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Human Medicines Development and Evaluation

Report on the survey of all paediatric uses of medicinal products in Europe

Executive summary

One of the provisions of the Regulation (EC) No 1901/2006 of the European Parliament and of the Council1 on paediatric medicines requests the EU Member States to collect available data on all existing uses of medicinal products in the paediatric population and to communicate these data to the European Medicines Agency. A mainly retrospective survey was initiated and the Agency's Paediatric Committee provided guidance on the content and format of the data to be collected and submitted. The expectations from the survey were to provide general statistics on the extent of use of the different medicinal products in children, knowing that due to the various information sources and data collection methods across European countries, different levels of detail and completeness and data heterogeneity were also expected.

Based on the analysis of the received data, a report on the survey was prepared. The document includes several parts. The first part is a detailed description of the datasets submitted by each country participating in the survey. It is followed by the data analysis, which focuses on the use of authorised medicinal products outside the terms of the marketing authorisation and of the summary of the product characteristics (off-label use), and on the use of unauthorised medicinal products which are of particular interest for the identification of the therapeutic needs of children.

The therapeutic classes that are used most frequently off-label or without a marketing authorisation are presented: antiarrhythmics, antihypertensives (renin-angiotensin inhibitors and beta-blockers) proton pump inhibitors and H2-receptor antagonists, antiasthmatics, and antidepressants (mainly selective serotonin reuptake inhibitors, serotonin-norepinephrine reuptake inhibitors and tricyclic antidepressants), contraceptives (in adolescents), and antibiotics (in very young children).

The report identifies some of the limitations to which the data analysis was subject. The main limitations were: data heterogeneity, which made an inter-country comparison not possible, the absence of a quantitative measurement of the extent of use for some datasets (and parts of some datasets), the lack of distinction between different types of use in children (authorised, unauthorised and off-label) further confused by the lack of common terminology in the different European countries. Although the survey was intended to provide information on the safety of use of medicines in children,

[1] Art. 42 of the said Regulation

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the data provided were very scarce and confirm the difficulty of identifying information regarding children among other age groups .

The fifth part of the report discusses the survey results, starting from the fact that prescription of off-label and unauthorised medicinal products is widespread in Europe, both in paediatric in- and outpatients, with higher rates in very young children and children with very severe conditions. One important reason for off-label and unauthorised use in children is the lack of age-appropriate formulations (including form and strength). The main therapeutic areas (contraceptives, gastroenterology, cardiovascular and respiratory medicines) and the main age groups (preterm and term neonates) requiring future clinical trials to render supportive efficacy and safety data are identified. The regulatory aspects of the identified paediatric needs and possible corrective actions are mentioned.

The final part of the document concludes that the report provides an overview of the unmet therapeutic needs and underlines the usefulness of the results in identifying whether waivers of the paediatric medicine development could be granted or not by the Paediatric Committee. It sheds further light on the still unsatisfactory situation of the treatment of European children and represents a strong argument for the usefulness of the existing Paediatric Regulation.