FRIENDS of CANCER RESEARCH

May 2, 2023

Administrator Chiquita Brooks-LaSure
Centers for Medicare & Medicaid Services (CMS)
200 Independence Ave., S.W.
Washington, D.C.

Dear Administrator Brooks-LaSure:

Last week you told a Congressional committee that CMS "consider(s) accelerated approval in a different category." This was in response to questions by the committee about an earlier decision by CMS to not pay for an Alzheimer's drug approved by the Food and Drug Administration (FDA) under accelerated approval unless patients were enrolled in a randomized clinical trial of that drug. In setting the agency's policy for this Alzheimer's drug, CMS had previously described their decision as a reflection of, "very unique circumstances around this class of treatments" due in part to the uncertainty associated with the novel intermediate endpoint used for accelerated approval and available data regarding outcomes and potential harms for the Medicare population. However, it would be detrimental if a unique circumstance were used to establish a new category of FDA approved drugs.

The Accelerated Approval Program has enabled timely access to new cancer therapies for over 30 years, with approximately a third of all new cancer drugs receiving approval through this mechanism. Such approvals are based on an intermediate measure, such as tumor shrinkage, which has evidence to support its association with clinical benefit but can be evaluated earlier than long-term endpoints such as overall survival. While understanding the long-term effect of the drug is important, accelerated approval allows for earlier patient access to promising therapies based on the earlier measure while additional data is being gathered to confirm the benefit.

Over 200 cancer treatments have received accelerated approval, with only 26 instances where an approval was rescinded or withdrawn (less than 13% of all accelerated approvals) because the additional studies required did not confirm benefit. This demonstrates the importance of timely completion of confirmatory studies (something Congress recently provided FDA additional authorities to ensure) yet also highlights the success of the program. Recent estimates demonstrate that the Accelerated Approval Program enables patient access to new drugs in several cases years earlier – time which is critical for people faced with serious or life-threatening illnesses.

When the FDA approves a drug, it has determined that drug is effective and that its expected benefits outweigh the potential risks – of either the drug itself or the underlying illness. As such, clinical guidelines are quickly updated to reflect the new evidence to inform doctors, as well as insurance companies, including government insurance programs such as Medicare, who pay for the drug to ensure patients have access.

The apparent position shift from addressing unique circumstances for a specific drug class to categorically reclassifying all accelerated approvals for the purposes of reimbursement sets a dangerous precedent. It not only ignores the significant medical expertise and role of the FDA, but it also places risk to thousands of cancer patients and those suffering from serious illnesses that could be subject to access impediments that may accompany CMS's new categorization if it applies to all accelerated approvals.

We respectfully ask that CMS clarify its position on accelerated approvals, particularly how it applies to reimbursement of cancer drugs approved by FDA and/or included in clinical guidelines developed by subject matter experts.

Sincerely,

Ellen V. Sigal
Chair and Founder, Friends of Cancer Research