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Opinion

Closing the Gap in Pediatric Drug Development

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It has been 20 years since the US Congress passed the Best Pharmaceutical for Children's Act (BPCA) in 2002 ushering in an era of expanding pediatric labeling studies in return for 6 months of additional exclusivity in drug marketing [1]. Ten years later BPCA and the Pediatric Research Equity Act (PREA) were made permanent and gave the Food and Drug Administration (FDA) the power to require pediatric studies as part of the drug approval process. At the end of June 2022 more than 1000 medicines have had evidenced-based pediatric information added to the drug label [2]. Many of these medicines have received FDA approval for pediatric use, while others had updated information added to the drug label to help healthcare professionals make informed decisions. The European Medications Agency implemented their pediatric regulation in 2007 extending the requirement for pediatric studies to the European Union [3]. Currently regulatory agencies around the world are collaborating to support global development plans for pediatric medicines. Two notable exceptions to pediatric labeling studies have been neonates and children with cancer.

Most drugs used in neonatal intensive care units (NICUs) around the world continue to be used 'off-label' [4]. Recognizing these deficiencies in the most vulnerable and rapidly changing patient population, the FDA has published nonbinding recommendations for studies of medicines in neonates [5]. Within the PREA requirements for the initial pediatric study plan is the inclusion of the neonatal population unless a waiver is sought. It is imperative since most drugs are used off-label in NICUs, that studies be conducted in neonates to address the need for evidence-based information in the labeling.

Pediatric cancer survival has been a remarkable success with about 85% surviving at least 5 years from diagnosis. Most adult cancers for which molecular targeted drug therapy is being developed do not have a comparable pediatric cancer. However, many pediatric cancers have relevant molecular targets that affect the tumor growth and progression of disease. Although these target therapies have been available for pediatric cancers, legislative efforts have sought to incentivize the research of new cancer medicine in children. In the 2017 FDA Reauthorization Act, PREA was amended under the Research to Accelerate Cures and Equity for Children (RACE) Act to require cancer drugs being developed for adults to also be studied in children when the molecular target of the drug being developed also has a molecular target that is relevant to the growth or progression of pediatric cancer [6]. The RACE Act came into full effect in August 2021. The FDA publishes a relevant molecular target list with potential importance to the growth and progression of pediatric cancers.

Legislative and regulatory guidance continues to push the expansion of pediatric labeling studies across the age and disease spectrum in children. It is only with the establishment of safe and effective therapies in children that can they can achieve the full benefits from the multitude of medicines being developed primarily to treat adult diseases.

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