Dear Chairmen Alexander and Walden and Ranking Members Murray and Pallone:

Thank you for the opportunity to provide comments on the Food and Drug Administration (FDA) Reauthorization Act of 2017. On behalf of the undersigned organizations representing medical providers, patients, and consumers, we offer the following comments for your consideration. We look forward to working with you to ensure that the FDA Reauthorization Act of 2007 includes policies that improve the study and labeling of drugs that are used in children.

Since PDUFA II in 1997 and every 5 years thereafter, Congress has included modifications that have strengthened and improved the efficiency of the Best Pharmaceuticals for Children Act (BPCA) and the Pediatric Research Equity Act (PREA). Because of Congress’s work on BPCA and PREA, today, more than 675 drug labels have been updated with important pediatric information. Children are not small adults. Drugs work differently in children than in adults and must be studied specifically for their use. Data resulting from BPCA and PREA studies are added to drug labeling to give parents and providers essential information on the safety and efficacy of drugs used in children.

We note that the FDA Reauthorization Act of 2017 does not address BPCA and PREA and we look forward to working with you to ensure that as the legislation moves forward, Congress will address the recommendations our organizations and others, including the Food and Drug Administration, have identified.

Specifically, we urge the Committees to include language that:

1) **Removes the orphan drug exemption in PREA** - Orphan drugs are currently exempt from PREA’s pediatric study requirements. In recent years, roughly 40% of all drugs approved by FDA annually were designated as orphan drugs, meaning FDA cannot require these drugs to be studied in children under PREA despite that fact that 50-75% of orphan diseases occur in children. Removing the rare disease exemption in PREA would ensure that FDA can require the study of drugs for orphan diseases, where appropriate.

Removing the orphan exemption would also eliminate a concerning loophole. Currently, companies can receive orphan designation for the pediatric population affected by a disease even if it is not an orphan disease in adults. Some companies, however, have received this pediatric orphan designation and never actually conduct the pediatric studies—and FDA is unable to require these studies to be completed under PREA.

2) **Allows PREA to apply to targeted therapies** - Currently under PREA, FDA can only require a company to conduct pediatric studies for a new drug or a new use of a drug if the indication is the same in adults as in children. To increase children’s access to modern therapeutics, Congress should allow
FDA to use PREA to require pediatric studies for a drug when it affects specific molecular targets or mechanisms that are shared between the adult and pediatric disease.

3) **Improves accountability** - PREA studies are typically deferred until after the drug is approved for adults and companies can receive a deferral extension to allow even more time. However, the deadlines to complete studies are too often missed. Congress should give FDA additional enforcement tools to ensure that critical pediatric studies are completed in a timely manner.

4) **Ensures earlier pediatric study planning for deadly diseases** - Under current law, sponsors are required to submit an initial PREA pediatric study plan no later than the end of phase II in the drug development process for adults. FDA cannot require planning for pediatric studies at the end of phase I, even though companies are subject to that requirement in Europe. Congress should ensure that planning for pediatric studies under BPCA and PREA occurs in a timely manner, especially for the small subset of drugs to treat serious and life-threatening conditions.

5) **Increases transparency** - Details on the studies being conducted under BPCA are not made public until after all the studies are completed, which can be about 5-10 years after the FDA requested the studies. FDA cannot share the specifics of the BPCA study request with their counterparts in other countries, which hinders their ability to inform pediatric studies in those countries. The public is also not informed when companies decline BPCA study requests. Congress should provide greater transparency to improve collaboration and coordination between industry, researchers, and patients.

6) **Reauthorizes and makes permanent the BPCA NIH program just like BPCA and PREA are permanent** - The FDA Reauthorization Act of 2017 reauthorizes several authorities that were included in the Food and Drug Administration Safety and Innovation Act of 2012 with one notable exception, the BPCA program that funds the study of older, off-patent drugs. We urge you to include a permanent reauthorization of this program at $35 million in the FDA Reauthorization Act of 2017.

7) **Promotes studies in neonates** - Congress should build on the progress made at FDA by making permanent its neonatology expertise and issuing guidance regarding the development of studies in neonates.

We appreciate your consideration of our request. We look forward to working with your offices on legislative language to carry out these recommendations that will improve and strengthen BPCA and PREA for all children. If we can be of further assistance, please contact Tamar Magarik Haro or James Baumberger with the American Academy of Pediatrics at tharo@aap.org or jbaumberger@aap.org.

Sincerely,

American Academy of Child and Adolescent Psychiatry
American Academy of Pediatrics
American Pediatric Society
American Thoracic Society
Association of Medical School Pediatric Department Chairs

Children’s Hospital Association
Elizabeth Glaser Pediatric AIDS Foundation
March of Dimes
National Association of Pediatric Nurse Practitioners
Pediatric Pharmacy Advocacy Group
Society for Pediatric Research