Paediatric medicine: Challenges of early development

Introduction

Developing medicines for children poses both a scientific and regulatory challenge. Researchers must determine the right time to introduce a new candidate medicine to children, a decision which requires careful discussion and planning.

When should paediatric development begin?

Before a new medicine is studied in humans, it has already undergone a significant amount of research, including in animals. Early human studies focus on the safety of the medicine in adults and then move to determine whether the medicine works (‘proof of concept’) before beginning larger confirmatory trials, designed to inform on both safety and efficacy. At some point during the clinical studies, paediatric development of the medicine should be discussed and, potentially, begin.

The actual timing of the discussion depends on the individual project; however, it should always be early on in the development discussions to allow enough time for various activities including the development of new formulations and appropriate non-clinical and clinical trials. The right time to include medicines development for children often depends on the disease and the unmet need, and whether or not the medicine is novel (new) or part of a group of medicines in which the mechanism is already well understood. The timing is

also dependent on the requirements of regulatory agencies – for instance, it is typically earlier in the EU than in the US. Agreements between the EU and the US may also vary.

Legislation on paediatric medicines development

In the EU, a single piece of legislation governs the development of paediatric medicine and the authorisation of medicines for paediatric use.[1] This legislation requires children to be included earlier medicines development: it mandates that a Paediatric Investigation Plan (PIP) be submitted once the first studies in humans are completed, when the understanding of the effect of the medicine is just emerging. The legislation also provides incentives to those who comply with this mandate and submit their PIPs on time.

Ideally, and especially when a medicine has the potential for use in both adult and paediatric populations, the sponsor will aim to have almost parallel paediatric development from the time that the medicine begins clinical studies in adult humans. This would mean that the medicine would be available for children at the same time, or shortly after, it is available to adults. Although the discussion happens in parallel, the outcome might be staggered adult and paediatric development plans.

References

1. European Parliament (2006). *Regulation (EC) No 1901/2006 of the European Parliament and of the Council of 12 December 2006 on medicinal products for paediatric use*. Retrieved 11 July, 2021, from https://op.europa.eu/en/publication-detail/-/publication/f02fd0de-82a9-42d8-9cd1-723176bb5ce0
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