Innovation in Pediatric Drugs Act of 2023

Children are not just small adults. Drugs work differently in children and must be studied specifically for their use. Yet too often, drug development still leaves children behind. The **Innovation in Pediatric Drugs Act of 2023**, sponsored by Rep. Anna Eshoo (D-Calif.) and Rep. Mike McCaul (R-Texas), will help speed therapies to children who need them—including children with pediatric cancer and other rare diseases—by making needed changes to the pediatric drug laws.

PEDIATRIC DRUG LAWS: AN OVERVIEW

The Innovation in Pediatric Drugs Act would make needed improvements to the **Best Pharmaceuticals for Children Act (BPCA)** and the **Pediatric Research Equity Act (PREA)**, two laws that encourage and require the study of drugs in children. Data resulting from BPCA and PREA studies are added to drug labels to give parents and providers essential information on the safety and efficacy of drugs used in children.

PREA requires drug companies to study adult drug indications in children when children could benefit from pediatric studies. In 2017, the **Research to Accelerate Cures and Equity** (**RACE) for Children Act** amended PREA so that FDA could require companies to conduct pediatric studies of new adult cancer therapies whose molecular targets are relevant to pediatric cancers. Prior to 2017, PREA in effect excluded most oncology drugs from pediatric testing because cancers were understood by their location in the body.

BPCA is a voluntary incentive for drug companies to conduct FDA-requested pediatric studies—especially for off-label drug uses—in return for an additional six months of marketing exclusivity.

THE INNOVATION IN PEDIATRIC DRUGS ACT

Ensuring Drugs for Rare Diseases are Studied in Children

There are close to 7,000 rare diseases without appropriate treatments, and the vast majority of orphan diseases affect children. Unfortunately, in most cases, FDA is not allowed to require orphan drugs (drugs for rare diseases) to be studied in children under PREA.

Orphan drugs used to account for a small minority of annual drug approvals. Yet today, the majority of drugs approved are orphan drugs, meaning that the majority of newly approved drugs are exempt from pediatric study requirements.

In 2019, FDA released a study on the pediatric research gaps that have resulted from the PREA orphan exemption. It showed that 36% of pediatric-relevant orphan drugs approved since 1999 lacked some or all pediatric data.

Congress previously passed the RACE for Children Act, which lifted the PREA orphan drug exemption to allow studies for some orphan drugs for cancer. The time is now to lift the orphan exemption for all children with rare diseases.

The Innovation in Pediatric Drugs Act would amend PREA to lift its orphan exemption, ensuring that children with rare diseases can benefit fully from the pediatric research requirements.

Providing Equal Accountability for Pediatric Study Requirements

Due dates for PREA studies are typically deferred by FDA until a date after the approval of the drug for adults. Unfortunately, FDA has no effective enforcement tools to ensure that these studies actually get completed on time—or at all.

Congress tried to solve this problem in 2012. It allowed FDA to send "non-compliance letters" to companies that failed to complete their pediatric studies. Disappointingly, this did not fix the problem. According to an analysis conducted by the American Academy of Pediatrics, as of early 2021, 123 PREA non-compliance letters had been issued, yet only 41 (33%) of these instances of non-compliance had been resolved. That left 82 (67%) instances of non-compliance unresolved with studies still late. The average late study was 4.4 years late. Twenty-one studies were between 5-10 years late, 7 were 10-15 years late, and 3 were more than 15 years late.

FDA requirements for postmarket studies in adults can be effectively enforced, but requirements for postmarket studies in children cannot. If a company fails to complete adult postmarket studies, FDA can penalize the company by imposing a fine but it is prohibited, by law, from applying those penalties to pediatric postmarket studies under PREA.

If FDA is not given additional enforcement tools, not only will these studies required in the past not get completed, but future studies will be in jeopardy too, including pediatric cancer studies required under the RACE for Children Act, which went into effect in 2020.

The Innovation in Pediatric Drugs Act would amend PREA to give FDA the authority it needs to ensure that legally required pediatric studies are completed in a timely way.

Investing in Pediatric Studies of Older Off-Patent Drugs

The FDA incentives and requirements under BPCA and PREA work for many newer drugs, but unfortunately cannot help encourage studies of older drugs.

For this reason, Congress in 2002 authorized a BPCA program at the National Institutes of Health. This program funds NIH to do studies of off-patent drugs used in children that companies cannot be incentivized or required to conduct. To date, 18 pediatric drug labels have been changed through this program.

The BPCA NIH program has been flat-funded at $25 million since its original authorization in 2002. Drug studies are expensive and while the program is an efficient use of scare resources, the $25 million funding level is insufficient to meet the current needs. When accounting for biomedical research inflation, the

purchasing power of the program in 2022 was only 56% of what it was in 2002.

The Innovation in Pediatric Drugs Act would address this inequity by amending BPCA to increase the authorization level of this program to $50 million to keep up with the increasing need for and cost of these studies.