Alder Hey Children's NHS Foundation Trust

Growth Hormone Therapy in Patients with Noonan Syndrome

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Introduction

Noonan Syndrome (NS) is an autosomal dominant condition that affects 1 in 1000 to 2500 live births and is associated with short stature. Studies have shown that height velocity (HV) improved significantly with the use of growth hormone (GH) in the first year of treatment and sustained for the second year. We aimed to look at the effects of GH therapy in our cohort of patients with NS.

Method

In this retrospective study, we collected data on patients with NS who were treated with GH in a tertiary endocrine centre.

Table 1 shows the mean HV during the growth hormone treatment.

Length of GH Treatment (years)	Mean HV (cm/yr)
Prior to starting GH	5.16
1	7.76
2	6.51
3	4.95
4	5.15
5	4.03
6	7.5
7	3.7
8	6.3

Results

- Twelve patients with a mean birth weight of 3.3 kg (-0.5 SDS) were included in the study (M:10).
- GH was commenced at an average age of 8 years (± 3.3 years) and the mean treatment duration was 3 years (range 1-8 years).
- Average height SDS prior to starting treatment was -2.96, which improved to -2.05 after treatment for a variable period of time, demonstrating an overall average improvement of +0.91 SDS.
- Average height SDS improved from -2.96 to -2.50 after one year of treatment and subsequently to -2.22 following two years of treatment.
- Mean HV improved significantly (*p=0.007*) in the first year of GH treatment, which slowed (*p=0.2*) between the first and second year of GH therapy.
- Average starting dose of GH was 34mcg/kg/day, with an average maximum dose of 37mcg/kg/day during the course of GH treatment.
- 67% of patients had associated cardiac co-morbidities [including pulmonary valve stenosis, atrial septal defect and hypertrophic cardiomyopathy] as part of NS. Other co-morbidities included hearing problems, scoliosis, nystagmus and strabismus.

Conclusion

GH treatment in patients with NS does improve the HV in the first few years of treatment but the long term benefits remain to be ascertained.

Inspired by Children

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Growth and syndromes (to include Turner syndrome)

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