December 5, 2003



Mark McClellan, MD, PhD
Commissioner
Food and Drug Administration
14-71 Parklawn Bldg.
5600 Fishers Lane
Rockville, MD 20857

RE: Citizen Petition of the Abigail Alliance and the Washington Legal Foundation (Docket No. 2003P-0274)

Dear Dr. McClellan:

As you know, clinical trials in pediatric cancer constitute one of the most impressive success stories in medical science. Whereas no more than 5% of adult cancer patients participate in clinical trials, the overwhelming majority of children with cancer are treated in the context of a clinical trial conducted at major pediatric cancer centers. As a result, most American children enjoy access to the highest quality cancer care, and significant advances have been achieved in cancer treatment for many childhood cancers. The undersigned groups oppose the Citizen Petition filed by the Abigail Alliance because we fear that the relief requested would undermine quality care for individual pediatric cancer patients while also deterring progress against pediatric cancer generally.

The Citizen Petition seeks to create a new category of Food and Drug Administration (FDA) approval referred to as "Tier 1." This approval would be on a patient-by-patient basis and could occur after collection of as little clinical data as those developed in Phase 1 trials. Unlike the situation under current law, companies providing drugs to patients under Tier 1 approval would be permitted to charge full market price. This change is apparently the primary—and perhaps the only—difference between the proposal and current law, which permits liberal access to investigational drugs through the "compassionate use" regulatory mechanism, assuming the willingness and ability of the sponsoring company to make such drugs available to individuals not enrolled in clinical trials.

We believe this proposal is misguided for the following reasons:

1. Phase 1 data do not provide sufficient evidence of safety, much less efficacy, to enable patients to access investigational drugs on demand outside a clinical trial. Concerns for patient safety are likely to be even greater for children than for adults with cancer.

2. Ready access to investigational drugs absent enrollment in clinical trials will jeopardize the very successful track record of clinical trials

participation, especially in children. What patient or parent of a patient will submit to randomization if a desired investigational drug is available without enrolling in a clinical trial?

3. Since Tier 1 drugs would almost certainly not be covered by third-party payers, only the wealthy would be able to enjoy access to them, thus creating an unseemly disparity between them and less affluent patients.

4. FDA's authority as the gatekeeper for access to new drugs would be undermined as patients, for the first time in modern memory, would have access to potentially toxic or ineffective drugs without the benefit of FDA review.

At present, children with cancer receive high quality cancer care, often through participation in clinical trials. The current system provides assurances to pediatric cancer patients and their families through the quality of clinical trials data, the integrity of ethical review processes and the strength of FDA regulatory oversight. If adopted, the proposal would threaten these assurances and pose a significant risk to both individual patients and the overall clinical research enterprise. We trust that FDA will take steps to ensure that the proposal is not accepted.

Sincerely,

American Society of Clinical Oncology
American Society for Therapeutic Radiology & Oncology
Association of Pediatric Oncology Nurses
Cancer Research Foundation of America/Hopestreet Kids
Candlelighters Childhood Cancer Foundation
Childrens Oncology Group
Leukemia & Lymphoma Society
National Childhood Cancer Foundation
National Patient Advocate Foundation
Pediatric Brain Tumor Foundation of US