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Generic Name:

Pexidartinib

Trade Name:

Turalio

Company:

Daiichi Sankyo

Notes:

On August 2, FDA approved [pexidartinib](#) capsules for the treatment of adult patients with symptomatic tenosynovial giant cell tumor (TGCT) associated with severe morbidity or functional limitations and not responsive to improvement with surgery.

TGCT is a rare tumor that affects the synovium (thin layer of tissue that covers the surfaces of the joint spaces) and tendon sheaths. The tumor is rarely malignant but causes the synovium and tendon sheaths to thicken and overgrow, resulting in damage to surrounding tissue. Pexidartinib is the first FDA-approved therapy to treat this rare disease.

Approval of pexidartinib was based on the results of a multicenter international clinical trial of 120 patients, 59 of whom received placebo. The primary efficacy endpoint was the overall response rate (ORR) analyzed after 25 weeks of treatment. The clinical trial demonstrated a statistically significant improvement in ORR in patients who received pexidartinib, with an ORR of 38% compared with no responses in patients who received placebo. The complete response rate was 15%, and the partial response rate was 23%. Twenty-two of 23 responders who had been followed for a minimum of 6 months after the initial response maintained their response for 6 or more months, and 13 out of 13 responders who had been followed for a minimum of 12 months after the initial response maintained their response for 12 or more months.

The prescribing information for pexidartinib includes a boxed warning to advise health professionals and patients about the risk of serious and potentially fatal liver injury. Health professionals should monitor liver tests before beginning treatment and at specified intervals during treatment. If liver tests become abnormal, pexidartinib may need to be withheld or the dose reduced or permanently discontinued, depending on the severity of the liver injury.

Pexidartinib is available only through a Risk Evaluation and Mitigation Strategy (REMS) Program.

Common side effects for patients taking pexidartinib were increased lactate dehydrogenase, increased aspartate aminotransferase, loss of hair color, increased alanine aminotransferase, and increased cholesterol. Additional adverse effects included neutropenia, increased alkaline phosphatase, decreased lymphocytes, eye edema, decreased hemoglobin, rash, dysgeusia, and decreased phosphate.

FDA advises health professionals to tell females of reproductive age and males with a female partner of reproductive potential to use effective contraception during treatment. Women who are pregnant or breastfeeding should not take pexidartinib because it may cause harm to a developing fetus or newborn baby.

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