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On Behalf of the American Academy of Pediatrics

Before the U.S. House of Representatives
Committee on Energy and Commerce
Subcommittee on Health

"Modernizing FDA's Regulation of Over-the-Counter Drugs"

September 13, 2017

Chairman Burgess and Ranking Member Green, thank you for the opportunity to speak here today about the importance of modernizing the regulation of over-the-counter drugs (OTC) for America's children. My name is Dr. Bridgette L. Jones. I am a practicing pediatrician who specializes in the treatment of children with asthma and allergic disease. I also conduct clinical pharmacology research to improve the safety and efficacy of drugs for children. I hold a faculty appointment as Associate Professor of Pediatrics at the University of Missouri-Kansas City in the divisions of Pediatric Clinical Pharmacology, Toxicology and Therapeutic Innovation and Allergy/Asthma/Immunology at Children's Mercy in Kansas City, MO. I am board certified in Pediatrics and Allergy/Asthma/Immunology and have completed fellowship training in Pediatric Clinical Pharmacology. I am here today in an official capacity to represent the American Academy of Pediatrics (AAP). The AAP is a non-profit professional membership organization of over 66,000 primary care pediatricians and medical and surgical pediatric subspecialists dedicated to health and well-being of children. I serve as the chair of the AAP Committee on Drugs.

Every day in the United States, pediatricians get urgent calls from anxious parents, often in the middle of the night, asking about the best way to treat their sick child. Sometimes the answer is a prescription drug, sometimes the answer is non-drug supportive treatment, and sometimes the answer is an OTC medicine they can access at their local drug store. In my practice, I frequently need to discuss with parents the risks and benefits of using OTC medicines to treat common pediatric ailments such as allergies and asthma. Because parents often rely on OTC drugs to treat their children, it is essential that they can feel confident in knowing that those products are safe and effective. As a pediatrician advising parents, I want to know that the products I recommend have been tested in children and labeled appropriately for their use. As such, we must have a process set up to regulate them that is modern and responsive to the best and most recent medical science.

The current OTC regulation process at the Food and Drug Administration (FDA) is antiquated and not nimble enough to adapt to emerging evidence and to changes in how pediatricians practice medicine. The monograph that dictates how OTC drugs can be marketed was in large part developed based on the state of the evidence from over 40 to 50 years ago. Some of these drugs continue to be mainstays of pediatric practice, but others provide little or no benefit to children. Much of the pediatric drug labeling included in the OTC monograph was based on evidence that no longer meets today's rigorous standards for safety and efficacy or was based on incorrect assumptions about how adult data should inform the labeling of drugs in children.

Because we know that children are not just little adults, the AAP believes that drugs used in children should be appropriately studied specifically for their use. While we have made great strides in improving new prescription drug therapies for children through the Best Pharmaceuticals for Children Act and the Pediatric Research Equity Act, we have a long way to go to bring this record of success to OTC drugs.

The process for revising the OTC monograph is cumbersome and slow, and therefore the FDA cannot act quickly to respond to developments in the science, public health and safety concerns, or product innovation. The process is resource intensive while being significantly underfunded. It does not serve the needs of children and, for that matter, does not serve the needs of the public at large. The only way to ensure that consumers are afforded reliable, safe, and quality medicines is to change how the monograph system works and provide significant new resources for the endeavor. It is for this reason, the AAP supports reforms to the current OTC monograph system and the creation of a user fee program to fund FDA's monograph work. Over the past year, the AAP has worked closely with the Pew Charitable

Trusts and other public health and medical associations to speak up for the needs of patients as FDA, industry, and Congress have worked to develop a modernized approach to OTC regulation.

OTC Cough and Cold Medicines for Children

An illustrative example of how the current OTC monograph does not meet the needs of children can be found in the case of cough and cold medicines for children. The OTC drug review—the process FDA used to review cough and cold drugs and other grandfathered OTC products on the market prior to the enactment of FDA's modern standards for safety and efficacy—was a massive and complicated undertaking. While FDA reviewers did their best to evaluate the safety and efficacy of these products, the data available to them was often extremely limited. And in the case of drugs for children, much has changed in the area of pediatric therapeutics since the 1970s. We have moved from an era where drugs were seldom studied in children, and pediatric drug studies were often considered to be unethical, to today, where failure to study drugs in children can be considered unethical.

The data that led FDA to label cough and cold medicines for children does not come close to meeting today's standards for pediatric data. Not only that, but additional data gathered since that time has clearly shown certain cough and cold products to be completely ineffective in the pediatric population. Nevertheless, these products are still commonly marketed to children and often in combination with other products that can increase the safety risks. The monograph process has proven ineffective in ensuring that OTC drugs marketed to children and families have data to justify their use.

Over a decade ago, numerous pediatric experts submitted a citizen petition to FDA regarding the labeling of OTC monograph drugs for the treatment of cough and cold in children. The petition highlighted safety concerns but also—in the case of some products—a demonstrated lack of efficacy in the pediatric population. FDA held an advisory committee meeting in response to the petition. The committee voted unanimously that it was no longer appropriate for adult data on cough and cold products to be extrapolated to establish efficacy of the drugs in children under 12. The committee also voted to recommend that cough and cold drugs not be used in children under 6 years of age, consistent with the AAP's recommendation at the time. After this critical and decisive meeting, FDA embarked on a process to revise the pediatric cough and cold monograph to better reflect the current state of the evidence.

Sadly, it's now 2017 and FDA has yet to publish even draft changes to this monograph, despite pleas from Congress, pediatricians, and the public. We are convinced that this lack of progress is not for lack of effort on the part of FDA. Rather, progress has not been realized because the monograph process simply does not work.

