European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

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EMEA/PEG PROCEDURE FOR IDENTIFYING PAEDIATRIC NEEDS

Background

Since its creation, the Paediatric Working Party (PEG) has been working on therapeutic areas and products which may be considered as paediatric needs for drug development and/or data from appropriate trials in children. One important step in this process is to base the choice of products on available evidence and on unmet therapeutic needs.
Several Member States attempted similar work, which have been considered by the group when establishing the methodology (e.g. France, UK, Germany), although most of them related to defining priorities list of products to be studied rather than needs.

Objectives

The objective of such lists is to identify the needs in the different therapeutic areas where there should be research and development of medicinal products, either old (i.e. off patent) or new ones (including those under development). Products on the list are not in any order of prioritisation.

Methodology

Principle

The work is being carried out from a public health perspective and the outcome is supported as much as possible by evidence-based medicine.

The methodology used is based originally on the work carried out at the French Medicines Agency (AFSSAPS). The AFSSAPS drew up lists of substances of current and potential use, the legal status (authorised for adult use or not), available paediatric information and appropriate formulations, if any.

To put the list into a European perspective, the PEG carries out extensive consultation of:
- experts in the relevant areas, as recommended by PEG
- Contact points at National Authorities,
- European Learned societies relevant to the therapeutic areas

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All the parties consulted are asked the following questions:

1. The lists present paediatric needs in the therapeutic area <name of the area>, as assessed by one Member State only. In this context, would you concur with the conclusions of the French paediatric Committee in terms of choice of substances? Do you see other substances of interest?
2. Do you agree with the request for either supply, formulation, information or additional studies?
3. In view of the lists, are you aware of existing (authorised) formulations adapted to children that are available in any other Member State for the selected substances?
4. Do you have other needs in these therapeutic areas?
5. Do you want to make other comments about this request?

Next steps

All comments received are going to be compiled for review by the PEG.

Based on the information gathered, a summary will be prepared highlighting:

- what is known to be authorised (indication in broad term, lower age group authorised in at least one Member State, authorised dose and formulations in at least one Member State)
- what is needed in terms of indication, age range, "age-appropriate" formulation and data.

Two outcomes are possible:

1. If a European Learned Society exists and is willing to take the task , they will extensively review , based on available evidence, the information gathered by the PEG. Both the Learned Society and the EMEA will then publish the conclusions of the review.

2. If no European Learned Society exists as such or if it cannot take on board such activity, the summarised outcome of the work will be published by the EMEA to stimulate feedback from paediatricians, Industry and Academia. As no systematic review of the evidence will be done by the EMEA, the references will not be provided. The summary will therefore list needed products, including some which may already have sufficient level of evidence. It is expected that any interested parties would provide information to complete the list.

Information will be passed on to industry associations for comments/actions.

Limits of the methodology chosen

This assessment has to be considered as a "snapshot" of a very dynamic process of drug development and research in medicines and that it should be considered as positive that new data becoming available may make some part of the assessment outdated. Still the general picture given by this assessment process will remain valid.

Information on existing marketing authorisations is very limited. It may not be easily accessible as not all national competent authorities have an electronic searchable database of all authorised medicinal products, including the non-marketed ones.
Information on existing data (e.g. publication of trial results) has not been systematically obtained.

The list will specify which kind of data would be needed but neither the design, nor the number of studies (e.g. PK, efficacy). The lists will indicate the need for 'age-appropriate' formulations, without specifying which one, to keep options open and room for innovation.
The summary may list required data, which are actually available. If so the needs would then be limited to submission and assessment of these data.

The lists are intended to include potential products. However, they may not have identified all promising products which are still in the pipeline, and would ultimately prove to be the best answer to the current needs.

This is no priority ranking in the order of the reviews of the various therapeutic areas.

Actions subsequent to the publication of the lists

Feedback on the lists from interested parties is expected to be provided to EMEA.

When an indication/formulation has already been authorised in one Member State, it is assumed that the data would already have been submitted and evaluated. The need is then limited to extending the marketing authorisation to other Member States using the appropriate regulatory procedures.

If the required data are in fact already available, the need is then limited to the submission of these data to the relevant Competent Authorities for assessment.

Records and Updates

All lists and comments received are recorded on Word, and kept at EMEA according to archiving rules. Some lists may be updated following receipt of comments from interested parties.

Disclaimer

The lists should not be viewed as a prescription tool. Accuracy of data including in particular authorised doses cannot be guaranteed.