birth to less than 18 years) will receive;
- To provide more information on the use of paediatric medicines
- To allow the authorisation of medicines for diseases that affect children, with age-appropriate pharmaceutical forms and composition (formulation).

More high quality research in paediatric medicines:

1. The European Medicines Agency (EMA) and its Paediatric Committee have agreed more than 600 Paediatric Investigation Plans (PIPs) with pharmaceutical companies, to provide data on the efficacy and safety of medicines for diseases of children.
2. More paediatric clinical trials were done (data from EudraCT, accessible at the [European Clinical Trials Register](#)):
 - Around 350-400 clinical trials per year, including children (0-18 years);
 - The proportion of clinical trials including children has increased in the last 6 years, to approximately 10%.
3. Neonates are the most neglected group when it comes to medicines development, but medicines very often need to be used without any proper data on efficacy and safety. The EMA requested the inclusion of more neonates and infants in clinical trials to obtain these data in a safe way; currently, 30% of the paediatric investigation plans include studies with neonates. More neonates and infants (26%) have been included in trials in recent years (EudraCT figures).
4. A network of paediatric research networks has been created by the EMA ([Enpr-EMA](#)), putting together 18 research networks that meet research quality criteria. Enpr-EMA works by fostering collaboration from within and outside the European Union (EU), including between members, patients associations, academia and the pharmaceutical industry.



More information on medicines:

Better information on use in children of medicines is regularly included in the medicines' Product Information, also called the Summary of Product Characteristics (SmPC) and the Package Leaflet. Changes include:

- 221 changes about safety and efficacy, from submission of old or new studies;
- 89 additions of dosing information for children (a direct consequence of studies from PIPs),
- 77 other modifications to add new study data;
- 61 mentions of 'Deferrals' (indicating that paediatric data are expected but later) and of Waivers (where paediatric data are not requested) in the product information.

For the first time ever, results of paediatric medicines studies are available to the public and health professionals. These studies had not been seen by regulatory authorities (and often never published).

More than 3,200 reports on the results of paediatric studies are now included in a searchable and [specific database](#) published by the EMA; approximately 3,000 more are being added gradually.

General information on paediatric clinical trials can now be found on the [European Clinical Trial Register](#) (EU-CTR), which presents protocol-related information on all paediatric trials with investigators in the EU, or anywhere in the world when they are included in a PIP.

Patients, parents and healthcare professionals can also access and search [EMA Decisions on PIPs](#) by condition or therapeutic areas, to identify the development and future clinical trials with children for a given medicine.

More medicines for children, with age-appropriate forms

A. Medicines under the responsibility of the EMA and the European Commission:

- Medicines authorised initially with a paediatric indication: 34% (31/152), including 10 linked to a PIP;
- New paediatric uses authorised for existing medicines: 39, including 18 linked to a PIP;
- New pharmaceutical forms adapted for children: 15, including 3 linked to a PIP.

B. Medicines under the direct responsibility of EU Member States:

- Medicines authorised initially with a paediatric indication: 3 linked to a PIP;
- New paediatric uses authorised for already existing medicines: 33, including 12 linked to a PIP;
- New pharmaceutical forms adapted for children: 11, including 6 linked to a PIP.

No delays for adult medicines

Developing medicines for children did not cause delays to the authorisation of adult medicines.

No unnecessary studies with children

The EMA with its Paediatric Committee have to ensure that children are not subject to unnecessary studies and work on maximising the use of existing data, developing new tools such as extrapolation of data from adults, or modelling and simulation of the behaviour of medicines in children.

Links:

- Information on the Paediatric Regulation:
http://www.ema.europa.eu/ema/index.jsp?curl=pages/regulation/general/general_content_000023.jsp&mid=WC0b01ac05800240cd
- [Report from the European Medicines Agency to the European Commission](#) after 5 years of entry into force of the Paediatric Regulation.
- The EMA and [Medicines for Children](#)
- The [Paediatric Committee](#) of the EMA