FDA NEWS RELEASE

FDA approves first cancer treatment for any solid tumor with a specific genetic feature

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The U.S. Food and Drug Administration today granted accelerated approval to a treatment for patients whose cancers have a specific genetic feature (biomarker). This is the first time the agency has approved a cancer treatment based on a common biomarker rather than the location in the body where the tumor originated.

Keytruda (pembrolizumab) is indicated for the treatment of adult and pediatric patients with unresectable or metastatic solid tumors that have been identified as having a biomarker referred to as microsatellite instability-high (MSI-H) or mismatch repair deficient (dMMR). This indication covers patients with solid tumors that have progressed following prior treatment and who have no satisfactory alternative treatment options and patients with colorectal cancer that has progressed following treatment with certain chemotherapy drugs.

“This is an important first for the cancer community,” said Richard Pazdur, M.D., acting director of the Office of Hematology and Oncology Products in the FDA’s Center for Drug Evaluation and Research and director of the FDA’s Oncology Center of Excellence. “Until now, the FDA has approved cancer treatments based on where in the body the cancer started—for example, lung or breast cancers. We have now approved a drug based on a tumor’s biomarker without regard to the tumor’s original location.”

MSI-H and dMMR tumors contain abnormalities that affect the proper repair of DNA inside the cell. Tumors with these biomarkers are most commonly found in colorectal, endometrial and gastrointestinal cancers, but also less commonly appear in cancers arising in the breast, prostate, bladder, thyroid gland and other places. Approximately 5 percent of patients with metastatic colorectal cancer have MSI-H or dMMR tumors.

Keytruda works by targeting the cellular pathway known as PD-1/PD-L1 (proteins found on the body’s immune cells and some cancer cells). By blocking this pathway, Keytruda may help the body’s immune system fight the cancer cells. The FDA previously approved Keytruda for the treatment of certain patients with metastatic melanoma, metastatic non-small cell lung cancer, recurrent or metastatic head and neck cancer, refractory classical Hodgkin lymphoma, and urothelial carcinoma.

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Merck Sharp v. Johns Hopkins
IPR2024-00649

Keytruda was approved for this new indication using the Accelerated Approval (/patients/fast-track-breakthrough-therapy-accelerated-approval-and-priority-review/accelerated-approval) pathway, under which the FDA may approve drugs for serious conditions where there is unmet medical need and a drug is shown to have certain effects that are reasonably likely to predict a clinical benefit to patients. Further study is required to verify and describe anticipated clinical benefits of Keytruda, and the sponsor is currently conducting these studies in additional patients with MSI-H or dMMR tumors.

The safety and efficacy of Keytruda for this indication were studied in patients with MSI-H or dMMR solid tumors enrolled in one of five uncontrolled, single-arm clinical trials. In some trials, patients were required to have MSI-H or dMMR cancers, while in other trials, a subgroup of patients were identified as having MSI-H or dMMR cancers by testing tumor samples after treatment began. A total of 15 cancer types were identified among 149 patients enrolled across these five clinical trials. The most common cancers were colorectal, endometrial and other gastrointestinal cancers. The review of Keytruda for this indication was based on the percentage of patients who experienced complete or partial shrinkage of their tumors (overall response rate) and for how long (durability of response). Of the 149 patients who received Keytruda in the trials, 39.6 percent had a complete or partial response. For 78 percent of those patients, the response lasted for six months or more.

Common side effects of Keytruda include fatigue, itchy skin (pruritus), diarrhea, decreased appetite, rash, fever (pyrexia), cough, difficulty breathing (dyspnea), musculoskeletal pain, constipation and nausea. Keytruda can cause serious conditions known as immune-mediated side effects, including inflammation of healthy organs such as the lungs (pneumonitis), colon (colitis), liver (hepatitis), endocrine glands (endocrinopathies) and kidneys (nephritis). Complications or death related to allogeneic hematopoietic stem cell transplantation after using Keytruda has occurred.

Patients who experience severe or life-threatening infusion-related reactions should stop taking Keytruda. Women who are pregnant or breastfeeding should not take Keytruda because it may cause harm to a developing fetus or newborn baby. The safety and effectiveness of Keytruda in pediatric patients with MSI-H central nervous system cancers have not been established.

The FDA granted this application Priority Review (/patients/fast-track-breakthrough-therapy-accelerated-approval-and-priority-review/priority-review) designation, under which the FDA's goal is to take action on an application within six months where the agency determines that the drug, if approved, would significantly improve the safety or effectiveness of treating, diagnosing or preventing a serious condition.

The FDA granted accelerated approval of Keytruda to Merck & Co.

The FDA, an agency within the U.S. Department of Health and Human Services, protects the public health by assuring the safety, effectiveness, and security of human and veterinary drugs, vaccines and other biological products for human use, and medical devices. The agency also is responsible for the safety and security of our nation's food supply, cosmetics, dietary supplements, products that give off electronic radiation, and for regulating tobacco products.

Related Information

- FDA: Office of Hematology and Oncology Products (/about-fda/about-center-drug-evaluation-and-research/office-hematology-and-oncology-products-ohop)
- FDA: Approved Drugs: Questions and Answers (/drugs/information-consumers-drugs/approved-drugs-questions-and-answers)
- FDA: Fast Track, Breakthrough Therapy, Accelerated Approval, and Priority Review ()

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