FDA Grants Accelerated Approval to Futibatinib (Lytgobi) for Cholangiocarcinoma

Cholangiocarcinoma is a rare cancer of the bile ducts that is often diagnosed late and can progress rapidly.

October 3, 2022 By Food and Drug Administration (FDA)

On September 30, 2022, the Food and Drug Administration granted accelerated approval to futibatinib (Lytgobi, Taiho Oncology, Inc.) for adult patients with previously treated, unresectable, locally advanced or metastatic intrahepatic cholangiocarcinoma harboring fibroblast growth factor receptor 2 (FGFR2) gene fusions or other rearrangements.

Efficacy was evaluated in TAS-120-101 (NCT02052778), a multicenter, open-label, single-arm trial that enrolled 103 patients with previously treated, unresectable, locally advanced, or metastatic intrahepatic cholangiocarcinoma harboring a FGFR2 gene fusion or other rearrangement. The presence of FGFR2 fusions or other rearrangements was determined using next generation sequencing testing. Patients received 20 mg of futibatinib orally once daily until disease progression or unacceptable toxicity.

The major efficacy outcome measures were overall response rate (ORR) and duration of response (DoR) as determined by an independent review committee according to RECIST v1.1. ORR was 42% (95% Confidence Interval [CI]: 32, 52); all 43 responders achieved partial responses. The median DoR was 9.7 months (95% CI: 7.6, 17.1).

The most common adverse reactions occurring in 20% or more of patients were nail toxicity, musculoskeletal pain, constipation, diarrhea, fatigue, dry mouth, alopecia, stomatitis, abdominal pain, dry skin, arthralgia, dysgeusia, dry eye, nausea, decreased appetite, urinary tract infection, palmar-plantar erythrodysesthesia syndrome, and vomiting.

The recommended futibatinib dose is 20 mg orally once daily until disease progression or unacceptable toxicity occurs.

View full prescribing information for Lytgobi.

This review used the Real-Time Oncology Review (RTOR) pilot program, which streamlined data submission prior to the filing of the entire clinical application, and the Assessment Aid, a voluntary

submission from the applicant to facilitate the FDA’s assessment.

This application was granted priority review and breakthrough designation. A description of FDA expedited programs is in the Guidance for Industry: Expedited Programs for Serious Conditions-Drugs and Biologics. The application also was granted orphan drug designation.

Healthcare professionals should report all serious adverse events suspected to be associated with the use of any medicine and device to FDA’s MedWatch Reporting System or by calling 1-800-FDA-1088.

This announcement was published by the Food and Drug Administration on September 30, 2022.
