Controversial treatment for dwarfism meets clinical trial goals. Is FDA approval coming?

A treatment for the most common cause of dwarfism met its goal of increasing height in a pivotal study, the drug's maker said [December 16], setting the stage for Food and Drug Administration approval.

The company, BioMarin, enrolled 121 children with achondroplasia, the most common cause of dwarfism. Those who got the treatment, called vosoritide, grew 1.6 centimeters more over the course of a year than those who received placebo, BioMarin said.

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Next year, BioMarin will begin making its case to global regulators, advancing a debate that has been polarizing among people with dwarfism. To many, vosoritide's effect on height is reason enough to approve the drug, as it might provide long-term relief from skeletal problems, hearing loss, and other complications brought by achondroplasia. To others, approving a treatment that has proved only to make children taller threatens to undermine years of advocacy without offering a guaranteed benefit.

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[The FDA] is likely to convene a panel of expert advisers before deciding on vosoritide, a meeting that would invite public comment from those who support the drug and those who stand against its approval.
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