FDA Grants Accelerated Approval of Ziihera for HER2-Positive Biliary Tract Cancer

Zanidatamab, a bispecific antibody, is indicated for previously treated unresectable or metastatic bile duct or gallbladder cancer.

November 26, 2024 By Food and Drug Administration (FDA)

On November 20, 2024, the Food and Drug Administration granted accelerated approval to zanidatamab-hrii (Ziihera, Jazz Pharmaceuticals, Inc.), a bispecific HER2-directed antibody, for previously treated, unresectable or metastatic HER2-positive (IHC 3+) biliary tract cancer (BTC), as detected by an FDA-approved test.

Today, the FDA also approved Ventana Pathway anti-HER-2/neu (4B5) Rabbit Monoclonal Primary Antibody (Ventana Medical Systems, Inc./Roche Diagnostics) as a companion diagnostic device to aid in identifying patients with BTC who may be eligible for treatment with Ziihera.

Full prescribing information for Ziihera will be posted on Drugs@FDA.

Efficacy and Safety

Efficacy was evaluated in HERIZON-BTC-01 (NCT04466891), an open-label multicenter, single-arm trial in 62 patients with unresectable or metastatic HER2-positive (IHC3+) BTC. Patients were required to have received at least one prior gemcitabine-containing regimen in the advanced disease setting.

The major efficacy outcome measures were objective response rate (ORR) and duration of response (DOR) as determined by an independent central review according to RECIST v1.1. ORR was 52% (95% CI: 39, 65) and median DOR was 14.9 months (95% CI: 7.4, not estimable).

The prescribing information contains a boxed warning for embryo-fetal toxicity. The most common adverse reactions reported in at least 20% of patients who received zanidatamab-hrii were diarrhea, infusion-related reactions, abdominal pain, and fatigue.

Expedited Programs

This application was granted priority review, breakthrough therapy designation, and orphan drug designation. FDA expedited programs are described 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.

For assistance with single-patient INDs for investigational oncology products, healthcare professionals may contact OCE's Project Facilitate at 240-402-0004 or email OncProjectFacilitate@fda.hhs.gov.

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