Réponse de l'EMA à une sollicitation de l'ESPE (European Society for Paediatric Endocrinology) concerns re rare disease treatments

Dear Professor Butler,

Thank you for your letter of 6 December, regarding the issue of the availability of phosphate preparations. As a specialist in endocrinology who previously treated adults and children with a variety of endocrine disorders, before joining the European Medicines Agency, I feel particularly involved in this problem.

The European Medicines Agency (EMA) plays a central role in the development and authorisation of medicines for rare diseases, and is responsible for reviewing applications from sponsors for the designation of medicines for rare diseases. More information on this process, and the available incentives, can be found in
http://www.ema.europa.eu/ema/index.jsp?curl=pages/special_topics/general/general_content_000034.jsp&mid=WC0b01ac058002d4eb

The EMA also plays an important role in the development of medicines for children. More information can be found in
http://www.ema.europa.eu/ema/index.jsp?curl=pages/special_topics/general/general_content_000302.jsp&mid=WC0b01ac058002d4ea

The EMA is obliged to operate within the frame of European Union legislation, where authorisation of a medicinal product (in adults or in children) is dependent on a procedure started by an applicant, and supported by the appropriate data/documents. Therefore, the EMA cannot grant an indication in children of its own motion, or impose on a pharmaceutical company an obligation to market a specific product in the EU (or in some of its Member States).

The professional organisation you represent may wish to suggest to the marketing authorisation holder(s) of appropriate products, marketed in only one or few EU Member States, to extend the marketing authorisation to other Member States, as this is possible in the EU (see 1.1.2 in http://www.ema.europa.eu/docs/en_GB/document_library/Regulatory_and_procedural_guideline/2009/10/WC500004134.pdf, page 7).

On the issue of phosphate preparations we have started a consultation of the members of the Paediatric Committee of the EMA, who work in all 27 EU Member States, and I will contact you again in a few weeks when we have its results. I have already received comments from one of the members of the Committee, Dr Carine de Beaufort, who is also a practicing paediatric endocrinologist.

Yours sincerely,
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