FDA was unable to act decisively in the face of mounting evidence that these products were resulting in thousands of pediatric overdose-related emergency department visits each year—all for products with modest or non-existent efficacy in children. Currently, for FDA to change a warning in the monograph it must go through a lengthy notice and comment rulemaking process to modify federal regulations. This unwieldy process comes with numerous bureaucratic steps and layers of review. FDA's only recourse for cough and cold drugs was to initiate a rulemaking process that has never concluded. This must change. If FDA identifies safety issues associated with a monograph drug, it needs the authority to require prompt label changes without going through a prolonged and burdensome regulatory process including the lengthy Office of Management and Budget review. Considerations of safety, effectiveness and

innovation, not economics, should drive FDA's process for modifying OTC drug monographs. Additionally, the agency needs appropriate resources to conduct safety surveillance for monograph products and allow quick action when safety issues arise.

FDA must have the authority and resources necessary to identify monograph products that lack appropriate data. Using a risk-based approach, FDA should be able to either require products to immediately come off the shelves or to give manufacturers a period of time during which they must submit new efficacy data to FDA to justify their continued marketing after which a product lacking such data would be removed from the monograph. Today's monograph process is ill-equipped to handle this task. Modernizing the monograph process will ensure FDA's ability to address products that do not meet appropriate efficacy standards.

Product Innovation

While the new drug application (NDA) process is the gold standard for the approval of new and innovative drugs, there are certainly instances where industry-initiated changes to the monograph are appropriate. Such changes can lead to improved drug formulations, increased safety, and other benefits for patients.

For instance, industry has for years been requesting that the monograph be amended to provide acetaminophen dosing instructions for children under the age of two. Even though there are well-accepted guidelines for acetaminophen dosing for children aged 6 to 24 months, the label of "infant" and "children's" acetaminophen (oral suspension) still asks parents of children under 2 to "ask a doctor" for dosing directions. Parents unable to quickly reach a physician may be tempted to make a guess of an appropriate dose, putting their infant at risk. The AAP supports such a change in labeling, and if the monograph process worked better, surely this change would have happened years ago.

Much like the long-delayed FDA action on the cough and cold citizen's petition, the existing backlog of industry-requested monograph changes currently awaiting FDA review is unacceptable. The uncertainty and complexity of the review process likely also reduces industry's incentive to invest research and development resources into monograph products. Congress should act to create a reformed monograph system that would add certainty to the evaluation of industry-initiated monograph revisions.

Safe Packaging for Children

While we generally support the goal of increasing industry innovation in the OTC drug market, we must ensure that this innovation meets the needs and expectations of consumers and does not have unintended negative consequences. With a reformed, more responsive, and better resourced system, one area where we anticipate greater industry innovation is in the development of novel formulations for OTC drug products. It is possible that the industry may work on developing gummy formulations of drugs, much like supplement manufacturers have done in recent years with their marketing of gummy bear vitamins.

Gummy formulations of OTC drugs—whether intended for children or for adults—would greatly concern pediatricians because we know that when a product looks and tastes like candy, children will eat it. If a child consumed a number of gummy medicines outside the watchful eye of parents, it could lead to injury, a trip to the emergency room, or worse. It is therefore vitally important that FDA have sufficient authority to regulate the packaging of OTC drugs.

Unfortunately, FDA's current authority over packaging is unclear, and as such we believe that this legislation must explicitly grant FDA authority to require specific types of packaging to prevent harm to children. For instance, if FDA did decide that approving a gummy drug product was appropriate, we would insist that FDA have the authority to require that drug to be sold in unit-dose packaging, such as blister packs, that would only allow access to one dose at a time. As a hypothetical example, a bottle of 100 loose, colorful and tasty gummy acetaminophen, if left open by an adult, would be nightmare scenario for a pediatrician from a poison prevention perspective. The margin between a therapeutic dose of acetaminophen and an amount that could kill a child is small. Without unit dose packaging, that open bottle of candy-like acetaminophen could be attractive and potentially fatal to a child. FDA must not wait until a product is already on the market and injuries, or even deaths, have occurred in children before requiring appropriate packaging.

Allowing FDA to require unit-dose packaging or other appropriate packaging would not interfere with the existing authority the Consumer Product Safety Commission (CPSC) has to require child-resistant packaging under the Poison Prevention Packaging Act because CPSC cannot require specific types of packaging like blister packs. What CPSC can do, and should continue to do, is to require drugs to be sold in what's called "special packaging," or packaging that is tested to ensure that it is sufficiently difficult for children to open. However, while CPSC regulations currently require all prescription drugs to come in this special packaging—and even requires the same for all prescription drugs that switch to OTC status—CPSC only requires a small handful of specific drugs regulated under the OTC monograph to be sold in child-resistant packaging.

For this reason, it is essential that FDA and CPSC have established processes for communicating about their regulatory activities. If FDA, for instance, were preparing to approve a new formulation of a drug that CPSC does not currently require come in child-resistant packaging—and this new formulation raised concerns about possible child poisoning—it would be important for the FDA to be in communication with the CPSC so that the Commission could decide whether updating its packaging regulations would be warranted.

We look forward to continuing to work with you and your staff to ensure that the legislation gives FDA the clear authority it needs to require specific types of packaging to prevent harm to children. Similarly, we look forward to continuing to work with you to ensure that the FDA and CPSC have established processes for notification when the FDA takes regulatory action on an OTC product that might warrant CPSC reevaluation of its packaging regulations.

Thank you for the opportunity to speak here today about the importance of safe and effective over-the-counter medicines for children. We look forward to working with you, FDA and other stakeholders as this process moves forward.