FDA Approves Xpovio for Heavily Treated Multiple Myeloma

First nuclear export inhibitor gets the nod for patients lacking treatment options.

July 3, 2019 By Liz Highleyman

The Food and Drug Administration (FDA) has granted accelerated approval of Xpovio (selinexor) in combination with dexamethasone for the treatment of relapsed or refractory multiple myeloma in people who have tried or are unable to use a variety of other therapies.

Although an expert panel voted against approval of Xpovio because of safety concerns and a desire for more data from a randomized trial, the FDA ultimately overruled the advisors, judging that the benefits outweigh the risks for people who have run out of treatment options.

"While there is no cure for multiple myeloma, there are FDA-approved treatments to target the cancer and slow down the spread of the disease. Sadly, often over time, patients can exhaust all available treatments and still see their disease progress," Richard Pazdur, MD, director of the FDA's Oncology Center of Excellence, said in a news release. "Today we approved a treatment under our accelerated approval program that provides a treatment option for patients with multiple myeloma with no available therapy."

Multiple myeloma is a blood cancer that involves uncontrolled growth of plasma cells, a type of immune system white blood cell that produces antibodies. These malignant mature B cells multiply in the bone marrow and can interfere with normal blood-forming cells and cells that build new bone, leading to blood cell deficiencies and fractures. They produce abnormal antibody fragments, known as M proteins, that are unable to fight infection.

Treatment for multiple myeloma may include traditional chemotherapy, steroids such as dexamethasone and prednisone, immunomodulators such as Revlimid (lenalidomide) and Pomalyst (pomalidomide), proteasome inhibitors including Velcade (bortezomib) and Kyprolis (carfilzomib) and other targeted therapies such as the CD38 inhibitor Darzalex (daratumumab). While current treatments can often slow disease progression, relapse is common.

Xpovio is the first approved selective inhibitor of nuclear export. It works by binding to the nuclear export protein exportin 1 (XPO1), which blocks the transport of numerous tumor suppressor, growth-regulating and anti-inflammatory proteins. These proteins then accumulate in the cell nucleus, which enhances their anticancer activity. The forced nuclear retention of these proteins

can counteract oncogenic pathways that allow uncontrolled growth of cells with severe DNA damage, according to the drug's manufacturer, Karyopharm Therapeutics.

Xpovio was approved for people who have received at least four prior therapies and whose cancer is resistant to other forms of treatment, including at least two immunomodulatory drugs, two proteasome inhibitors and an anti-CD38 monoclonal antibody.

This approval was based on results from the Phase IIb STORM trial, which included a subgroup of 83 people with relapsed or refractory multiple myeloma who were previously treated with several other therapies. Participants in this open-label study received 80 milligrams of Xpovio plus 20 mg of dexamethasone. Xpovio is taken as a pill on days 1 and 3 each week until disease progression or unacceptable toxicity.

The overall response rate in this group, meaning complete or partial cancer remission, was 25%, and the median duration of response was 3.8 months. This included 16 partial responses (19%), four very good partial responses (5%) and one so-called stringent complete response (1%).

About a quarter of study participants discontinued treatment because of side effects and just over half required dose reductions; 9% had fatal adverse events.

Common side effects of Xpovio plus dexamethasone include fatigue, nausea, vomiting, decreased appetite, weight loss, diarrhea, constipation fever and low sodium levels (hyponatremia). It can cause depletion of red blood cells (anemia), white blood cells (neutropenia) and platelets (thrombocytopenia), which can lead to infections and bleeding. The Xpovio label includes warnings about severe thrombocytopenia, neutropenia, gastrointestinal problems, infections and neurological side effects. Patients are advised to avoid taking Xpovio with other medications that may cause dizziness or confusion, and to avoid situations where dizziness may be a problem.

Xpovio received FDA fast track status and an orphan drug designation. It was granted accelerated approval based on the response rate; continued approval may be contingent upon the drug showing a clinical benefit in a confirmatory trial, according to the FDA. The Phase III BOSTON trial, evaluating Xpovio plus Velcade and low-dose dexamethasone, is currently under way. Xpovio is also being studied for diffuse large B-cell lymphoma and other types of cancer.

"Despite recent advances in the treatment of multiple myeloma, almost all our patients will develop disease that is resistant to the five most commonly used anti-myeloma drugs we currently have available, and the prognosis for this patient population is particularly poor," Paul Richardson, MD, of Dana-Farber Cancer Institute, said in a Karyopharm press release. "The accelerated approval of oral Xpovio marks an important advance in the treatment paradigm for patients with relapsed refractory multiple myeloma, and in my view, is an important addition to our therapeutic armamentarium."

Click here for full prescribing information for Xpovio.

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