Mobilizing to Conquer
Childhood Cancer

December 15, 2003

Dear Dr. McClellan:

The Children Cause is writing to you at this time because of our concern about the Abigail Alliance Citizen Petition's proposal to create a new category of Food and Drug Administration (FDA) approval, referred to as "Tier 1." We oppose the creation of such a category of approval and believe it would expose seriously ill children to unnecessary harm. Further, we maintain that Tier 1 will retard the pace of pediatric oncology research and cause delays in determining whether agents are safe and effective in treating children with lifethreatening illness.

The Children's Cause is a nonprofit, consumer-based, independent advocacy voice for those affected by childhood cancer. Through education and training, The Children's Cause enables family members and survivors to become national advocates on policies that affect research, treatment, healthcare, and services for patients and survivors. With advice from medical, scientific and policy experts, we work to ensure the rapid and safe development of new therapies, quality healthcare, and appropriate follow-up care for long term survivors.

The Citizen Petition seeks to allow FDA approval for agents after data from Phase 1 trials are available so that individual patients may have access to an agent outside of clinical trials. Further, Tier 1 would allow companies to sell their products at this point in development to individual patients.

For the following reasons, we believe that FDA should reject the Tier 1 proposal:

1. Tier 1 would allow excessive risk of harm to children – even those struggling with lifethreatening illness. Tier 1 would allow any agent with adult Phase 1 data to be used in a child with cancer without any restriction by disease type, stage, or treatment history. Phase 1 data from adults are typically insufficient to determine whether toxicities may be unacceptably severe in children. Further, given the many differences between pediatric and adult cancers, Phase 1 data are not designed to test the efficacy of an agent.

2. Individual access to drugs in development outside of clinical trials is likely to jeopardize advances in the treatment of pediatric cancer. Even with current high enrollment rates, it takes over 10 years to incorporate a new agent into standard therapy in pediatric oncology. Decreasing the numbers of children enrolled in clinical trials will make the safety and efficacy evaluation of new therapies virtually impossible.

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3. The Tier 1 proposal will impose additional cost burdens for families seeking treatment for their children. Families typically do not pay for the costs of agents in development because such drugs are part of research studies to evaluate them. Under Tier 1, access to drugs in development would almost certainly be available only to the wealthy. We believe that changes in childhood cancer policy should reduce – not increase -- economic barriers for families struggling to get treatment for their children with cancer.

Families are often willing to take substantial risks on behalf of their children with cancer. Indeed, some attempt to gain single-patient access to agents in development for their children toward the end of life. We believe that the current FDA rules governing single patient use provide adequate and appropriate protections and assurances to pediatric cancer patients and their families. Families, however, tell us that it can be difficult and stressful to attempt to get information about these procedures through the FDA and through sponsoring companies.

Therefore, we recommend that FDA take steps to create clear, easily accessible information to the public about single patient use procedures through the FDA website and printed materials.

We support FDA's current policies about single patient use and urge greater public education about procedures to make single patient use available. We trust that FDA will take steps to ensure that the Tier 1 proposal is not accepted and that the interests of children with cancer continue to be protected.

Sincerely yours,

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President

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