

# Better medicines for children

*Overview of the  
European Medicines Agency's  
role in the European  
regulatory environment for  
paediatric medicines*



*A new Paediatric Regulation<sup>1</sup> entered into force in the European Union on 26 January 2007.*

The objective of the Paediatric Regulation is to improve the health of children in Europe by:

- facilitating the development and availability of medicines for children aged 0 to 17 years,
- ensuring that medicines for use in children are of high quality, ethically researched, and authorised appropriately,
- improving the availability of information on the use of medicines for children,

without:

- subjecting children to unnecessary trials,
- or delaying the authorisation of medicinal products for use in adults.

The Paediatric Regulation dramatically changes the regulatory environment for paediatric medicines in Europe.

This leaflet provides a brief overview of the key aspects of the changes in which the European Medicines Agency (EMA) has a role to play.

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<sup>1</sup>Regulation (EC) No 1901/2006 of the European Parliament and of the Council on medicinal products for paediatric use, amended by Regulation (EC) No 1902/2006.

## **New system of rewards, incentives and obligations for pharmaceutical companies**

### *For unauthorised medicinal products*

As of 26 July 2008 (18 months after entry into force of the Regulation), there will be an obligation to submit the results of studies conducted according to a paediatric investigation plan in order to have a valid application for marketing authorisation throughout the EU. This obligation can be waived for medicines that are unlikely to benefit children. In some cases, studies may be deferred until after the medicine has been authorised for use in adults.

The reward for conducting the paediatric development in compliance with a paediatric investigation plan is a six-month extension of the supplementary protection certificate, provided that the results are included in the product information and that authorisation is obtained in all EU Member States.

### *For orphan medicinal products*

The obligations for orphan-designated medicinal products are the same as for unauthorised medicinal products. The reward is two years of market exclusivity in addition to the existing 10-year exclusivity awarded under the EU Orphan Regulation, provided that the results are included in the product information and that authorisation is obtained in all EU Member States.

### *For authorised, patented medicinal products*

As of 26 January 2009 (24 months after entry into force of the Regulation), there will be an obligation to submit the results of studies conducted in accordance with an agreed paediatric investigation plan when seeking a variation or extension of the marketing authorisation for a new indication, new route of administration or new pharmaceutical form. As with new medicines, waivers or deferrals may also be granted, and the reward is a six-month extension of the supplementary protection certificate.

### *For off-patent medicinal products*

Off-patent medicines developed specifically for paediatric use and with an appropriate formulation can benefit from a new marketing authorisation — the paediatric-use marketing authorisation (PUMA) — which benefits from 10 years of data protection.

## Paediatric investigation plans (PIPs)

A drug-development plan — known as a paediatric investigation plan — must be agreed in advance by the EMEA's Paediatric Committee. The PIP shall cover the timing and measures proposed to obtain a paediatric indication, with an age-appropriate formulation, in all paediatric subsets affected by the condition. Once agreed by the Paediatric Committee, the PIP is binding on companies developing a medicinal product for the EU.

A draft guideline on the requirements for PIP applications, requests for waivers or deferrals, the operation of compliance checks, and criteria for assessing significant studies was released by the European Commission on 31 January 2007, with a final version being expected soon.

The EMEA will publish templates for PIP applications and requests for waivers and deferrals on its website.

## New EMEA committee of experts: the Paediatric Committee

A new Paediatric Committee will be established at the EMEA in 2007. It will be composed of five members of the EMEA's Committee for Medicinal Products for Human Use, representing five EU Member States, and one expert representing each of the 22 other EU Member States. Additional experts will represent Iceland, Liechtenstein and Norway. Six representatives of patients' or families' organisations and healthcare professionals will also join the Paediatric Committee once they have been appointed by the European Commission.

This main responsibility of the Paediatric Committee will be to decide on the content of paediatric investigation plans.

## Free scientific advice for paediatric medicines

Scientific advice became free for paediatric development questions with the entry into force of the Paediatric Regulation.

## Improved communication and transparency of paediatric information

Certain aspects of paediatric investigation plans and waivers will be made public after deletion of commercially confidential data. Paediatric data and information will be included in product information, including when study results are negative.

Details and results of clinical trials performed in children will be made public, using the EU clinical trials database (EudraCT) for paediatric trials performed inside or outside the EU.

## Measures to monitor long-term efficacy and potential adverse drug reactions

The Paediatric Regulation reinforces existing pharmacovigilance activity in the EU and, where appropriate, maintains the requirement to have a risk-management system in place.

Guidance on paediatric pharmacovigilance and risk-management plans is available on the EMEA website.

## Inventory of paediatric needs

The Paediatric Committee will take into consideration the needs of children when assessing a paediatric investigation plan. Several lists identifying needs in major therapeutic areas are published on the EMEA website. The Paediatric Committee will work to establish an inventory of paediatric needs based on a survey of use in the EU Member States.

EU funding is already available for paediatric research, especially for products that are off patent and identified in one of the priority lists. The first calls for proposals have been launched by the European Commission's Directorate-General for Research.

## Paediatric research network

The EMEA is developing an EU-wide network of bodies conducting paediatric research. The objectives are to build up competences at European level, facilitate the conduct of studies and avoid duplication of studies.

## Submission of available paediatric data

All available results of paediatric studies and trials should be submitted by marketing authorisation holders to the national competent authorities or to the EMEA before 26 January 2008. Further paediatric studies should be submitted on an ongoing basis within six months of their completion.

## Symbol on all paediatric medicines

A symbol will be included on the packaging of all medicinal products with a paediatric indication, to help identify the product as being authorised for paediatric use. The symbol will be selected by the European Commission on a recommendation from the Paediatric Committee.

## EU-US cooperation on development of paediatric medicines

The EMEA and the US Food and Drug Administration (FDA) are working to establish principles for interaction and exchange of information on paediatric matters, with a view to global development of medicines for children.

## NEED MORE INFORMATION?

Visit the EMEA website:

[www.emea.europa.eu](http://www.emea.europa.eu)

(Select 'Medicines for children' from the 'Fast track to a topic' drop-down menu.)

Questions on paediatric issues may be submitted by e-mail to: [peg@emea.europa.eu](mailto:peg@emea.europa.eu)

Questions on general issues may be submitted by e-mail to: [info@emea.europa.eu](mailto:info@emea.europa.eu)

Further information on the regulatory environment for medicines in Europe is also available from the European Commission's DG Enterprise and Industry ([http://ec.europa.eu/enterprise/index\\_en.htm](http://ec.europa.eu/enterprise/index_en.htm)) and DG Research ([http://ec.europa.eu/dgs/research/index\\_en.html](http://ec.europa.eu/dgs/research/index_en.html)).