FDA Approves Cabometyx for Differentiated Thyroid Cancer

The targeted therapy delayed disease progression or death by approximately nine months.

September 23, 2021 By Food and Drug Administration (FDA)

On September 17, 2021, the Food and Drug Administration approved cabozantinib (Cabometyx, Exelixis, Inc.) for adult and pediatric patients 12 years of age and older with locally advanced or metastatic differentiated thyroid cancer (DTC) that has progressed following prior VEGFR-targeted therapy and who are ineligible or refractory to radioactive iodine.

Efficacy was evaluated in COSMIC-311, a randomized (2:1), double-blind, placebo-controlled, multicenter clinical trial (NCT03690388) in patients with locally advanced or metastatic DTC that had progressed following prior VEGFR-targeted therapy and were ineligible or refractory to radioactive iodine. Patients were randomized to receive either cabozantinib 60 mg orally once daily or placebo with best supportive care until disease progression or intolerable toxicity.

The primary efficacy outcome measures were progression-free survival (PFS) in the intent-to-treat population and overall response rate (ORR) in the first 100 randomized patients, assessed by a blinded independent radiology review committee per RECIST 1.1. Cabometyx significantly reduced the risk of disease progression or death versus placebo ($p<0.0001$). The median PFS was 11.0 months (95% CI: 7.4, 13.8) in the cabozantinib arm compared to 1.9 months (95% CI 1.9, 3.7) for those receiving placebo. The ORR was 18% (95% CI: 10%, 29%) and 0% (95% CI 0%, 11%) in the cabozantinib and placebo arms, respectively.

The most common adverse reactions (≥25%) were diarrhea, palmar-plantar erythrodysesthesia (PPE), fatigue, hypertension, and stomatitis. A warning was added for hypocalcemia.

The recommended single-agent cabozantinib dose is 60 mg once daily until disease progression or unacceptable toxicity. The recommended cabozantinib dose in pediatric patients (12 years of age and older with BSA less than 1.2 m2) is 40 mg once daily until disease progression or unacceptable toxicity.

View full prescribing information for Cabometyx.

This review used the Assessment Aid, a voluntary submission from the applicant to facilitate the FDA's assessment. The FDA approved this application 2 months ahead of the FDA goal date.

This application was granted priority review, breakthrough 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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