Growth Hormone (GH) Treatment in Achondroplasia

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SYNOPSIS

Achondroplasia is one of the most commonly nown types of skeletal dysplasia in the adult ading to short stature. Before beginning owth hormone (GH) treatment of short sta- ire in patients with achondroplasia, we valuated their growth pattern and their ypothalamic-pituitary function, including GH ecretion. We studied 22 patients with chondroplasia (7 males and 15 females: age ange, 3 to 12 years).

The z-score of their height at admission was 5.4±1.2 (mean ± SD), and that of their annual eight gain before admission was -3.1±1.3 mean ± SD). GH response to provocative tests vas normal in all patients except five: four howed subnormal (<10 ng/ml) response to L-)opa stimuli, and one patient showed sub- ormal (<20 ng/ml) response to GRF stimuli. The mean GH concentration during sleep was ound to be low (<5 ng/ml) in three patients. These three patients were suspected to have atent GH deficiency, as they also showed a narkedly low IGF-1 level and marked delay of)one age. LH, FSH, TSH, and cortisol response o provocative tests were normal in all the)atients.

We treated this group of patients with ecombinant human GH (1 IU/kg/week). In 18 patients who were treated with GH for more than 6 months, height velocity during GH therapy was significantly increased compared to that before GH therapy (4.1±0.8 cm/year vs 7.2±1.4 cm/year).

We conclude that parameters reflecting ypothalamic-pituitary function, particularly GH secretion, should be examined in achondroplasia patients, and that GH treatment may be beneficial in the treatment of short stature in achondroplasia.

INTRODUCTION

Achondroplasia is one of the well-known skeletal dysplasias resulting in short stature /1/. Average final height is about 130 cm in males, and about 120 cm in females. Though the etiology of the short stature in achondroplasia is attributed to bone disorder /2/, there may be an underlying hypothalamic-pituitary dysfunction including abnormal growth hormone (GH) secretion, as the majority of patients suffer from the dilatation of the ventricle, or hydrocephalus /3/, and show delayed development, particularly in motor skills /4,5/.

In this study, before beginning growth hormone (GH) treatment of short stature in patients with achondroplasia, we evaluated their growth pattern and their hypothalamic-pituitary function, including GH secretion. With these results in hand, we began treating them with recombinant human GH (1 IU/kg/week).

SUBJECTS

We studied 22 patients with achondroplasia: 7 males and 15 females, age range 3 to 12 years, with an average age of 6 years old.

METHODS

In all the patients, we evaluated their devel-

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