Effect of growth hormone treatment on adult height in peripubertal children with idiopathic short stature: A randomized, double-blind, placebo-controlled trial

Leschek, Ellen Werber
Rose, Susan R.
Yanovski, Jack A.
Troendle, James F.
Quigley, Charmian A.
Chipman, John J.
Crowe, Brenda J.
Ross, Judith L.
Cassorla, Fernando G.
Blum, Werner F.
Cutler, Gordon B.
Baron, Jeffrey

GH is often used to treat children with idiopathic short stature despite the lack of definitive, long-term studies of efficacy. We performed a randomized, double-blind, placebo-controlled trial to determine the effect of GH on adult height in peripubertal children. Subjects (n = 68; 53 males and 15 females), 9-16 yr old, with marked, idiopathic short stature [height or predicted height \( \leq -2.5 \) SD score (SDS)] received either GH (0.074 mg/kg) or placebo sc three times per week until they were near adult height. At study termination, adult height measurements were available for 33 patients after mean treatment duration of 4.4 yr. Adult height was greater in the GH-treated group (-1.81 ± 0.11 SDS, least squares mean ± SEM) than in the placebo-treated group (-2.32 ± 0.17 SDS) by 0.51 SDS (3.7 cm; \( P < 0.02; \) 95% confidence interval, 0.10-0.92 SDS). A similar GH effect was demonstrated in terms of adult height SDS minus baseline height SDS and adult height SDS minus baseline predicted height S.