children's cause cancer advocacy

August 18, 2016

Dr. Robert M. Califf
Commissioner
Food and Drug Administration
10903 New Hampshire Avenue
Silver Spring, MD 20993

Dear Dr. Califf:

The Children's Cause for Cancer Advocacy (the Children's Cause) is responding to the Food and Drug Administration's (FDA) July 2016 Status Report to Congress on the Best Pharmaceuticals for Children Act (BPCA) and Pediatric Research Equity Act (PREA). The Children's Cause, established in 1999, was founded to ensure that the needs and perspectives of children with cancer and survivors are integrated into federal health care, research and cancer policy.

Improving the Best Pharmaceuticals for Children Act and the Pediatric Research Equity Act for children with cancer continue to be priorities for the Children's Cause. BPCA provides an incentive of six months of exclusivity – or patent extension – for drugs that are approved for use in children. PREA requires pharmaceutical companies who are developing a new drug for an adult indication to also test that drug in children. Two exceptions to this requirement constrain their impact for children with cancer. First, pediatric studies of a drug are limited to the same disease for which it is being studied in adults. Second, the PREA requirement has special application to any drug being developed for a "rare" disease in adults. As we discuss further below, we are pleased the Status Report addresses both of these issues.

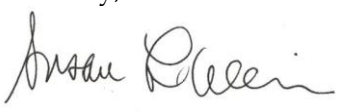
With the imminent reauthorization of the Prescription Drug User Fee Act, the Children's Cause co-chaired an Alliance for Childhood Cancer workgroup that developed a series of policy recommendations to improve PREA's and BPCA's effect on developing drugs to treat childhood cancer. The Alliance for Childhood Cancer represents more than thirty national patient advocacy groups and professional medical and scientific organizations. The Alliance recommendations address longstanding and specific concerns, such as the PREA requirements which involve dependency on adult indications, exclusion of drugs with orphan indications, the late award of BPCA incentives, and risk in testing new agents in children.

The Children's Cause, along with a large cohort of the childhood cancer community developed and supports a consensus group of recommendations for changes to BPCA and PREA. These BPCA and PREA proposals would create an improved and more effective framework for the creation of new drugs for children with cancer. ***We are pleased that several of the Alliance recommendations are contained in your Status Report.***

Specifically, in the Status Report, the FDA acknowledged the limitations of PREA with regard to pediatric cancer. Because most oncology products are developed for adult cancer indications that do not occur in children, products that would otherwise be studied under PREA are generally not applicable to pediatric cancers. In some types of childhood cancers, the same molecular targets for which new drugs are developed in adults may be important in the etiology of a specific cancer type, albeit in a different location, seen in the pediatric population. Early clinical evaluation of targeted drugs developed for adult cancers in children with certain cancers present opportunities to address significant unmet medical needs. Therefore, expanding the scope of PREA to require pediatric studies, based on a pediatric tumor's expression of a molecular target or the known molecular mechanism of action of a new drug, could significantly increase the number of pediatric studies conducted under PREA. ***We are encouraged the FDA recommends an amendment of PREA, as the Alliance does, to require certain drugs (including biological products) developed for adult cancer indications to be evaluated for a pediatric cancer indication, when there is evidence the drug affects specific molecular targets and/or molecular mechanisms shared between adult and pediatric cancers.***

FDA found that another significant limitation of PREA for children with cancer is PREA's exemption of all agents receiving the orphan designation for adult disease, including cancer. This exclusion effectively eliminates PREA from having **any** impact on pediatric oncology drug development. Timely evaluation and approval of certain drugs in children would not jeopardize the exclusivity associated with orphan-designation. ***We agree with the FDA recommendation to eliminate the exemption under PREA for relevant orphan-designated adult oncology products, found in section 505B(k) of the Food, Drug and Cosmetic Act, which could be useful for pediatric indications.***
In addition to these key areas of agreement, the Alliance has proposed six other recommendations for consideration by the FDA and Congress. These recommendations are attached for your review. Over the coming year, we look forward to working with the FDA and Congress to improve BCPA and PREA for children with cancer.

The Children's Cause is committed to supporting policy initiatives that advances therapies and improves care for children with cancer. The effort to change the laws that affect pediatric research and therapies will require continued commitment, vision, and collaboration. Working with the FDA, Congress, the Alliance, and other key stakeholders, we look forward to improving the lives of children with cancer. Please contact George Dahlman at GDahlman@childrenscause.org if you have questions or comments on this letter.
Sincerely,



Susan Weiner
President & Founder