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AAP Praises House Energy and Commerce Subcommittee on Health for Passage of FDA User Fee Legislation
Inclusion of pediatric drug and device reauthorizations and drug shortages provisions a great step toward improving laws critical to children's health

Washington, DC—The American Academy of Pediatrics (AAP) today praised the U.S. House Energy and Commerce Subcommittee on Health for its passage of legislation to reauthorize Food and Drug Administration user fee programs. The legislation included language to reauthorize and strengthen three essential laws to improve drugs and devices for children, the Best Pharmaceuticals for Children Act (BPCA), the Pediatric Research Equity Act (PREA), and the Pediatric Medical Device Safety and Improvement Act. The AAP also commends the Committee for addressing drug shortages and for the inclusion of pediatric studies in neonates, which will help increase the collection of data for this vital pediatric age group.

Until BPCA and PREA were passed in 1997 and 2003 respectively, most medicines used to treat children had been tested for safety and efficacy only in adults. Under PREA, drug companies have been required to study adult drug indications in children, and the incentive under BPCA has been a successful mechanism to encourage drug companies to conduct pediatric studies requested by the FDA—especially for off-label drug uses—in return for an additional six months of marketing exclusivity. Unless reauthorized, BPCA and PREA expire on Oct. 1, 2012.

“Children are not just small adults. Drugs work differently in children than in adults and must be studied specifically for use in children,” said AAP President Robert W. Block, MD, FAAP. “BPCA and PREA have revolutionized pediatric practice because all industry-sponsored studies result in labeling changes that provide valuable new pediatric information. Now we have new dosing information, new indications of use, new safety information, and new data on effectiveness.”

Drugs studied under BPCA and PREA treat a wide range of diseases in children, including HIV/AIDS, cancer, diabetes, allergy and asthma. Over 425 drug labels have been revised with important pediatric information as a result of these policies. While this represents significant success, more progress is needed to promote timely labeling of drugs for pediatric use.

Similar to the legislation passed out of the Senate Health, Education, Labor and Pensions (HELP) Committee last month, this legislation renews the two laws and makes several important policy improvements that are consistent with the recommendations made by the Institute of Medicine in its recent Safe and Effective Medicines for Children report. The bill will improve the timing and quality of pediatric research by moving pediatric study planning earlier in the drug development process. The legislation also gives the FDA new tools to ensure that studies required under PREA are completed by their due dates unless there is an appropriate reason for delay. Importantly, the bill will help to advance clinical information in product labeling for neonates, the tiniest babies ages birth to one month where off-label use remains around ninety percent, through enhancing FDA expertise in neonatology and by ensuring that all BPCA studies neonates, where possible.

The bill reauthorizes the important BPCA program at the National Institutes of Health that provides for pediatric studies of older drugs that no longer qualify for pediatric exclusivity, which can include some of the most commonly used drugs in children. In addition, the bill preserves the role of two FDA advisory committees in monitoring pediatric drug safety and advising the FDA on pediatric issues.

The legislation also builds on the tremendous success of the *Pediatric Medical Device Safety and Improvement Act of 2007* by reauthorizing for another five years the incentives to device manufactures to create needed medical devices specifically designed to meet the needs of pediatric patients. As a result of the profit incentive in the 2007 law, there has been a more than five-fold increase in the number of pediatric Humanitarian Use Device designations at the FDA. Similarly, the innovative Pediatric Device Consortia program, which the legislation reauthorizes for five years, has demonstrated great promise for children and for small business jobs. The consortia have assisted in 135 proposed pediatric medical device projects, and several of these devices have either been approved for pediatric patients in the U.S. or have been able to remain on the U.S. market. Lastly, the legislation requires FDA to issue a final rule implementing a pediatric device-tracking requirement from the 2007 law by December 31, 2013. The agency was forced to withdraw an earlier attempt to implement this provision due to industry opposition.

The bill reflects bipartisan legislation introduced by Representatives Mike Rogers, Anna Eshoo, and Edward Markey, H.R. 4274, the *BPCA and PREA Reauthorization Act of 2012*, and H.R. 3975, the *Pediatric Medical Device Safety and Improvement Act Reauthorization*. The AAP has endorsed these bills and commends the bill sponsors for their leadership on behalf of children.

The legislation passed by the subcommittee will also help provide FDA additional tools to prevent and better address drug shortages. Specifically, it expands the mandatory 6-month advance notification requirement of discontinuances or manufacturing interruptions of approved and unapproved drugs that could lead to supply disruptions.

“Drug shortages have had an impact in virtually every aspect of pediatric patient care,” said Dr. Block. “There may not be a more acute or more immediate threat to pediatricians’ ability to provide high-quality care to their patients than drug shortages.”

The legislation will require that shortages involving controlled substances, such as the recent widespread shortages of medications to treat attention deficit/hyperactivity disorder, trigger actions by the HHS Secretary and the Attorney General that may allow for increases in supply of such products. Consistent with the Academy position, it also requires the Secretary to create a comprehensive, up-to-date drug shortage list that would be made publicly available.

“AAP praises Chairman Pitts, Ranking Member Pallone, and Representatives Rogers, Markey and Eshoo for their dedication to the health and well-being of all children. The full Energy and Commerce Committee will be voting on the legislation this Thursday and we urge them to pass this critical legislation without delay.” said Dr. Block.

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The American Academy of Pediatrics is an organization of 60,000 primary care pediatricians, pediatric medical subspecialists and pediatric surgical specialists dedicated to the health, safety and well-being of infants, children, adolescents and young adults. (www.aap.org)