MEMO/04/XXX

Brussels, 29 September 2004

Regulation on medicines for children: frequently asked questions

What is the proposal on medicines for children, what is the objective?

On 29 September 2004, the European Commission adopted a proposal for a regulation of the Council and the European Parliament on medicinal products for paediatric use. The overall objective is to improve the health of the children of Europe by increasing the research, development and authorisation of medicines for use in children. This will be achieved through: increasing the development of medicines for use in children, ensuring that medicines used to treat children are subject to high quality research, ensuring that medicines used to treat children are appropriately authorised for use in children, and, improving the information available on the use of medicines in children.

Why do we need a proposal?

Before any medicine is authorised for use in adults, the product must have undergone extensive testing including pre-clinical tests and clinical trials to ensure that it is safe, of high quality and effective. In contrast, it has been demonstrated that more than 50% of the medicines used to treat the children of Europe have not been tested for use in children and are not authorised for use in children. Why should this matter? The answer is clear: the health of the children of Europe is suffering from a lack of testing and authorisation of medicines for their use. Every time a doctor in Europe writes a prescription for a child for an untested, unauthorised product, that doctor can not be sure the medicine will be effective, can not be sure what dose is appropriate and can not predict what adverse reactions (side effects) the child may suffer. Furthermore, new innovative products developed by the pharmaceutical industry to meet the therapeutic challenges we face today are denied to children. Innovative medicines can and do save lives and the children of Europe deserve at least the same access to such innovation as that enjoyed by adults.

The absence of suitable authorised medicinal products to treat conditions in children is an issue that has been of concern for some time. It results from the fact that frequently pharmaceutical companies do not perform the necessary research and development to adapt medicinal products to the needs of the paediatric population. This leaves no alternative to the prescriber than to use products “off-label” and use unauthorised (unlicensed) products with the associated risks of inefficacy and/or adverse reactions. In addition, existing data which could provide useful and important information are frequently not made available to the health practitioner. This is particularly ironic considering that our modern system of medicines regulation, that ensures the high standards of safety, quality and efficacy necessary for the authorisation of medicinal products for use in adults, was developed primarily in response to therapeutic disasters, such as the thalidomide tragedy of the 1950s and 60s, that occurred in children. Yet today children continue to be exposed to risks, and at the same time miss out on therapeutic advances.

How has this situation arisen?

Industry has a free choice as to what medicines to develop, and if successful, authorise and market. Industry bases its choice on potential revenue from sales balanced against the costs of research and development, manufacturing and marketing. The main drivers of overall return on investment are the size of the pharmaceutical market and the price achievable within the market-place. The number of children suffering specific diseases is generally lower than the number of adults and, in terms of research, “children” can not be considered a single population (consider a premature new-borne compared to a fifteen-year old) so studies may be more complex. The current situation in the EU regarding medicines for children is clear evidence that market forces alone are insufficient to stimulate adequate research into and authorisation of medicines for children. The industry has considered that for many childhood diseases the potential return on investment is insufficient to justify such investment in research and development.

What did the Commission do to develop its proposal?

A Council resolution in December 2000 invited the Commission to find solutions to the issue of inadequate medicines for children. The Commission has intensively researched the problem and potential solutions. Part of the research has included study of how other regions are tackling this issue and how regulation has dealt with similar but distinct problems such as how to stimulate the development and authorisation of medicines for rare diseases (orphan medicines).

Because of the complexity of healthcare delivery and the pharmaceutical sector, the Commission has conducted a detailed assessment of the social, economic and environmental impacts of it proposal on the different stakeholders involved (e.g. children and their families, healthcare workers, the pharmaceutical industry and those that pay for medicines). The results of the ‘Extended Impact Assessment’ show that the proposal will lead to the availability of more and better medicines for children and that the pharmaceutical industry will benefit through increased innovation (see http://pharmacos.eudra.org/F2/home.html and look under ‘Extended Impact Assessment’ for details).

What are the key measures proposed?

The key measures included in the draft paediatric regulation are:

Newer medicines

- a requirement at the time of marketing authorisation applications for new medicines and line-extensions for existing patent-protected medicines for data on the use of the medicine in children resulting from an agreed paediatric investigation plan;
- a system of waivers from the requirement for medicines unlikely to benefit children;
- a system of deferrals of the requirement to ensure medicines are tested in children only when it is safe to do so and to prevent the requirements delaying the authorisation of medicines for adults;
- excluding orphan medicines, a mixed reward and incentive for compliance with the requirement in the form of six-months extension to the supplementary protection certificate (in effect, six-month patent extension on the active moiety);
- for orphan medicines, a mixed reward and incentive for compliance with the requirement in the form of an additional two-years of market exclusivity added to the existing ten-years awarded under the EU orphan regulation;

Older medicines

- a new type of marketing authorisation, the Paediatric Use Marketing Authorisation (PUMA), which allows ten-years of data protection for innovation (new studies) on off-patent products;
- amended data requirements for PUMA applications to attract SMEs including generics companies;
- a reference in the explanatory memorandum to the establishment, via a separate initiative, of an EU paediatric study program Medicines Investigation for the Children of Europe (MICE) to fund research leading to the development and authorisation of off-patent medicine for children.

Old and new medicines

- the establishment of an expert committee, the Paediatric Committee within the EMEA;
- measures to increase the robustness of pharmacovigilance (safety monitoring) for medicines marketed for children;
- a requirement for industry to submit to the authorities study reports they already hold on use of their medicines in children, to maximise the utility of existing data and knowledge;
- an EU inventory of the therapeutic needs of children to focus research, development and authorisation of medicines;
- an EU network of investigators and trial centres to conduct research and development on medicines for children;
- a system of free scientific advice for the industry, provided by the European medicines Agency (EMEA);
- a database of paediatric studies.

When will this become law?

The proposal will now be delivered to the Council and the European Parliament where it will go through the co-decision procedure. The earliest that the proposal is likely to become law is late 2006.

For more frequently asked question see:

http://pharmacos.eudra.org/F2/home.html

See also IP/04/xxx

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