FDA Approves Tibsovo Targeted Therapy for Cholangiocarcinoma

The IDH1 inhibitor is the third medication approved this year for hard-to-treat bile duct cancer.

September 2, 2021 By Liz Highleyman

On August 25, the Food and Drug Administration approved Tibsovo (ivosidenib), an IDH1 inhibitor, for the treatment of locally advanced or metastatic cholangiocarcinoma with a specific genetic mutation. This is the third targeted therapy to win approval for this malignancy in the past four months.

“This new treatment will positively impact the lives of patients diagnosed with IDH1-mutated cholangiocarcinoma and will advance our progress for the whole cholangiocarcinoma community,” said Stacie Lindsey, founder and CEO of the Cholangiocarcinoma Foundation.

Tibsovo, from Servier Pharmaceuticals, inhibits isocitrate dehydrogenase-1 (IDH1), an enzyme that plays a role in cellular metabolism. It was approved for cholangiocarcinoma with an IDH1 mutation as detected by an FDA-approved diagnostic test; around 15% to 20% of cholangiocarcinoma patients have this mutation. The drug was initially approved for acute myeloid leukemia in 2018.

Cholangiocarcinoma is a rare cancer of the bile ducts within or outside the liver. If detected early, bile duct tumors can sometimes be surgically removed, but more often, the cancer is diagnosed at later stages. Advanced cholangiocarcinoma often progresses rapidly and has a high mortality rate.

Tibsovo was evaluated in the Phase III ClarIDHy trial (NCT02989857). Interim results were presented at the 2019 European Society for Medical Oncology Congress, with an update at this year’s American Society of Clinical Oncology annual meeting.

The study enrolled 185 adults with locally advanced or metastatic cholangiocarcinoma with an IDH1 mutation that progressed despite prior treatment. They were randomly assigned to receive once-daily Tibsovo or a placebo pills until they experienced disease progression or unacceptable side effects. Those who progressed in the placebo group were allowed to cross over to the Tibsovo arm, and about 70% did so.

The overall response rate was low in both groups: Just three Tibsovo recipients and no placebo recipients saw their tumors shrink. However, Tibsovo significantly delayed disease progression, with a 63% improvement in progression-free survival. Just over half (52%) of Tibsovo recipients

experienced disease progression compared with 72% of placebo recipients; 10% of patients died in each group.

Overall survival was also higher in the Tibsovo group, but the difference was not statistically significant. The median overall survival was 10.3 months in the Tibsovo group versus 7.5 months in the placebo group. The large proportion of placebo recipients who crossed over to Tibsovo likely made it harder to see a survival advantage.

The most common adverse reactions in people taking Tibsovo were fatigue, nausea, vomiting, abdominal pain, diarrhea, cough, decreased appetite, ascites (abdominal fluid accumulation), anemia, rash and elevated aspartate aminotransferase and bilirubin.

“Patients living with IDH1-mutated cholangiocarcinoma, especially those whose disease progresses following chemotherapy, are in urgent need of new treatment options,” Rachna Shroff, MD, of the University of Arizona Cancer Center, said in a Servier press release. “In addition to an acceptable safety profile, Tibsovo demonstrated an impressive, significant benefit in progression-free survival, underscoring its importance as a new option for patients battling this aggressive cancer.”

Servier announced that it has launched ServierONE Patient Support Services, a program that offers one-on-one support for patients and helps them access financial assistance, emotional support and other resources. More information can be found at www.servierone.com.

Click here for full prescribing information for Tibsovo.

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