FDA Approves Koselugo for Pediatric Neurofibromatosis

The kinase inhibitor is the first approved treatment for children with this rare disease.

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FDA approves selumetinib for neurofibromatosis type 1 with symptomatic, inoperable plexiform neurofibromas

On April 10, 2020, the Food and Drug Administration approved selumetinib (Koselugo, AstraZeneca) for pediatric patients, 2 years of age and older, with neurofibromatosis type 1 (NF1) who have symptomatic, inoperable plexiform neurofibromas (PN).

Selumetinib, a kinase inhibitor, is the first therapy approved for pediatric patients who have this debilitating, and often disfiguring, rare disease.

Efficacy of selumetinib was investigated in SPRINT (NCT01362803), a National Cancer Institute (NCI) sponsored, open-label, multicenter, single-arm trial in pediatric patients with NF1 and a measurable target PN that could not be surgically removed without risk of substantial morbidity. Patients in the efficacy population (N=50) were also required to have at least one significant morbidity related to the target PN. Morbidities present in ≥20% of patients included disfigurement, motor dysfunction, pain, airway dysfunction, visual impairment, and bladder/bowel dysfunction. Patients received selumetinib 25 mg/m2 orally twice a day until disease progression or unacceptable toxicity.

The primary efficacy outcome measure was overall response rate (ORR) as assessed by NCI and defined as the percentage of patients who experienced ≥20% reduction in tumor volume on Magnetic Resonance Imaging (MRI) confirmed on a subsequent MRI within 3-6 months. The ORR was 66% (n=33; 95% CI: 51,79). All patients had a partial response, and 82% of responders had sustained responses lasting at least 12 months. An independent central review of ORR was performed using the same response criteria and demonstrated an ORR of 44% (95% CI: 30,59).

The primary safety data were from 74 pediatric patients with NF1 and PN who received selumetinib during SPRINT. The most common adverse reactions (≥40% of patients) were vomiting, rash, abdominal pain, diarrhea, nausea, dry skin, fatigue, musculoskeletal pain, fever, acne, stomatitis, headache, paronychia, and pruritus.

Selumetinib can also cause cardiomyopathy, ocular toxicity including retinal vein occlusion, retinal pigment epithelial detachment and impaired vision, and increased creatinine phosphokinase. Selumetinib should be withheld, dosage reduced, or permanently discontinued based on the severity of adverse reactions.

The recommended selumetinib dose is 25 mg/m^2 orally twice a day on an empty stomach until disease progression or unacceptable toxicity.

View full prescribing information for Koselugo.

FDA granted this application priority review and Breakthrough Therapy designation. Selumetinib also received fast track and orphan drug designations. 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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