

Consultation response

Medicines and Healthcare products Regulatory
Agency
Room 16-162
Market Towers
1 Nine Elms Lane
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DATE: 17 August 2005
TO: Caroline Brennan
RESPONSE BY: Kate Webb

MLX 323: Consultation on the European Commission's proposal for a regulation of the European Parliament and of the Council on Medicinal Products for paediatric use and amending council regulation (EEC) number 1768/92, Directive 2001/83/EC and Regulation (EC) number 726/2004

1. Which? is an independent, not-for-profit consumer organisation with around 700,000 members. Based in the UK, we are the largest consumer organisation in Europe. Entirely independent of government and industry, we actively campaign on behalf of consumers and are funded through the sale of our Which? range of consumer magazines and books.
2. Our health campaign aims to ensure all consumers have access to safe, high-quality and patient-focused healthcare whenever and wherever they need it, and they have the necessary information and support to be able to make informed decisions about their healthcare. This aim is supported through consumer and health policy research, *Which?* magazine reports and *Treatment Notes* - a patient information series providing practical, unbiased information about medical conditions, medicines and other treatments. *Treatment Notes* is based on articles from *Drug and Therapeutics Bulletin (DTB)* - our publication for healthcare professionals.
3. Thank you for asking Which? to comment on this consultation. We outline our comments on the Commission's proposals below.

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4. General comments

- 4.1. Which? (formerly known as Consumers' Association) and *DTB* have campaigned since the late 1990s around these issues of paediatric prescribing. When children, their carers and healthcare professionals use medicines they want to be confident of the efficacy and safety of these treatments. However, it has been estimated that at least 50% of medicines used in treating children across Europe have not been licensed for use in paediatric populations, and so this confidence is lacking.
- 4.2. Which? supports moves to provide consumers with easier access to safe, effective and appropriate medicines. For many prescribers, there is no alternative than to use off-label or unlicensed products when treating children. We welcome proposals in this regulation to improve the health of children by increasing the availability of authorised medicines and the amount of information available to patients, their carers and healthcare professionals about the use of medicines for children.
- 4.3. This regulation offers the opportunity to identify where children's needs are not being met, and where efforts should be directed to meet these needs. The inventory of therapeutic needs and the publication of clinical trial results from previously completed and future paediatric research will help to raise awareness of current treatment best practice and identify where future work can be carried out to best effect.

5. Paediatric Committee

- 5.1. Under these proposals, a Paediatric Committee will be convened at the European Medicines Agency. We welcome the proposals to include patient representatives on this Committee. However, it is not clear from the proposals whether the six seats reserved for patient and professional interests could all be occupied by professionals without any patient representation. We strongly advocate ensuring that patient and public interests on the Paediatric Committee are protected, and that consideration is given to allocating a fixed number of seats (perhaps three) to patient representatives.
- 5.2. The proposed strict conflict of interest policy is to be welcomed. It should also apply to the patient and professional representatives appointed to the Committee, both in their personal and professional lives. Details of financial relationships between the organisations they are representing and the pharmaceutical industry should be registered in an open and



transparent way to ensure confidence in the decision-making processes of the Committee.

6. Paediatric Investigation Plan

6.1. We welcome the proposal to require companies to produce a paediatric investigation plan for all new medicines, outlining the trials to be conducted in children and agreed with the Paediatric Committee. It is reassuring that the plan will take account of different age groups of children, so age-appropriate advice may be given to patients, their carers and healthcare professionals. We welcome the publication of the status of paediatric investigation plans and the results of studies in children in product information.

6.2. In approving the paediatric investigation plan, the Committee will have a key role in ensuring appropriate and ethical research only when it is necessary among children. This poses a question around the necessity and ethics of paediatric clinical trials of 'me-too' drugs - medicines that perform identical or near-identical therapeutic role as a drug already available. This is not addressed by the proposed regulation. We would welcome more information on how the Committee will approach this issue.

7. Extension of the duration of the supplementary protection certificate (SPC); Paediatric use marketing authorisation (PUMA)

7.1. The proposed regulation will award a six-month extension to the supplementary protection certificate on those new medicines that have completed their paediatric investigation plan and 10 years data protection for paediatric development of off-patent medicines. Over years of campaigning on this issue, it has become clear to Which? and DTB that without incentives the pharmaceutical industry will not carry out the necessary clinical trials. However, we remain concerned about the impact of the extension of patent and data protection on the generics market and believe that this should be actively monitored and reviewed.

8. Identification of paediatric medicines

8.1. We understand from this proposal that medicines that have completed their paediatric investigation plan successfully and with positive results, along with those off-patent medicines awarded a PUMA will be allowed to be identified as authorised for paediatric use on the package label. We support the MHRA in rejecting the 'P' suffix as this could be confused with pharmacy supply in the UK, and the use of an alternative such as 'authorised for paediatric use'.



8.2. However, we have some reservations about how patients, their carers and healthcare professionals should interpret the absence of such a statement on a product. If a medicine is not 'authorised for paediatric use' this may be because it has not been tested in children, with the result that little or nothing is known about its safety and efficacy, as is likely with some off-patent medicines. Alternatively, it could mean that trials have indeed been carried out, but led to negative results.

8.3. It is essential that there is clarity on how this absence of information should be interpreted by patients, carers and healthcare professionals, especially with regard to those medicines where use in children is definitely not supported by evidence collected by the paediatric investigation plan. It may be necessary to develop a system that provides clear signposting of medicines shown to be inappropriate for children as a conclusion of paediatric investigation plan.

9. Information on the use of medicines for children

9.1. The plan to develop a clinical trials database based on paediatric studies is to be welcomed, but Which? strongly believes that access to this should be open to all, and not hidden from public view as proposed by the Commission. This would help to avoid unnecessary clinical trials in children, and will help to provide further information for patients, their carers and healthcare professionals about paediatric medicines.

10. Inventory of therapeutic needs

10.1. The inventory of therapeutic needs is to be welcomed, but with provisos. It is important that paediatric research and development is driven by therapeutic need and not market forces. For example, the emphasis should be on encouraging testing of medicines that could offer genuine therapeutic advantages rather than me-too competitors for established authorised treatments. The proposal suggests that the inventory will be based on current medicines' use. This does not necessarily equate to an understanding of the current therapeutic needs of children. There will be quantitative and qualitative differences between these two approaches and different inventories will result.

10.2. The inventory should reflect the experience of children and their medicines and not be driven by industry-related perception of need. Further details are needed of how the therapeutic need will be assessed, and the opportunities for involving the public, patients and their carers in establishing and reviewing the inventory.



10.3. We also need to know more about how these research priorities identified by the inventory will be addressed. The inventory could have extra value as it would help to identify where comparative, head-to-head trials of similar treatments would be useful for patients, their carers and healthcare professionals.

11. Obligatory submission of completed paediatric clinical trials

11.1. We welcome the opportunity offered by this regulation to update product information for existing licensed medicines with results of completed but previously unpublished industry trials. In the interests of openness, the trial details and data that are submitted should be made publicly available, as with the trials and data generated by paediatric investigation plans under this regulation. This will allow patients, their carers and healthcare professionals to benefit from the wider availability of this information, providing greater detail about the appropriate use of medicines in children.

11.2. We would like to know more details of any sanction that may be brought to bear on those pharmaceutical companies who fail to comply with the regulation's requirement to submit paediatric trial data from completed trials.

12. If you have any queries or require further information about this response, please contact Kate Webb, Senior Policy Adviser, Which?, 2 Marylebone Road, London, NW1 4DF; telephone 020 7770 7258; email kate.webb@which.co.uk