

The effect of vosoritide on children with achondroplasia and the controversies surrounding its use

Achondroplasia is a form of dwarfism which affects around 1 in 25,000 births. It is a genetic disorder caused by mutations in the FGFR3 gene; this gene provides instructions for making a protein involved in the development and maintenance of bone tissue. The mutation causes abnormal bone growth and development leading to short stature in those affected; average adult height is 4'4 (feet and inches) in males and 4'1 in females. The mutation usually arises spontaneously however it can be inherited. Achondroplasia often causes other medical complications such as bowlegs and curvature of the spine.

Until recently, there has been no known "treatment" for achondroplasia. American company Biogen's development of the drug Vosoritide (sold under the name "Voxzogo") is unlike anything that has been seen before. It uses a synthetic form of a protein that humans produce naturally to target the "overactive signal that prevents bone growth in children with achondroplasia" according to Dr Melita Irving. In August 2021 it was approved by the EU for medical use after successful clinical trials; children administered Vosoritide grew an additional 2.1cm each year compared to those on the placebo. It is given by injection once a day and is suitable for children 2 years of age and older or until the growth plates close. Despite its negligible side effects, the use of the drug is a controversial topic among members of the dwarfism community. And so the question arises; is this really ethical?

Gillian Martin, chair of the Restricted Growth Association, a charity that supports people with dwarfism says "there are people who are genuinely afraid of where this could lead... [some] adults with dwarfism in the community feel that average-height parents are being an advocate for a disability that doesn't directly affect them. There is a fear – that this research is leading the way to eradicate dwarfism." Joe Stramondo, a professor of philosophy at San Diego State University says views tend to be stronger in the US, where there is "a more robust dwarf culture and identity". "We recognise our situation as one of oppression, and of being subjected to stigma as being the main source of our difficulty {more so than medical issues}. He believes that the drug has been designed to "normalise" people's bodies rather than to help with any co-occurring issues. Perhaps money should be spent on education and making the world a more accessible place instead of on vosoritide? Is it fair that the studies focus so much on individual's height?

"The main purpose of the drug is to improve the quality of life" says Melita Irving. The research's emphasis has been on "height as an endpoint because it represents many other things". It is not unusual for people with achondroplasia to have difficulty with tasks such as washing their hair and walking for longer periods because of limb length. "Samuel* does not," – perhaps extra height would help patients for this reason? And does it really matter if some individuals would prefer to be taller?

*Samuel is a child who has been on vosoritide

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For some context - This is a topic that is important to my family as my brother who was born with achondroplasia took part in a trial for vosoritide and is consequentially taller than he otherwise would've been.