FDA Approves Immunotherapy for Liver Cancer

Opdivo will be used to treat hepatocellular carcinoma, which can be caused by hepatitis B or C.

September 25, 2017 By Liz Highleyman

On September 22, the Food and Drug Administration (FDA) approved Opdivo (nivolumab), a treatment that helps the immune system fight cancer, for people with hepatocellular carcinoma (HCC), a type of liver cancer that can result from hepatitis B or C.

The accelerated approval was based on results from the CheckMate 040 trial, which showed a response rate of 14.3 percent among HCC patients previously treated with Nexavar (sorafenib), according to a Bristol-Myers Squibb press release. As with many immunotherapy trials, although only a minority of patients may benefit, those who do often respond quite well.

Over years or decades, chronic hepatitis B or C, heavy alcohol use, fatty liver disease and other causes of liver injury can lead to development of cirrhosis and hepatocellular carcinoma. Liver cancer is often detected late and is difficult to treat. Nexavar, a tyrosine kinase inhibitor, is the only FDA-approved first-line treatment, but it doesn't work for many HCC patients, and most will experience disease progression. Stivarga (regorafenib), a similar drug, was recently approved for second-line HCC treatment. Several other experimental liver cancer therapies have failed in clinical trials.

Opdivo is a monoclonal antibody that interferes with the PD-1 receptor, an immune "checkpoint" on T cells, the main soldiers of the immune system. Some tumors can hijack PD-1 to disable immune responses. Drugs that block PD-1 can release the brakes and restore T-cell activity against cancer cells. Opdivo is currently approved for several types of cancer, including advanced lung, kidney and bladder cancer.

Opdivo is the first immunotherapy to be approved for liver cancer. It is indicated for patients with HCC who were previously treated with Nexavar, and it can be used by people with hepatitis B or C. Unlike some other immunotherapy approvals, there are no restrictions based on PD-L1 expression level.

The recommended dose of Opdivo is 240 milligrams administered by intravenous infusion every two weeks until disease progression or unacceptable toxicity, according to the drug's prescribing information.

The multinational Phase I/II trial Checkmate 040 trial enrolled 262 people with advanced HCC who could not be cured by surgery to remove their tumors. The majority were men and the average age was 63. About two thirds of participants had previously used Nexavar. About a quarter had hepatitis B, another quarter had hepatitis C and half had neither virus. Nearly 70 percent had metastases, or cancer spread beyond the liver. Study results were published in The Lancet and presented earlier this year at the American Society of Clinical Oncology (ASCO) annual meeting.

In one part of the study, 154 patients who experienced disease progression on Nexavar or were unable to tolerate it were treated with 3 milligrams per kilogram of Opdivo every two weeks.

Overall, 14.3 percent of this group demonstrated some degree of response, or tumor shrinkage. Of these, 1.9 percent had complete responses and 12.3 percent had partial responses. Another 40 percent had stable disease without progression. Among the 22 patients classified as responders, 55 percent had responses lasting a year or more. Responses were observed across PD-1 expression levels.

The results presented at ASCO indicated that people who had never used Nexavar had somewhat better long-term outcomes, but the FDA has not yet approved Opdivo for this group of patients.

Treatment with Opdivo was generally safe in people with compensated cirrhosis and HCC. More than 10 percent had seriously elevated liver enzymes and 5 percent developed immune-mediated hepatitis requiring treatment with corticosteroids. Eleven percent discontinued treatment due to adverse reactions, according to the press release.

The major concern with checkpoint inhibitors like Opdivo is immune-related adverse events. These drugs work by restoring immune responses against cancer cells, but they can also take the brakes off the immune system more broadly, leading to excessive inflammation of healthy tissue.

Continued approval of Opdivo for HCC may be contingent upon the demonstration of clinical benefit in later-stage trials that are currently underway, according to the press release. Opdivo is also being studied alone and in combination with other drugs for a variety of different cancer types.

To read the Bristol-Myers Squibb press release, click here.

To see the full prescribing information for Opdivo, click here.

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