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1 Global Regulatory Initiatives

By Linda McBride, RPh, RAC

Introduction

Regulations and initiatives continue to be established to foster research and development of drugs, biological products, and medical devices for the pediatric population. However, there continues to be a lag from the time of approval in adults to the time of approval for pediatrics. To facilitate the development of medicinal products for pediatrics, global initiatives have been created and are described in this chapter.

World Health Organization (WHO)

WHO plays a significant role in improving child health from many different angles, including nurturing care, promoting healthy growth and development, and strengthening health services. In 2007, WHO published *Promoting Safety of Medicines for Children*[1] following a recommendation of the WHO Advisory Committee for the Safety of Medicinal Products. The Committee expressed concerns on the lack of data on medicines used in children. To further facilitate the market access of pediatric drugs, WHO published *Model List of Essential Medicines for Children* in 2013 as the core drug list for a basic healthcare system. This resource list considers the most efficacious, safe, and cost-effective drugs for priority pathologies in pediatric patients up to 12 years old. In addition, WHO launched the International Clinical Trials Registry Platform (ICRTP) to ensure a complete view of research is accessible to all those involved in healthcare decision making. The ICRTP also has been leveraged to improve awareness and provide easier access for clinical trials in children, contributing to the Millennium Development Goal of reducing child mortality.

The International Council for Harmonization of Technical Requirements for Pharmaceuticals for Human Use (ICH)

ICH has published two guidelines to recommend international standards and promote harmonization in support of development of pharmaceuticals intended for pediatric use. These published guidelines are: *Clinical Investigation of Medicinal Products in the Paediatric Population E11*, adopted in 2000 with an addendum in 2017, and *Nonclinical Safety Testing in Support of Development of Paediatric Medicines* S11, adopted in 2020. A third guideline is under development, with the objective of aligning terminology on pediatric extrapolation, providing a systematic approach for utilizing extrapolation, and providing guidance for both study designs and statistical analysis methods for incorporating extrapolation into a pediatric drug development plan.[2]

The purpose of the E11 guideline[3] is to encourage and assist sponsors with global medicinal product development. The guideline provides considerations for initiating pediatric development along with recommendations for timing of studies and the types of studies to be conducted, specifically pharmacokinetic,