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The efficacy and safety of recombinant biosimilar growth hormone treatment in children with GHD and SGA: Czech retrospective national longitudinal study

M. Snajderova[1], D. Zemkova[1], Z. Sumnik[1], J. Zapletalova[2], R. Pomahacova[3], S. Pruhova[1], J. Cermak[4], B. Sadovska[4]

[1]Second Faculty of Medicine, Charles University in Prague and University Hospital Motol, Prague, Czech Republic,
[2]Faculty of Medicine, Palacky University, Olomouc, Czech Republic,
[3]Faculty of Medicine, Charles University, Plzen, Czech Republic,
[4]Sandoz GmbH, Prague, Czech Republic.

INTRODUCTION

Recombinant growth hormone (rhGH) treatment helps to achieve a final height close to the parental growth potential in children with GH deficiency (GHD) and small for gestational age (SGA).

Less is known about efficacy and safety of long term therapy with biosimilar rhGH.

AIM

- To assess height gain and safety of therapy with biosimilar rhGH (Omnitrope®, Sandoz) in Czech children enrolled into the PATRO-Children study (PAtient TReated with Omnitrope) with GHD and SGA over the first three years of treatment.
- To compare our effectiveness data with longitudinal international study GeNeSIS Italian Cohort (Humatrope®, Eli Lilly).

METHODS

- Patients were treated with rhGH Omnitrope (Omnitrope®, Sandoz) by the dose recommended for GHD 0.025-0.035 mg/kg/day, and for SGA 0.035 mg/kg/day.
- Auxological data to assess height gain were compared with published data of GeNeSIS study.
- Adverse events (AEs) were analysed as well (questionnaire data).

RESULTS

Table 1. Characteristics of cohort.

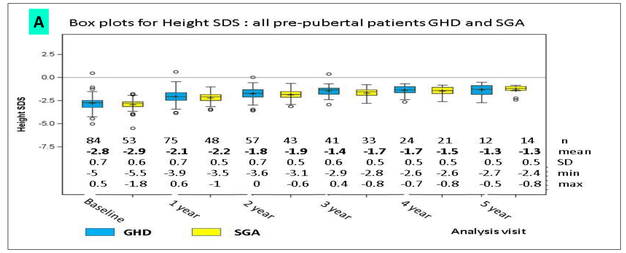
Patients	n	165
Female/Male	n	62/103
Idiopathic GHD	n	100
SGA	n	65
Age at start of GH therapy GHD cohort SGA cohort	years median (range)	 7.2 (2.7 - 15.7) 5.9 (2.9 - 14.3)
Duration of GH therapy GHD cohort SGA cohort	months (mean±SD)	 42.6 ± 19.2 45.9 ± 21.4

Growth data

Table 2. Data in GeNeSIS study[1] compared with PATRO Czech study (3 year cohort). Data show mean (95 % CI)

	GHD		SGA/IUGR	
	GeNeSIS Italy	PATRO Cz	GeNeSIS Italy	PATRO Cz
Number	193	55	8	40
At baseline	median (range)	median (range)	median (range)	median (range)
Age years	9.8 (9.3 - 10.3)	7.2 (6.6 - 8.3)	9.9 (6.4 - 10.8)	6.3 (5.5 - 7)
Height velocity cm/year	4.8 (4.1 - 5.6)	4.3 (4 - 4.5)	4 (1.97 - 6.01)	4.79 (4 - 5.4)
Height velocity SDS	-1.2 (-1.6 to -0.9)	-2 (-2.8 to -1.2)	-1.3 (-2.2 to -0.3)	-1.8 (-2.6 to -1.04)
Height SDS	-2.4 (-2.5 to -2.3)	-2.9 (-3.0 to -2.7)	-2.9 (-3.5 to -2.4)	-3.0 (-3.2 to -2.8)
GH dose mg/kg/week	0.23 (0.22 - 0.24)	0.21 (0.2 – 0.22)	0.23 (0.19 - 0.27)	0.25 (0.24 - 0.26)
1 year				
Height velocity cm/year	8.8 (8.5 - 9.2)	9.4 (8.8 - 9.9)	8.2 (7.1 - 9.2)	9.3 (8.9 - 9.7)
Height velocity SDS	2.3 (2.0 - 2.6)	3.5 (2.9 - 4.0)	1.6 (0.9 - 2.4)	2.3 (2.5 - 2.1)
Height SDS	-1.9 (-2 to - 1.8)	-2.1 (-2.3 to -2)	-2.5 (-3.2 to -1.9)	-2.3 (-2.4 to -2.1)

Fig 1. Body height (SDS) in all pre-pubertal Czech children included to PATRO Children study (A) and the difference between current and target height SDS during 3 years of follow-up: GHD and SGA cohort (B).

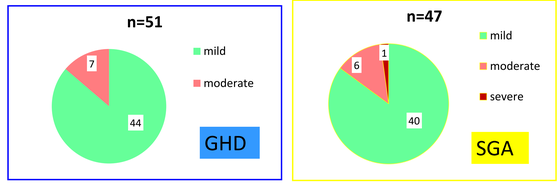


Safety of therapy

Table 3. Number of patients with adverse events related and probably unrelated to GH treatment, number of adverse events (AEs) in Czech GHD and SGA cohort.

	GHD (n=100) n (%)	SGA (n=65) n (%)	Total (n=165) n (%)
Subjects with AEs related to GH therapy	0	0	0
Subjects with AEs probably unrelated to GH therapy	23 (23)	26 (40)	49 (29.7)
Number of AEs	51	47	98

Fig 2. Incidence and intensity of adverse events (AEs), all probably unrelated to GH therapy in the Czech cohort during follow-up.



The side effects were in **most cases** in both the GHD and SGA cohorts of **mild intensity**. **Their association with GH treatment was unlikely**.
These included (in some cases recurrent) acute respiratory infections, diarrhea, urinary tract infections, and trauma (fracture of the arm and leg). GH therapy was interrupted for a short time in 2 children with GHD (acute infection) and in 1 SGA subject (trauma).

- No cases of serious adverse events associated with GH treatment were observed in the GHD and SGA cohorts.
- No cancer, headache, edema, hypertension, asthma, diabetes mellitus or other serious problems were observed during follow-up.

CONCLUSIONS

Our data confirm significant improvement of growth parameters and safety of therapy with biosimilar rhGH Omnitrope® in Czech children with GHD and SGA which is consistent with results from international database GeNeSIS study.

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CONTACT INFORMATION

marta.snajderova@lfmotol.cuni.cz

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Marta Snajderova
P2-297