FPIN's Help Desk Answers

Growth Hormone for Treatment of Idiopathic Short Stature in Children

SUFIAN ZAYED, MD, and DIANE J. MADLON-KAY, MD, MS, *University of Minnesota Medical Center, Smiley's Family Medicine Residency Program, Minneapolis, Minnesota*

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Clinical Question

Is recombinant growth hormone (rGH) effective in the treatment of idiopathic short stature in children?

Evidence-Based Answer

Final adult height is modestly increased in children who receive rGH, with growth increases of 3.5 to 7.5 cm. However, the use of rGH does not improve health-related quality of life. (Strength of Recommendation: B, based on systematic reviews of low-quality randomized controlled trials [RCTs] and nonrandomized trials.)

A Cochrane review examined the effect of treatment with rGH vs. no treatment or placebo in 741 children with height less than the 3rd percentile who had no apparent underlying pathology.[1] One trial found that the near-final height in 10 pubertal girls who received 30 IU of rGH per m^2 per week for three years was 7.5 cm greater than that in 30 untreated girls (155 cm vs. 148 cm; $P = .003$). Another trial that reported adult height found that 37 children who received rGH were 3.7 cm taller than those in the placebo group ($P < .04$). The remaining eight trials reported short-term height gains, which varied from none to 0.7 standard deviations in a year. One study that reported health-related quality of life found no significant improvement in 20 children who received rGH compared with 20 children in a control group. The quality of the studies was rated as moderate for two, poor for six, and very poor for two.

A systematic review included only trials that evaluated adult height (three RCTs [N = 115] and seven nonrandomized trials [N = 477]).[2] Participants had a growth velocity of less than 1.5 cm per year or a bone age of 15 years in females or 16 years in males. The treatment group in the RCTs received 0.033 to 0.067 mg of rGH per kg per day. The adult height of the treated group exceeded that of the control group, with a mean difference of 0.65 standard deviations (about 4 cm; 95% confidence interval [CI], 0.40 to 0.91). In the seven nonrandomized trials, the mean adult height of treated children exceeded that of the control group by a mean difference of 0.45 standard deviations (about 3 cm; 95% CI, 0.18 to 0.73). Eight of the studies were rated low quality because of a high percentage of dropouts, and two of the RCTs were rated moderate quality.

In 2008, 32 leaders in the field of idiopathic short stature published a consensus statement based on a review of the literature and clinical experience.[3] The consensus was that rGH therapy following country-specific regulatory guidelines is appropriate, and that stronger consideration should be given to shorter children. The mean increase in adult height with rGH therapy is 3.5 to 7.5 cm.

Address correspondence to Diane J. Madlon-Kay, MD, MS, at madlo001@umn.edu. Reprints are not available from the authors.

Author disclosure: No relevant financial affiliations.

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