CANCER LEADERSHIP COUNCIL

A PATIENT-CENTERED FORUM OF NATIONAL ADVOCACY ORGANIZATIONS ADDRESSING PUBLIC POLICY ISSUES IN CANCER

October 20, 2017

Eric Hargan
Acting Secretary
Department of Health and Human Services
Hubert H. Humphrey Building
200 Independence Avenue, SW
Washington, DC 20201

Re: Massachusetts Section 1115 Demonstration Amendment Request

Dear Acting Secretary Hargan:

The undersigned organizations, representing cancer patients and survivors, oncologists, and other cancer care professionals, are writing in opposition to the proposal from the Commonwealth of Massachusetts to amend its Section 1115 waiver. We are concerned that the waiver amendment, by establishing a closed formulary and by substantially rejecting the regulatory decisions of the Food and Drug Administration, will adversely affect the access of cancer patients to necessary treatment.

We applaud the Commonwealth of Massachusetts for achieving nearly universal health insurance coverage. This achievement is meaningful to cancer patients who have relied on MassHealth, the state's Medicaid program, for access to treatment. We also appreciate the fiscal challenges that the Commonwealth faces in sustaining its accomplishment, and we understand its interest in implementing cost containment initiatives. However, we are concerned that the plan outlined by the Commonwealth could hinder access to quality cancer care.

The Commonwealth proposes to adopt a "commercial-style closed formulary with at least one drug available per therapeutic class" for MassHealth. We are concerned that a closed formulary would unreasonably restrict access to appropriate cancer therapies for those enrolled in MassHealth. Cancer patients often require combination drug therapy that includes multiple drugs, and a closed formulary with one drug per class may pose significant obstacles to obtaining access to cancer drug therapy. Moreover, cancer patients may have a benefit from one drug in a class but not others and may also suffer very distinct side effects. For these reasons, a closed formulary is not in the best interest of cancer patients.

At a time when cancer treatments are increasingly targeted and, in some cases personalized, a closed formulary moves MassHealth in a direction that will make the proper targeting of cancer treatments more difficult. If a patient needs a targeted therapy that is not on the closed

formulary, it is unclear that an exceptions process will be adequate for that individual or for a system moving toward targeted cancer treatment.

The Commonwealth indicates that it wishes access to "widely-used commercial tools" to obtain lower drug prices and enhanced rebates. The Commonwealth does not explain why the tools that are currently available to it, including use of a preferred drug list, prior authorization, and a rebate system, are not adequate for managing its drug program and how a closed formulary would better serve its needs.

We also object to the way in which the waiver application characterizes the process for accelerated approval of drugs that is utilized by the Food and Drug Administration (FDA) and the suggestion of the Commonwealth that it will substitute its own review process, in partnership with the University of Massachusetts, in place of FDA review. The Commonwealth maintains that there is limited or inadequate evidence of clinical efficacy of drugs that are approved according to the FDA accelerated approval process.

We take exception to the characterization of accelerated approval as a process that is based on inadequate evidence of clinical efficacy. In a guidance document addressing the standards for several review programs, FDA states about accelerated approval:

> Drugs granted accelerated approval must meet the same statutory standards for safety and effectiveness as those granted traditional approval. For effectiveness, the standard is substantial evidence based on adequate and well-controlled clinical investigations. For safety, the standard is having sufficient information to determine whether the drug is safe for use under conditions prescribed, recommended, or suggested in the proposed labeling. Under accelerated approval, FDA can rely on a particular kind of evidence, such as a drug's effect on a surrogate endpoint, as a basis for approval. FDA carefully evaluates such evidence to ensure that any remaining doubts about the relationship of the effect on the surrogate to clinical benefit are resolved by additional postapproval studies or trials. An application for accelerated approval should also include evidence that a proposed surrogate endpoint or an intermediate clinical endpoint is reasonably likely to predict the intended clinical benefit of a drug.[1]

It is also important to note that accelerated approval is intended to be utilized in situations where there is an unmet medical need for a serious condition or disease. FDA uses this approval pathway to ensure that therapies for serious illnesses are available as soon as it can be concluded that their benefits justify their risks. Many cancer drugs are reviewed through the accelerated approval pathway, and as a result of the appropriate utilization of this review pathway, cancer patients have enjoyed the benefits of a number of drugs for forms of cancer that would otherwise have inadequate treatment options.

[1] U.S. Department of Health and Human Services, Food and Drug Administration, Guidance for Industry: Expedited Programs for Serious Conditions – Drugs and Biologics, 2014.

Even if we conceded that the standards of the accelerated approval process are lacking, we would not find the review process generally referenced by the Commonwealth to be an appropriate substitute review process. The waiver application does not detail the standards for review, the data that would be reviewed (clinical trials data, proprietary data from drug sponsors, real world clinical evidence) or the process for review. There is no definition of a conflicts of interest policy for the efficacy review to be undertaken by the Commonwealth, and there is no process defined for public comment about the efficacy review. As cancer advocates, we have demanded measures of transparency as well as public participation in FDA review, and we do not see the Commonwealth review process meeting those standards.

Many cancer patients rely on MassHealth for access to quality cancer care. We urge the Centers for Medicare & Medicaid Services (CMS) to reject the waiver application submitted by the Commonwealth of Massachusetts because it may compromise access to quality care for people with cancer and others with serious illnesses.

Sincerely,

Cancer Leadership Council

Cancer*Care*
Cancer Support Community
Fight Colorectal Cancer
International Myeloma Foundation
The Leukemia & Lymphoma Society
Lymphoma Research Foundation
National Coalition for Cancer Survivorship
Prevent Cancer Foundation
Sarcoma Foundation of America
Susan G. Komen