Vosoritide beneficial for children younger than 5 years with achondroplasia

December 30 2023, by Elana Gotkine

For children with achondroplasia, vosoritide is associated with a gain in

the change in height Z score from baseline and a mild adverse event profile, according to a study published in the January issue of *The Lancet Child & Adolescent Health*.

Ravi Savarirayan, M.D., from the Royal Children's Hospital at the University of Melbourne in Australia, and colleagues conducted a double-blind, randomized phase 2 trial in 16 hospitals across Australia, Japan, the United Kingdom, and the United States to examine the safety and efficacy of vosoritide in children younger than 60 months with a clinical diagnosis of achondroplasia confirmed by genetic testing.

Participants were enrolled into three cohorts based on age at screening: 24 to 59 months, 6 to 23 months, and 0 to 5 months (cohorts 1, 2, and 3, respectively). Seventy-five participants were recruited: 11 were assigned as sentinels who received vosoritide to determine the appropriate daily dose, and 32 were randomly assigned to receive vosoritide or placebo for 52 weeks.

The researchers found that adverse events occurred in all participants, most of which were transient injection-site reactions and injection-site erythema. Three patients in the vosoritide group and six in the placebo group (7 and 19 percent, respectively) had serious adverse events. For a change from baseline in height Z score, the least-squares mean the difference between the vosoritide and placebo groups was 0.25.

"We hope these data will support pediatricians and other health care specialists who are assessing the risks and benefits of initiating vosoritide treatment in children with achondroplasia younger than 5 years," the authors write.

Several authors disclosed ties to biopharmaceutical companies, including BioMarin, which manufactures vosoritide and funded the study.

More information: Ravi Savarirayan et al, Vosoritide therapy in children with achondroplasia aged 3–59 months: a multinational, randomised, double-blind, placebo-controlled, phase 2 trial, *The Lancet Child & Adolescent Health* (2023). DOI: 10.1016/S2352-4642(23)00265-1

Copyright © 2023 HealthDay. All rights reserved.

Citation: Vosoritide beneficial for children younger than 5 years with achondroplasia (2023, December 30) retrieved 18 June 2024 from https://medicalxpress.com/news/2023-12-vosoritide-beneficial-children-younger-years.html

This document is subject to copyright. Apart from any fair dealing for the purpose of private study or research, no part may be reproduced without the written permission. The content is provided for information purposes only.