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REVIEW ARTICLE

Use of off-label and unlicenced drugs in hospitalised paediatric patients: a systematic review

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Abstract
Purpose The aim of this review is to assess the extent of the use of off-label and/or unlicenced drugs among hospitalised children.
Methods A systematic search was made in MEDLINE-PubMED for papers published from 1994 to 2012, addressing the prescription of off-label and/or unlicenced drugs for the paediatric population in hospital care.
Results Of the 829 studies retrieved, 34 met the inclusion criteria. Prescriptions ranged from 12.2 to 70.6 % for off-label and from 0.2 to 47.9 % for unlicensed drugs. The percentage of children who received at least one off-label and/or unlicensed drug ranged from 42.0 to 100 %, with newborns being the population that received most of such drugs. Off-label prescriptions were essential for dose modification (7.1–73.1 %) and unlicensed prescriptions for formulation modification purposes (3.6–100 %).
Conclusions These findings show that: (i) off-label and/or an unlicensed prescribing is widespread among the hospitalised paediatric population worldwide, (ii) there is no consensus on a definition of off-label and/or unlicensed drugs and (iii) preterm newborns receive most off-label and/or unlicensed drugs. By shedding new light on off-label and/or unlicensed drug prescribing, these findings will hopefully contribute to generating new, more effective knowledge about the paediatric population's need for quality drugs that are both safe and efficacious.

Keywords Paediatric · Unlicensed · Off-label · Drug · Hospital care

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Introduction

In general, when a drug is marketed, its efficacy and safety are evaluated by conducting clinical trials on highly selected populations from which the paediatric population has been systematically not sufficiently represented [1–4].

In clinical practice, however, untested drugs have to be administered to a paediatric population in spite of any problems that may entail in the form of: (i) a lack of information about proper dosages, thus leading to an increased risk of adverse reactions that may endanger life; (ii) ineffective treatments; (iii) use of magistral and officinal preparations with no information on stability testing; and (iv) administration of excipients which are included in the prescribed drug formulation and are potentially harmful to this population [1, 5].

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