Medicines in Europe Forum

Proposal for a regulation on medicinal products for paediatric use:

YES to research that truly benefits children!

Dear Sir/Madam

The Medicines in Europe Forum would like to reiterate its commitment to the basic principles of the proposal for a regulation on medicinal products for paediatric use. YES, some children need drugs. YES, it is necessary to encourage research leading to the development of medicines that meet children's real needs.

However, as the proposal for a European regulation is essentially modelled on the "Pediatric rule" developed in the USA in 1997-1998, it is instructive to take account of the American experience gained since its introduction.

The USA is more stringent. The Pediatric rule had been in force for a very short time before it was considerably amended by the "Food and Drug Administration Modernization Act" and the "Pediatric Research Equity Act" (PREA 2003) (1). The regulations now allow the FDA to demand clinical trials on some drugs (even going so far as to specify the design of the trial), based on a list of needs and priorities drawn up by the FDA and the National Institute of Health (NIH) (2,3). This provision was added following the observation that in developing medicinal products for paediatric use, the pharmaceutical firms do not spontaneously focus their R&D efforts on the priority needs of children. Nevertheless, the system currently being proposed in Europe relies solely on the drugs firms taking the initiative. The aim of the amendments supported by the Medicines in Europe Forum is to ensure that, following the example of the USA, needs and priorities are defined before rewards are handed out.

Furthermore, the American system of conditions and incentives relies on the notion of "*meaningful therapeutic benefit over existing therapies for pediatric patients*", in other words that "*the drug or biological product would represent a significant improvement in the treatment, diagnosis, or prevention of a disease, compared with marketed products adequately labeled for that use in the relevant pediatric population*" (1). The amendments to the proposed Regulation relating to "added therapeutic value" supported by the Medicines in Europe Forum ask no more than this.

The MEPs who tabled amendments aiming to identify children's needs and demanding "added therapeutic value" are not in any way "anti-research". Quite the opposite: they encourage research with the proviso that it meets real needs, which is, of course, the fundamental role of research that is beneficial to society; and they are seeking to take fully into account, in a balanced way, the American example that forms the basis of the proposal for a European regulation.

Make the best use of public money to reward only the best. The amendments designed to establish a link between reward and usefulness are not "anti-research", nor are they "anti-incentive". They are simply based on the principle that rewards should be proportional to effort, which is the only

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way to encourage optimal research. Especially as new demands by the pharmaceutical industry are looming on the horizon (6 months longer to produce antibiotics under the Bioshield II Act in the USA, 6 months longer requested to conduct research into the "neglected" diseases of the poor countries, etc.).

Daily examples demonstrate the urgency of amending the proposed Regulation. The Medicines in Europe Forum maintains that the proposal for a Regulation on medicinal products for pediatric use will not yield the best results unless it is amended to ensure:

1- that children's real needs are defined and rigorously evaluated (to avoid, for example, instances such as that of *desloratadine* (anti-allergic drug) which has just been granted a prescription extension in rhinitis to include children from the age of 1, even though the European Medicines Agency recognises that we do not yet know how to define infant rhinitis);

2- proven added therapeutic value (for example to prevent medicines such as *sildenafil* (Viagra°), a likely future candidate for being prescribed to children for pulmonary arterial hypertension, being marketed without having been compared to existing treatments (*bosentan,* another oral treatment which has already been the subject of a paediatric evaluation));

3- completely transparent processes (so as not to repeat the French case of *alprazolam* (tranquilliser), whose directions for use in children appeared surreptitiously, when even the official information on this drug recognised that there was no specific study on its paediatric use;

4- proactive collection of pharmacovigilance data (to avoid scandals like those of adolescents being prescribed certain antidepressants where the risk of self-harming and suicidal behaviour associated with the drugs was concealed and denied for a long time);

5- rewards commensurate with the pharmaceutical firms' efforts (because top-selling drugs currently obtain authorisation to be prescribed to children on the basis of insufficient evidence. For example: *sumatriptan,* not compared to other migraine treatments; *tramadol* (analgesic), compared to a drug used at too low a dose to be effective).

European children are entitled to benefit from research that best corresponds to their real needs, and medicines whose therapeutic benefit is properly evaluated, by comparison with existing drugs. Responding to children's needs in the best possible way: that is the challenge in the current debate on the proposal for a Regulation.

The Medicines in Europe Forum

1- http://www.fda.gov/opacom/laws/prea.html
2- http://www.actmagazine.com/appliedclinicaltrials/article/articleDetail.jsp?id=140817
3- http://www.bio.org/reg/action/pedhist.asp?p=yes&

The Medicines in Europe Forum brings together several dozen patients groups, health professionals, consumers and organisations paying for medicines in a number of EU countries.

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