

EPHA Briefing for members

Subject	Draft Regulation on Paediatric medicines
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Date	01 November 2004

On 29 September 2004 the European Commission published a draft ' Regulation on Medicinal products for paediatric use' that will aim to improve children' shealth in Europe by ensuring the authorisation of medicines that have been specifically researched and developed to meet their therapeutic needs.

Background

Extensive testing is required on all medicines authorised for use in adults to ensure they are safe, of high quality and effective. **However, more than 50% of the medicines currently used to treat children have neither been tested or authorised for paediatric use.**

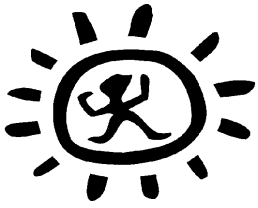
There are about 100 million children in Europe, more than 20% of the population. This is a large vulnerable group with developmental, physiological and psychological differences to adults, therefore making it crucial that medicines are developed specifically for the needs of children.

At present, children are prescribed medicines that are untested and unauthorised for use to treat children, which means that doctors cannot be sure the medicine will be truly effective, what dose is appropriate or exactly what the side effects may be.

The European Commission states that market forces have failed to stimulate adequate development of medicines specifically for children. The main reason listed for this is that the potential return on investment for children specific medicines does not justify the financial costs of research and development. Therefore public authorities need to intervene to address the market failure and find means of stimulating innovation.

Currently almost all areas of paediatric medicine lack appropriately researched and authorised products. Examples of diseases and conditions not covered include;

- Meningitis,
- Tuberculosis,
- AIDS,



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- Diabetes,
- Asthma,
- Arthritis,
- Cardiac diseases,
- Epilepsy,
- Glaucoma,
- Malaria.

The key objective of the Regulation;

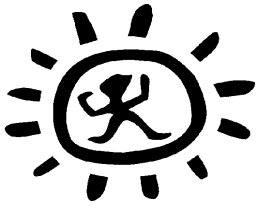
The Commission' stated aim is to increase the development and authorisation of medicines for use in children while ensuring that children' s medicines are subject to high quality research. It is also concerned that children are not subjected to unnecessary clinical trials as well as improving the information available on medicines for children.

The key elements of the new proposal:

The proposal covers both medicines that are patented (newer) and those that are non-patented (older). The intention of the measures is to provide both incentives and requirements to the Pharmaceutical industry to carry out studies in children and to increase development of new child specific medicines.

Newer (patented) medicines;

- A requirement at the time of marketing authorisation applications for data on the use of the medicine in children.
- A system of waivers from the previous requirement for medicines unlikely to benefit children and a system of deferrals to ensure medicines are tested in children only when it is safe to do so and to ensure the requirements do not delay the authorisation of medicines for adults)
- A reward for studying medicines for children of 6-months extension to the supplementary protection certificate - in effect, a six-month patent extension on the medicine.



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Older (non-patented) medicines ;

• Paediatric Use Marketing Authorisation (PUMAs) will be established to stimulate the development of studies on existing non-patented medicines (often produced by a number of different companies) in children. The incentive will be 10 years of protection for any data from the new studies on existing non-patented medicines.

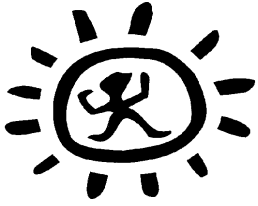
A number of measures will address both older and newer medicines;

- **A new expert committee within the European Medicines Evaluation Agency (EMA)** to assess and agree companies' investigation plans. The committee will have expertise in all areas of the research, development, authorisation and use of medicines in children.
- An EU inventory of the therapeutic needs of children and an EU network of investigators and trial centres to conduct the studies required;
- Increased safety monitoring for children' medicines and **compulsory submission by industry of existing studies in children;**

The proposal is based on a system of requirements and rewards to drive innovation. The burden of costs will be shared between the industry and society at large, this, it is envisaged, will minimise non-compliance by the industry. The combined measure of incentives and obligations exist in the United States where legislation similar to that proposed by the European Commission, has been in place in the since 1997. As of February 2004, 63 new paediatric labels and 661 studies have been requested.

The Commission (DG ENTERPRISE) conducted an '**Extended Impact Assessment**' on the draft legislation that addressed the social, economic and environmental impacts of the proposal on the different stakeholders (e.g. children and their families, healthcare workers, the pharmaceutical industry). Their conclusion was that the proposal would lead to more and better medicines for children with the pharmaceutical industry benefiting through greater innovation.

The Extended Impact Assessment also highlights that there will be costs. The requirement for phase III clinical trials in children will cost the industry an estimated average of EUR 4 million per product. This extra cost added to the cost of the medicine will see an increase, it is predicted, of less than 0.5% in the price of individual medicines. (see <http://pharmacos.eudra.org/F2/home.html> and look under 'Extended Impact Assessment' for details).



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When will it become law?

The proposal will be delivered to the Council and the European Parliament where it will go through the 'co-decision' procedure. A rapporteur will be designated for the first reading by the European Parliament which will be led by the Environment committee, who will be joined by the committee for Internal Market and Consumer Protection. If the Council accepts the outcome of the parliamentary hearing the proposal will be adopted. **The earliest that this proposal is would become law will be in late 2006.** If the council does not accept the proposal it will go to a second European Parliament reading.

The text of the proposal, an impact assessment study and other background documents can be downloaded from a dedicated page on the website of the Enterprise Directorate-General. <http://pharmacos.eudra.org/F2/Paediatrics/index.htm>