May 7, 2024

The Honorable Anna Eshoo
U.S. House of Representatives
272 Cannon House Office Building
Washington, DC 20515

The Honorable Michael McCaul
U.S. House of Representatives
2300 Rayburn House Office Building
Washington, DC 20515

Dear Representatives Eshoo and McCaul:

On behalf of the undersigned organizations, we write to offer our strong support for your legislation, the *Innovation in Pediatric Drugs Act of 2023* (H.R. 6664). Children are not just small adults. Drugs work differently in children and adolescents and must be studied specifically for their use. Yet too often, drug development leaves children behind. H.R. 6664 will help speed therapies to the children and adolescents who need them—including children with rare diseases—by making needed changes to the pediatric drug laws.

The pediatric drug laws—the Best Pharmaceuticals for Children Act (BPCA) and the Pediatric Research Equity Act (PREA)—have together revolutionized medicines for children. Since their enactment over twenty years ago, the laws have resulted in almost 1,200 drug labels changed with new pediatric information.[1] PREA requires certain new drugs to be studied in children and BPCA offers an incentive of six months of marketing exclusivity for drugs that are studied in children at the request of FDA. BPCA also authorizes a program at the National Institutes of Health (NIH) to fund the pediatric study of older, off-patent drugs that BPCA's incentive and PREA's requirements are unable to reach.

The *Innovation in Pediatric Drugs Act of 2023* would do three critical things to improve BPCA and PREA: increase the number of rare disease drugs studied in children, ensure that required PREA studies actually get completed, and give the NIH BPCA program its first funding increase in 22 years.

There more than 10,000 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 to be studied in children under PREA. When PREA was first passed in 2003, orphan drugs made up 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. H.R. 6664 would amend PREA to lift its blanket orphan drug exemption, while instructing FDA to promulgate guidance on when and how pediatric studies for rare disease drugs may be impossible or require modifications to the standard PREA requirements (i.e., deferrals and full or partial waivers), helping to ensure that more children with rare diseases can benefit fully from the pediatric research requirements. FDA confirmed the need for this policy change in 2019 when it released an evaluation of the pediatric research gaps that have resulted from the PREA orphan exemption. The report showed that 36% of pediatric-relevant orphan drugs approved since 1999 lack some or all pediatric data.[2]

PREA requires drug companies to study adult drug indications in children when children could benefit from pediatric studies. However, far too many pediatric studies required by FDA have never been completed.[3] 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. The *Innovation in Pediatric Drugs Act* would correct this discrepancy.

Finally, while the incentives and requirements under BPCA and PREA are effective in spurring the study of newer drugs in children, they are unable to encourage studies of older drugs. Congress authorized the BPCA NIH program to fund studies of off-patent drugs used in children that companies cannot be incentivized or required to conduct. Despite the increasing costs of drug studies, this program has been flat-funded at $25 million since its original authorization in 2002. 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.

When drugs are studied for their use, children are safer and the clinicians who care for them are better equipped to make medical decisions. We are grateful for your work to improve the study of drugs in children and adolescents and look forward to working with you to advance the *Innovation in Pediatric Drugs Act*.

Sincerely,

Academic Pediatric Association
American Academy of Family Physicians
American Academy of Pediatrics
American Association of Child and Adolescent Psychiatry
American Childhood Cancer Organization
American College of Rheumatology
American Pediatric Society
American Psychiatric Association
American Society of Pediatric Hematology/Oncology
American Society of Pediatric Nephrology
American Thoracic Society
Arthritis Foundation
Association of Medical School Pediatric Department Chairs
Association of University Professors of Ophthalmology
Child Neurology Society
Children’s Hospital Association
Children's Brain Tumor Foundation
Children's Cancer Cause
Children's Oncology Group Foundation
Children's Wisconsin
Council on Pediatric Subspecialties
Dana-Farber Cancer Institute
Elizabeth Glaser Pediatric AIDS Foundation
Family Voices
Leukemia & Lymphoma Society
March of Dimes
Mattie Miracle Cancer Foundation

MultiCare Mary Bridge Children's Hospital and Health Network
National Association of Pediatric Nurse Practitioners
National Organization for Rare Disorders
Nemours Children's Health
North American Society for Pediatric Gastroenterology, Hepatology and Nutrition
Pediatric Infectious Diseases Society
Pediatric Orthopaedic Society of North America
Pediatric Pharmacy Association
Pediatric Policy Council
Society for Adolescent Health and Medicine
Society for Developmental and Behavioral Pediatrics
Society for Pediatric Research
St. Baldrick's Foundation
St. Jude Children's Research Hospital

[1] Food and Drug Administration, Pediatric Label Changes Database: https://www.fda.gov/science-research/pediatrics/pediatric-labeling-changes.
[2] Food and Drug Administration, Pediatric Labeling of Orphan Drugs: https://www.fda.gov/media/130060/download.
[3] Food and Drug Administration, PREA Non-Compliance Letters, CDER and CBER: https://www.fda.gov/drugs/development-resources/non-compliance-letters-under-505bd1-federal-food-drug-and-cosmetic-act and https://www.fda.gov/about-fda/center-biologics-evaluation-and-research-cber/prea-non-compliance-letters.