FDA Approves First New Kaposi Sarcoma Treatment in Over 20 Years

The immune modulator Pomalyst led to remission in two thirds of people with AIDS-related KS.

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On May 14, the Food and Drug Administration (FDA) granted accelerated approval of pomalidomide (Pomalyst) for the treatment of AIDS-related Kaposi sarcoma (KS) in HIV-positive who are not responding to antiretroviral therapy as well as for KS in HIV-negative people.

“Pomalyst has shown positive results in Kaposi sarcoma patients, regardless of their HIV status,” Robert Yarchoan, MD, chief of the National Cancer Institute’s HIV and AIDS Malignancy Branch, said in a press release. “Also, it provides a therapy that is taken orally and works by a different mechanism of action than the cytotoxic chemotherapy drugs generally used to treat Kaposi sarcoma.”

Kaposi sarcoma is a cancer that involves the uncontrolled growth of cells that line blood and lymph vessels. It usually causes red or purple skin lesions, but it can also affect the mouth and internal organs including the lymph nodes, lungs and digestive system. It is caused by Kaposi sarcoma-associated herpesvirus, also known as human herpesvirus type 8.

One of the first observed manifestations of AIDS, KS has decreased dramatically in the era of effective antiretroviral treatment, as fewer people living with HIV have advanced immune suppression and very low CD4 counts. Nonetheless, KS remains the most common AIDS-defining cancer, even as rates of some non-AIDS cancers are rising as HIV-positive people live longer.

Pomalyst is an immunomodulatory drug related to thalidomide. It boosts immune function by promoting the activity of T-cells and natural killer cells. In addition, it inhibits the growth of cancer cells and blocks development of blood vessels that feed tumors. It was previously approved for multiple myeloma, a blood cancer that involves white blood cells in the bone marrow.

The new approval was supported by findings from Study 12-C-0047, a Phase I/II clinical trial conducted by the National Cancer Institute. This open-label study included 18 HIV-positive and 10 HIV-negative people with KS. Most had advanced disease, but those with symptomatic KS affecting their lungs or internal organs were excluded, as were those with a history of blood clots.

All participants received 5 milligrams of oral Pomalyst once daily on days 1 through 21 of each 28-

day cycle until they experienced disease progression or unacceptable side effects. They also received daily aspirin to reduce the risk of blood clots. All the HIV-positive participants were taking combination antiretroviral therapy.

Among the HIV-positive participants, the overall response rate was 67%, with a median response duration of 12.5 months. In the HIV-negative group, the overall response rate was 80%, with a median response duration of 10.5 months. In both groups combined, four people (14%) experienced complete remission, and half of the patients who responded were still doing so more than a year later.

Pomalyst is generally safe but side effects are common. In Study 12-C-0047, 11% of participants stopped treatment due to adverse events.

The most common adverse reactions include rash, constipation, nausea, diarrhea and lab test abnormalities including elevated creatinine, glucose or ALT liver enzymes and decreased phosphate or calcium. It can cause depletion of white blood cells (neutropenia), red blood cells (anemia) and platelets (thrombocytopenia) low white blood cell counts (neutropenia), which can lead to infections, fatigue and easy bleeding.

Less common but more serious side effects include deep vein thrombosis (blood clots), pulmonary embolism, heart attacks and strokes. Like thalidomide, Pomalyst can cause severe birth defects if used during pregnancy, and people of childbearing potential must use reliable contraception.

“Patients with Kaposi sarcoma have had few options to manage their disease for two decades,” Diane McDowell, MD, of Bristol Myers Squibb said in a company press release. “We’re excited that the additional research into Pomalyst in this rare disease area has resulted in our ability to provide a much-needed oral treatment option for patients.”

Drugs that receive accelerated approval based on response rates in early studies are expected to undergo further testing in larger randomized trials to confirm clinical benefits such as improved survival, and the FDA can rescind approval if they don’t measure up.

Click here for full prescribing information for Pomalyst.

Click here to learn more about Kaposi sarcoma.
