

July 30, 2004

Division of Dockets Management (HFA-305)
Food and Drug Administration
5630 Fishers Lane, Rm. 1061
Rockville, MD 20852

Via e-mail
Re: Docket No. 2004N-0181

To Whom It May Concern:

The undersigned organizations appreciate the opportunity to provide comments on the recent report issued by the Food and Drug Administration entitled "Innovation or Stagnation? – Challenge and Opportunity on the Critical Path to New Medical Products." The undersigned organizations represent both national patient advocacy groups and professional medical and scientific organizations that advocates on behalf of children with cancer.

We agree that enhancements and reforms are necessary if we are to reduce the barriers in designing and developing new products and bringing them to patients more rapidly. This need is particularly urgent in pediatric oncology. Despite remarkable progress, cancer still kills more children than any other disease. Yet, there is a lamentable record of new drug development in pediatric oncology. According to FDA, between 1980 and 2002 only one new cancer product was approved for pediatric use.

As you know, cancer drugs assessed for pediatric use are most often evaluated either just prior to or after FDA approval for adult cancers. Companies' reluctance to develop drugs for children with cancer in part reflects the small pediatric market and in part is an indication of persistent concern about the risk of toxicities in children in early trials. When children have life-threatening disease, many families are willing to take this risk, especially since data show that pediatric cancer patients often tolerate certain toxicities better than adults. The delays resulting from some companies' reluctance to test new anti-cancer therapies in children are unacceptable to pediatric oncologists and families alike, and we have looked to FDA to change this picture. As has been expressed at meetings of the Pediatric Subcommittee of the Oncologic Drugs Advisory Committee, FDA should make it clear to companies that pediatric cancer should be part of the mainstream of oncology drug development and can even be a primary target for new agents.

One of the significant challenges identified by the pediatric oncology community is the need to identify and prioritize individual drugs for study. To address this, there must be better methods for preclinical screening of new drugs as well as research into molecular disease mechanisms that might identify new therapeutic targets or surrogate endpoints that decrease sample size or study length.

There have been some steps taken that have the potential to improve drug development for pediatric cancers. The Best Pharmaceuticals for Children Act has resulted in a number of written requests for pediatric studies. Although FDA developed a program specifically for pediatric oncology, as of December 2002, the agency had granted only four of 30 requests for exclusivity, resulting in the addition of pediatric use to product labeling for those products. We believe that more must be done to expand the number of drugs being tested for pediatric oncology indications.

The larger cancer community is encouraged by recent initiatives for improving cancer drug development, but we remain concerned that pediatric cancer may be left behind in that effort. The July 16 announcement to establish a new cancer office and program is a positive step towards consolidation of review for oncology products. While we hope that these organizational changes improve coordination, the continuing fragmentation of pediatric oncology expertise and responsibility remains unaddressed and is a potential barrier to the more rapid pediatric oncology drug development that we seek.

We urge you to make pediatric oncology drug development a priority by strongly encouraging companies to focus their drug development efforts on children with cancer whenever data are promising. The sooner that children with cancer have access to new drugs, the sooner they will be able to benefit.

Sincerely,

American Cancer Society
American Society of Clinical Oncology
Association of Pediatric Oncology Nurses
Candlelighters Childhood Cancer Foundation
Chai Lifeline
Children's Brain Tumor Foundation
National Coalition for Cancer Survivorship
Pediatric Brain Tumor Foundation of U.S.
The Children's Cause