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DATE OF CORRESPONDENCE: 11/13/03 **DATE INTO FDA:** 11/17/03

TO: MARK MCCLELLAN, FDA - COMMISSIONER

FROM: ELIZABETH GOSS, ROPES & GRAY LLP

SYNOPSIS: FORWARDS LETTER ON BEHALF OF VARIOUS ORGANIZATIONS WHO ARE IN OPPOSITION TO THE CITIZEN PETITION FOR THE ABIGAIL ALLIANCE AND THE WASHINGTON LEGAL FOUNDATION (DOCKET # 2003P-0274).

LEAD OFFICE: HFA-305 **HOME OFFICE:** HF-40

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COORDINATION:

SIGNATURE REQUIRED:

REFERRALS FROM HF-40

ASSIGNED TO	ACTION	DUE DATE
HFA-305	NECESSARY ACTION	

November 13, 2003

Mark McClellan, M.D.
Commissioner
Food and Drug Administration
5600 Fishers Lane
Room HF-1
Rockville, Maryland 20857

RE: Citizen Petition of the Abigail Alliance and the Washington Legal Foundation (Docket 2003P-0274)

Dear Dr. McClellan:

RESPONSE TO CITIZEN PETITION

The undersigned organizations representing cancer patients, providers and researchers submit this response to a citizen petition filed June 17, 2003, on behalf of the Abigail Alliance and the Washington Legal Foundation. The citizen petition seeks a revision in Food and Drug Administration (FDA) policy to implement a new category of "approval" to make investigational drugs available to certain categories of patients outside of clinical trials. For the reasons set forth below, the undersigned organizations oppose the requested relief, despite the compassion that we all feel for patients with life-threatening diseases and limited treatment options.

Request for Relief

The petitioners urge creation of a new "Tier 1 Initial Approval" of new drugs for the treatment of life-threatening diseases with unmet needs, available after as little as a single phase 1 clinical trial. If adopted, the new approval status would permit charging a market price in excess of the cost-recovery currently permitted for certain investigational drugs but would apply "only to patients who have been found ineligible for or denied participation in a clinical trial for the same drug or who, in the judgment of their physician, are not reasonable candidates for a clinical trial." Sponsors receiving such a limited approval would be required to continue clinical trials in support of accelerated or full approval. In essence, the proposal seems to differ from current law and practice primarily in the ability of the sponsor to charge full market price for drugs receiving the limited approval.

Access to New Drugs Under the Current System

While some of the undersigned organizations have offered criticism in the past regarding the pace of review of new drugs for the treatment of cancer, there has been noted improvement in the review of oncology drugs during the past several years under the leadership of Dr. Richard Pazdur, and more recently during the brief tenure of Commissioner Mark McClellan. Specifically, the willingness of FDA to review and approve new cancer drugs on an accelerated basis using surrogate endpoints has hastened access to life-extending therapies. In addition, FDA has been receptive to facilitating access to "compassionate use" drugs for those ineligible for clinical trials where industry is willing and able to accommodate such requests.

Interest of the Undersigned Organizations

The undersigned organizations take many public policy positions in support of people with cancer. Among these are the following:

1. We support sound and thorough research as the best means to ensure access to quality cancer care through drugs or other interventions;
2. We support participation in quality clinical trials as beneficial to individual patients as well as to overall progress against cancer; and
3. We support third-party reimbursement for all approved drugs, including all medically appropriate unlabeled (or unapproved) uses of approved drugs in the treatment of cancer.

Our opposition to the petition is based on a belief that the relief requested is inconsistent with these principles.

Specific Concerns of the Undersigned Organizations

Safety and Efficacy of New Drugs

For a variety of reasons, FDA should not offer its stamp of "approval" to new products based on the meager data suggested by petitioners. First, it is hard to reconcile petitioner's request with the statutory requirements of "substantial evidence" or "adequate and well-controlled investigations." 21 U.S.C. § 355(d). Second, the request underestimates the risks of using largely untested, often toxic therapies, even in patients with likely terminal illnesses. A change in policy should be based, in part, on new evidence of comparative risks and benefits, and, in this instance, both risks and benefits are likely to be almost entirely unknown, but the risks are certainly not minimal.

Clinical Trials Participation

If the petition were adopted, clinical trials would inevitably be jeopardized. While the suggested criteria limit access to drugs under the new approval status to patients who are ineligible or otherwise denied enrollment in a relevant clinical trial, patients may also access new drugs if, "in the judgment of their physician, [they] are not reasonable candidates for a clinical trial." These criteria are an open invitation to avoid randomization and the other burdens of clinical trials, either through the subjective assessment of physicians or by submitting to an initial round of relatively mild chemotherapy that will render the patient ineligible. Though everyone sympathizes with those suffering from life-threatening disease, accommodation to individual patients cannot be allowed to undermine the integrity and efficiency of the clinical trials system that is the linchpin of drug development in this country and throughout the world.

Reimbursement Concerns

With the increased use of surrogate endpoints as the basis for accelerated approval, third-party payers have begun to question their longstanding obligation to cover drugs for cancer and other life-threatening diseases on the ground that clinical benefit in the form of survival has not been established. (For example, at least two new major cancer drugs approved during the past year have been subjected to unprecedented coverage reviews by the Centers for Medicare & Medicaid Services.) Acceptance of the scheme urged by petitioners would exacerbate the new second-guessing of FDA by reimbursement authorities. The likely result that third-party payers would, at the very least, decline to cover the so-called "Tier 1" drugs would create severe inequities in an environment where companies were permitted to charge full market price, as only wealthy patients would be able to afford to pay for unreimbursed drugs.

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For the above reasons, the undersigned organizations urge the Commissioner to reject the citizen petition submitted by the Abigail Alliance and the Washington Legal Foundation.

Sincerely,

American Cancer Society
American Society of Clinical Oncology
American Society for Therapeutic Radiology & Oncology, Inc.
Cancer Care, Inc.
Cancer Research and Prevention Foundation
Coalition of National Cancer Cooperative Groups
Colorectal Cancer Network
International Myeloma Foundation
The Leukemia & Lymphoma Society
National Coalition for Cancer Survivorship
National Patient Advocate Foundation
National Prostate Cancer Coalition
Y-ME National Breast Cancer Organization

cc: Dockets Management

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