American Academy of Pediatrics

DEDICATED TO THE HEALTH OF ALL CHILDREN™

ELIZABETH GLASER PEDIATRIC AIDS FOUNDATION

April 23, 2012

The Honorable Tom Harkin
U.S. Senate
Washington, DC 20510

The Honorable Michael B. Enzi
U.S. Senate
Washington, DC 20510

Dear Chairman Harkin and Ranking Member Enzi:

On behalf of the American Academy of Pediatrics and the Elizabeth Glaser Pediatric AIDS Foundation, we write to express our gratitude and support for the inclusion of S. 2289, the *Better Pharmaceuticals and Devices for Children Act of 2012* in the Manager's Amendment to the *Food and Drug Administration Safety and Innovation Act.* We thank you for your commitment to reauthorizing and strengthening 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.

Children are not just small adults. Drugs work differently in children than in adults and must be studied specifically for their use. BPCA and PREA have encouraged and required the study of drugs in children. Under PREA, drug companies have been required to study adult drug indications in children. In addition, the incentive under BPCA has been a successful mechanism to encourage drug companies to conduct FDA-requested pediatric studies—especially for off-label drug uses—in return for an additional six months of marketing exclusivity.

BPCA and PREA have changed pediatric practice because all studies result in labeling changes that provide valuable new pediatric information. These studies have resulted in 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, and juvenile arthritis.

Over 425 drug labels have been revised with important pediatric information as a result of these policies. Before BPCA and PREA, the vast majority of drugs—more than 80%—used in children were used off-label, without data on their safety or efficacy. Today that number has been reduced to approximately 50%. While there has been significant success, more progress is needed, and these laws must be reauthorized and strengthened.

The Manager's Amendment is critical because it both renews these important laws and makes several important policy improvements that are very consistent with the recommendations made by the Institute of Medicine (IOM) in its recent *Safe and Effective Medicines for Children* report. For instance, it will improve the timing and quality of pediatric research by moving pediatric study planning earlier in the drug development process. It will also give 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.

The IOM, Government Accountability Office (GAO), and our respective organizations have called attention to the continued lack of pediatric data for younger pediatric age groups, particularly neonates.

Despite the tremendous progress of BPCA and PREA on pediatric labeling, more than ninety percent of drugs used in neonates are used off-label. The IOM, GAO, and others have identified the lack of neonatal expertise at the FDA as a contributing factor. We look forward to continuing to work with your offices to ensure that the final reauthorization bill includes provisions that will advance pediatric studies in neonates.

The Manager's Amendment reauthorizes the important BPCA program at the National Institutes of Health (NIH) that provides for pediatric studies of older drugs that no longer qualify for pediatric exclusivity or fall under the requirements of PREA. It also preserves the role of two FDA advisory committees in monitoring pediatric drug safety and advising the FDA on pediatric issues. We commend you for the continuation of these key authorities.

The Manager's Amendment 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 Manager's Amendment 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.

We would like to express our gratitude for your commitment to reauthorizing and strengthening these three vital laws for children. We look forward to continuing to work with you to ensure swift passage of these reauthorizations. Thank you for your dedication to the health and well-being of children.

Sincerely,

Robert W. Block, MD, FAAP
President
American Academy of Pediatrics

Charles Lyons
President and Chief Executive Officer
Elizabeth Glaser Pediatric AIDS Foundation

cc. The Honorable Jack Reed
The Honorable Lamar Alexander
The Honorable Patty Murray
The Honorable Pat Roberts