St. Baldrick's FOUNDATION

Conquer Childhood Cancers

March 7, 2014

RE: Docket Number FDA–2013–N–0985

Ms. Leslie Kux
Assistant Commissioner for Policy
U.S. Food and Drug Administration
5630 Fishers Lane, Room 1061
Rockville, MD 20852

Dear Ms. Kux:

I am writing on behalf of the St. Baldrick's Foundation in response to the Food and Drug Administration's (FDA's) request for comments regarding the public workshop on complex issues associated with developing drug and biological products for rare diseases that was convened in early January, 2014. The St. Baldrick's Foundation is a volunteer-driven charity committed to funding the most promising research to find cures for childhood cancers and give survivors long, healthy lives. Since 2005, we have funded more than $127 million in childhood cancer research grants.

The St. Baldrick's Foundation commends the FDA for organizing this event and joins our fellow members of the Alliance for Childhood Cancer in applauding you for addressing critical issues related to drug development. The St. Baldrick's Foundation supports the comments the Alliance is submitting to the FDA for the record.

Cancer is the second leading cause of death for children in the United States between the ages of 5-15; and 1 out of every 285 U.S. children will be diagnosed with some form of cancer before age 20. While it was noted during the January 2014 FDA workshops that there have been "two to three oncology drugs approved for pediatric indications over the past 20 years," the fact is that only two drugs have been made available to treat children with cancer in the last 20 years and one of those was originally developed for adults.

Clearly more needs to be done — and quickly, as the incidence of cancer is on the rise. Today, 1 in 330 American adults is a survivor of a pediatric cancer.

There is no single disease known as "childhood cancer." The term describes 12 major types and dozens of subtypes of diseases that afflict children and adolescents. The drugs currently available to treat these cancers were, as noted, originally developed to treat adults. They are so toxic that when given to young, developing bodies they lead to a lifetime of late effects and psychosocial issues that require extensive medical care. Two-thirds of childhood cancer survivors live with lifelong impairment from the cancer treatments they received — many which are life-threatening.

1333 S. Mayflower Avenue, Suite 400, Monrovia, CA 91016
Toll Free (U.S. only) 888.899.BALD | Local 626.792.8247 | StBaldricks.org

During the FDA workshops great discussion centered on the fact that patient-reported outcomes are difficult to ascertain with childhood cancer drug development protocols as children may be unable to express their feelings. Opinions were offered that parents and other adult caregivers — frequently direct relatives — may not be suitable "proxies" for reporting treatment outcomes. Understanding that this is a complicated medical issue, we submit that parents and other adult caregivers are the closest to young patients and understand a child's experience better than others.

To the extent that patient-reported outcomes are necessary for new drug development, we hope that debate over the ability of parents and other adult care givers to speak for children in their care is resolved promptly.

The St. Baldrick's Foundation also takes issue with the repeated reference to childhood cancer as a "rare" disease. As previously above, there no single disease known as "childhood cancer" actually exists. To facilitate improved public understanding, we should at a minimum, refer to cancers in children as *childhood cancers*, and at best, we would classify them by their name as we do for adult cancers to transparently demonstrate how resources are allocated.

We acknowledge there are several forms of childhood cancer that are only diagnosed in a small number of cases each year, but referring to these as "rare" marginalizes patients and implies that it is understandable why limited treatment — or no treatment whatsoever — is available, and sends the clear message to the research community that they needn't bother. While nearly 60% of funding for adult cancer drug development originates from pharmaceutical companies, almost none is forthcoming for children because the drugs aren't profitable. We call on the FDA to work with industry and create programs and incentives that will encourage industry to significantly invest in the development, manufacture, and distribution of pediatric cancer drugs.

Further, the use of the word "rare" creates great confusion about where responsibility for studying these diseases lies. Is it in the rare disease community or is it in cancer? This has created the perception that no accountability exists and worse, that government doesn't care about this patient population.

At the St. Baldrick's Foundation we believe that the FDA, National Institutes of Health, National Cancer Institute, Department of Defense, Centers for Disease Control, National Science Foundation, and private sector researchers should diligently pursue treatment for *all forms* of childhood cancers and priorities ought not be set based on incident prevalence.

If doubt remains about the importance of developing pediatric cancer drugs, please consider this: the average age of diagnosis for a child with cancer is age 6, compared to an average age of diagnosis of 67 years for adults with cancer. This means that a child loses *71 years of life* due to a cancer diagnosis, compared to the 15 years lost by an adult.

"Curing childhood cancer is the equivalent of curing breast cancer in terms of productive years saved," says Dr. Eugenie Kleinerman, head of the division of pediatrics at the Children's Cancer Hospital at M.D. Anderson Cancer Center.

One final point relates to The Pediatric Research Equity Act (PREA) that requires pharmaceutical companies study the drugs they develop for use in children under certain circumstances. While PREA has contributed to significant research advances in how medicines can be used in children over the years, it has only had a modest impact on the development of new drugs for children with cancer.

Specifically, PREA requires pharmaceutical manufacturers to study their drugs in children *only* if the drug is developed for a disease that occurs in *both* adults and children. Because common adult cancers — such as breast, colon, lung and prostate cancers — typically do not affect children, companies receive waivers so they are *not* required to study these drugs in children with cancer, even though they may hold great promise.

Language which the St. Baldrick's Foundation supports with regard to PREA is: "For an oncology drug, an assessment may be required for a pediatric oncologic indication if the target of the drug for an adult oncologic indication is included in the drug's label and is highly relevant to any pediatric cancer to which it could apply."

The St. Baldrick's Foundation urges the FDA to seek every opportunity to strengthen PREA in hopes that promising cancer drugs for adults can more readily turn into promising cancer drugs for children as well.

On behalf of the St. Baldrick's Foundation I thank you for the opportunity to submit these comments and reiterate our support for the issues raised in comments from the Alliance for Childhood Cancer. If you have any questions or need any additional information I urge you to contact Lisa Parks, Senior Director of Advocacy, or Peter Mayberry, our Senior Manager of Legislative Affairs. They can be reached by e-mail addressed to Lisa.Parks@stbaldricks.org and Peter.Mayberry@stbaldricks.org, respectively.

Sincerely,

Kathleen Ruddy
Chief Executive Officer