Developing medicines for children: Overview

Introduction

In the 1960s, medicines were tested for efficacy in adults but not in children. Healthcare professionals had no alternative but to use medicines authorised for adults ‘off-label’ in children, with the associated risks of inefficacy or adverse reactions.

Pharmaceutical companies did not routinely carry out the necessary research and development to adapt medicines to the needs of the paediatric population.

In the EU today, more than half of medicines used to treat children are used ‘off-label’ – that is, they have never actually been studied in this population and are not always used to treat the same diseases in children as they are used to treat in adults.

Addressing the gaps in paediatric medicine

As a response to the absence of sufficiently suitable, authorised medicines for the treatment of conditions in children, guidelines in the EU and US were written in order to:

- Encourage and facilitate the timely development of paediatric medicines
- Ensure that the medicines used are subject to appropriate and ethical research and development
- Ensure that medicine are appropriately authorised for

use in the paediatric population
- Improve the information available on the use of medicines in children
- Help reduce ‘off-label’ use

A number of questions should be considered by the sponsor early in development to evaluate suitability for developing the medicine in the paediatric population. The right time to include medicines development for children often depends on the disease and the unmet need. It will also depend on whether the medicine is ‘novel’ (new) or whether it is part of a group of medicines, where the mechanism is already well understood.

As children are a vulnerable sub-group, developing medicines for children requires special measures to be taken in order to shield them from undue risk. Clinical trial design optimisation and ethical issues in paediatric studies are only part of what is needed. Age-specific formulations are essential to resolve issues such as difficulties swallowing tablets if a syrup is not available. Additionally, alternative flavours, colours, and taste masking may be required.

As a result of legislation in the EU and the US, medicines development is no longer possible without considering children, bringing paediatric development more in line with adult development. This change has resulted in increased paediatric development, and more medicines are beginning to be developed for children.

Attachments

- Presentation: Developing Medicines for Children
  Size: 384,528 bytes, Format: .pptx
  A presentation describing considerations for developing medicines for children, which can be adapted for own use.

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