

Selumetinib active in children with neurofibromatosis type 1

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(HealthDay)—The oral selective inhibitor of MAPK kinase 1 and 2,

selumetinib, is active in children with neurofibromatosis type 1 and inoperable plexiform neurofibromas, according to a study published in the Dec. 29 issue of the *New England Journal of Medicine*.

Eva Dombi, M.D., from the Center for Cancer Research in Bethesda, Md., and colleagues conducted a phase 1 trial of selumetinib in [children](#) with neurofibromatosis type 1 and inoperable plexiform neurofibromas. Selumetinib was administered twice daily at a dose of 20 to 30 mg/m² of body-surface area. Selumetinib was also tested using a mouse model of neurofibromatosis type 1-related neurofibroma.

Twenty-four children (median tumor volume, 1,205 ml) received selumetinib. The researchers found that children were able to receive selumetinib on a long-term basis, with a median of 30 cycles (range, six to 56 cycles). A dose of 25 mg/m² was the maximum tolerated. Acneiform rash, gastrointestinal effects, and asymptomatic creatine kinase elevation were the most common [toxic effects](#) associated with selumetinib. Seventeen of the 24 children (71 percent) had confirmed partial responses with selumetinib treatment; 67 percent of 18 mice had decreases from baseline in neurofibroma volume. There was no evidence of disease progression to date (tumor volume increase of ≥ 20 percent from baseline).

"Our early-phase data suggested that children with neurofibromatosis type 1 and inoperable plexiform neurofibromas benefited from long-term dose-adjusted treatment with selumetinib without having excess toxic effects," the authors write.

The trial was partially funded by AstraZeneca, which provided the selumetinib.

More information: [Full Text \(subscription or payment may be required\)](#)

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