Better medicines for children: are we there yet?

Catherine Tuleu UCL School of Pharmacy, 29-39 Brunswick Square, London WC1N 1AX, UK

Correspondence Catherine Tuleu, E-mail: c.tuleu@ucl.ac.uk

The study of medications among paediatric patients has increased worldwide since 1997 in response to new legislation and regulations. In Europe since January 2007, the Regulation aims to ensure that medicines for use in children are of high quality, are ethically researched and authorized appropriately and to improve the availability of information on the use of medicines for children. Therefore, we are only in the process of measuring the 10-year impact on paediatric research, availability of authorized medicines and information on medicines. However considering the lengthy overall development cycle times, we will probably have to wait yet another decade, for the next big anniversary, to appreciate a tangible influx of new age–appropriate paediatric treatments.

In the meantime, young patients are and will be commonly treated with off-label or unlicensed drugs. Not only is their use associated with a greater risk of harm than with the use of appropriately licensed medicine, but also there is a lack of safe formulations in suitable doses that children are able and willing to take. This is especially true for neonates and infants, the most vulnerable patients of the paediatric subsets, who remain 'therapeutic orphans' despite the potential known impact on pharmacokinetics of maturational changes relating to changes in body composition, organ weight and also function, on pharmacogenetics, and on pharmacodynamics. Even excipients, which are never prescribed yet almost, always administered carry potential adverse effects especially in neonates.

The regulatory stimulus requires companies to develop their paediatric investigation plan, discussing the proposed clinical trials in children of different ages and their intention for future commercial dosage forms. This has undeniably stimulated interest in furthering the way medicines are designed and made, the way they are studied in non-clinical and clinical phases.

The intent of this special issue on Paediatric Medicine in Journal of Pharmacy and Pharmacology is to celebrate progress and advances around not only paediatric medicines research and development but also to enable better-evidenced off-label and unlicensed medicines use practices. This collective of Research Papers, (mini)Reviews and letters range from pharmaceutics and drug delivery, experimental and clinical pharmacology to biopharmaceutics and rug disposition, all dedicated to children care across any therapeutic area, route of administration, clinical setting and borders