FDA Approves Truseltiq for Metastatic Cholangiocarcinoma

New targeted therapy shrank tumors in nearly a quarter of patients with bile duct cancer.

June 1, 2021 By Food and Drug Administration (FDA)

FDA grants accelerated approval to infigratinib for metastatic cholangiocarcinoma

On May 28, 2021, the Food and Drug Administration granted accelerated approval to infigratinib (Truseltiq, QED Therapeutics, Inc.), a kinase inhibitor for adults with previously treated, unresectable locally advanced or metastatic cholangiocarcinoma with a fibroblast growth factor receptor 2 (FGFR2) fusion or other rearrangement as detected by an FDA-approved test.

The FDA also approved FoundationOne CDx (Foundation Medicine, Inc.) for selection of patients with FGFR2 fusion or other rearrangement as a companion diagnostic device for treatment with infigratinib.

Efficacy was demonstrated in CBGJ398X2204 (NCT02150967), a multicenter open-label single-arm trial, that enrolled 108 patients with previously treated, unresectable locally advanced or metastatic cholangiocarcinoma with an FGFR2 fusion or rearrangement as determined by local or central testing. Patients received infigratinib 125 mg orally once daily for 21 consecutive days followed by 7 days off therapy, in 28-day cycles until disease progression or unacceptable toxicity.

The major efficacy outcome measures were overall response rate (ORR) and duration of response (DoR), as determined by blinded independent central review according to RECIST 1.1. The ORR was 23% (95% CI: 16, 32), with 1 complete response and 24 partial responses. Median DoR was 5 months (95% CI: 3.7, 9.3). Among the 23 responders, 8 patients maintained the response for 6 months or more.

The most common (incidence ≥ 20%) adverse reactions were hyperphosphatemia, increased creatinine, nail toxicity, stomatitis, dry eye, fatigue, alopecia, palmar-plantar erythrodysesthesia syndrome, arthralgia, dysgeusia, constipation, abdominal pain, dry mouth, eyelash changes, diarrhea, dry skin, decreased appetite, vision blurred and vomiting. The serious risks include hyperphosphatemia and retinal pigment epithelial detachment and monitoring for these adverse reactions during treatment is recommended.
The recommended infigratinib dose is 125 mg orally once daily on an empty stomach for 21 consecutive days followed by 7 days off therapy, in 28-day cycles.

View full prescribing information for Truseltiq.

This indication is approved under accelerated approval based on the overall response rate and duration of response. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trial(s).

This review was conducted under Project Orbis, an initiative of the FDA Oncology Center of Excellence. Project Orbis provides a framework for concurrent submission and review of oncology drugs among international partners. For this review, FDA collaborated with the Australian Therapeutic Goods Administration (TGA) and Health Canada. The application reviews are ongoing at the other regulatory agencies.

This review used the Real-Time Oncology Review (RTOR) pilot program, which streamlined data submission prior to the filing of the entire clinical application, as well as the Assessment Aid and the Product Quality Assessment Aid (PQAA), voluntary submissions from the applicant to facilitate the FDA's assessment.

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

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.

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