Growth hormone treatment in children with Silver-Russell syndrome

C.C.J. Smeets[1], J.S. Renes[1], M. van der Steen[2], A.C.S. Hokken-Koelega[1,2]
1. Dept. Paediatrics, Division of Endocrinology, Erasmus MC Sophia, Rotterdam, The Netherlands
2. Dutch Growth Research Foundation, Rotterdam, The Netherlands

l.smeets@erasmusmc.nl

Conclusion

Patients with Silver-Russell syndrome (SRS) are shorter at start of GH treatment than non-SRS patients born SGA. SRS patients show a better weight- and height gain in the first year of GH treatment and attain a similar adult height as non-SRS

Background

Silver-Russell syndrome (SRS) is characterized by small for gestational age (SGA) birth, dysmorphic features (picture 1) and severe short stature. SRS is a clinical diagnosis. In approximately 50% of patients, (epi)genetic alterations are detected (~40% hypomethylation of imprinting center region (ICR) 1 on 11p15, ~10% maternal uniparental disomy (mUPD) 7).
Growth hormone (GH) treatment improves height in short children born SGA. However, data on response to GH for SRS patients are very limited.

Picture 1: *Frontal bossing, triangular face and clinodactyly in SRS*

Objective

To investigate the response to GH treatment in SRS vs. non-SRS patients born SGA.

Methods

First year treatment response and adult height (AH) were compared between 33 SRS patients and 294 patients born SGA without SRS (non-SRS).
All SRS patients were diagnosed based on the scoring system of Netchine et al., 2007. The diagnosis was genetically confirmed in 19 (57.6%) patients (n=13 hypomethylation ICR1, n=6 mUPD7).
All subjects were treated with GH 1 mg/m2/day (0.035 mg/kg/d).

Results

- Mean height SDS increased in first year of GH with 0.93 SDS (from -3.45 to -2.52 SDS) in SRS vs. 0.83 SDS (from -2.99 to -2.16 SDS) in non-SRS (p=0.002).
- Weight for height SDS increased in first year of GH with 0.52 SDS in SRS vs. 0.30 SDS in non-SRS (p=0.001).
- Mean AH (n=22 SRS, n=131 non-SRS) was -2.19 SDS in SRS vs. -1.77 SDS in non-SRS (p=0.34). Total height gain was 1.37 SDS in SRS vs. 1.14 SDS in non-SRS (p=0.16). AH en total height gain were similar in genetically confirmed and idiopathic SRS.
- GH treatment was well tolerated in SRS as well as non-SRS. No adverse events related to GH treatment were observed.

Figure 1: *Height (SDS) at start of GH, after 1 year of GH and adult height in SRS and non-SRS.*

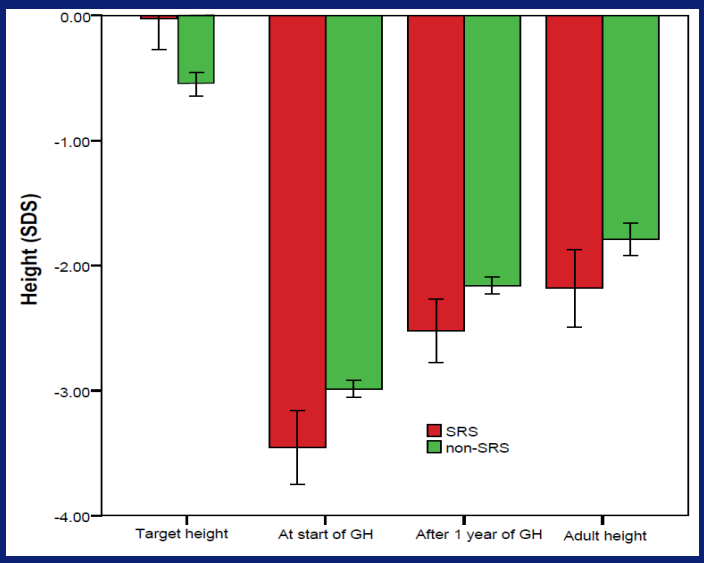

Table 1: *Patient characteristics*

	SRS	non-SRS	p-value
Boys/girls	17/16	158/136	
Age at start GH	5.2 (2.5)	6.5 (2.1)	0.77
Height SDS at start GH	-3.45 (0.83)	-2.99 (0.57)	0.003*
Weight/height SDS at start	-2.68 (1.26)	-1.35 (1.17)	0.72
Target height SDS	-0.03	-0.54	0.34
First year height gain SDS	0.93 (0.38)	0.83 (0.28)	0.002*
First year weight/height gain	0.52 (0.82)	0.30 (0.45)	0.001*
Adult height SDS	-2.18 (0.70)	-1.79 (0.75)	0.338
Total height gain SDS	1.37 (0.89)	1.14 (0.69)	0.164
Median duration of GH to AH (yrs)	9.94 (2.81)	8.59 (1.88)	0.17

No conflict of interest