Clinical and cost-effectiveness of Growth Hormone (GH) treatment for children in Wales

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INTRODUCTION

GH treatment has been used for the last 30 years for children with short stature with varying individual responses.

OBJECTIVE

Analysis of final height standard deviation score (SDS) & factors influencing it in children treated with GH.

METHODS

Children across Wales who received GH treatment, part supervised by tertiary center staff & reached final height while on treatment, were identified by database searching. Final height was defined when the switch to adult dosing occurred or a height velocity (HV) <1cm/year. Exclusion criteria included subjects on GH treatment for less than a year, a HV <1cm/year on GH or GH started after growth completion. Variables analyzed included age, sex, diagnosis, presence of concomitant hypothyroidism, adrenal failure, age at GH initiation, height SDS at diagnosis, number of years on treatment, puberty induction, mid-parental height SDS, age at GH cessation, mean GH dose throughout treatment, height gain at 1 year, total height gain & cost of treatment (£/cm gained). SPSS v. 17.0 was used for statistical analysis, with a level of significance of p=0.05.

RESULTS

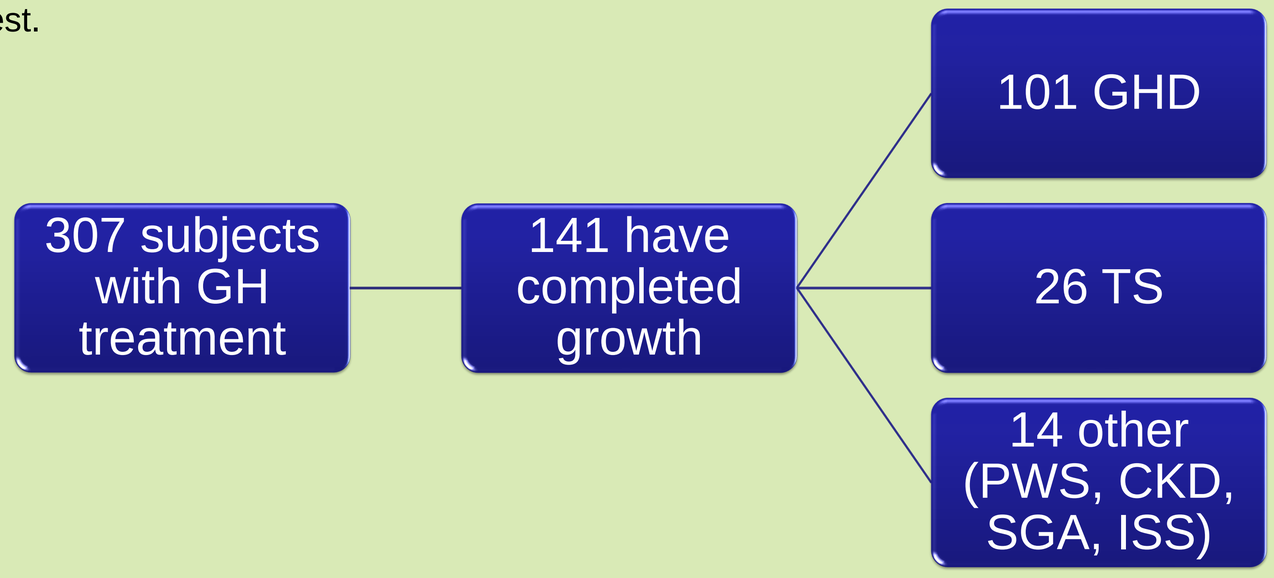
141 subjects were identified with a sex ratio of F:M of 1.2:1. 101 (71.6%) had GH deficiency (GHD) from various causes, 26 (18.4%) had Turner Syndrome (TS) & 14 (9.9%) had other diagnoses (Prader-Willi Syndrome, constitutional delay in growth and puberty, small for gestational age, renal disorders, idiopathic short stature). Mean age at treatment initiation was 10.4±3.3 years for the whole sample. The median period on GH treatment was 5.1years. 71 (50.3%) subjects required puberty induction, at a median age of 13.7 (12.4-14.7) years. Total height gain was 0.87SD for GHD, 0.09SD for TS & 0.74SD for the other diagnoses (p=0.043) (fig. 1) with a cost of £6324/cm for GHD, £8466/cm for TS & £4273/cm for other diagnoses (p=0.045) (fig. 2). 56 (53.3%) children reached a final height within mid-parental height range. Best predictors for response to treatment were height SD at diagnosis, HV in the first year, years on treatment & age at treatment start (r^2=0.68 (table 1) for GHD & 0.96 for TS). The mean final height for the group with TS was 148.7±6.1cm.

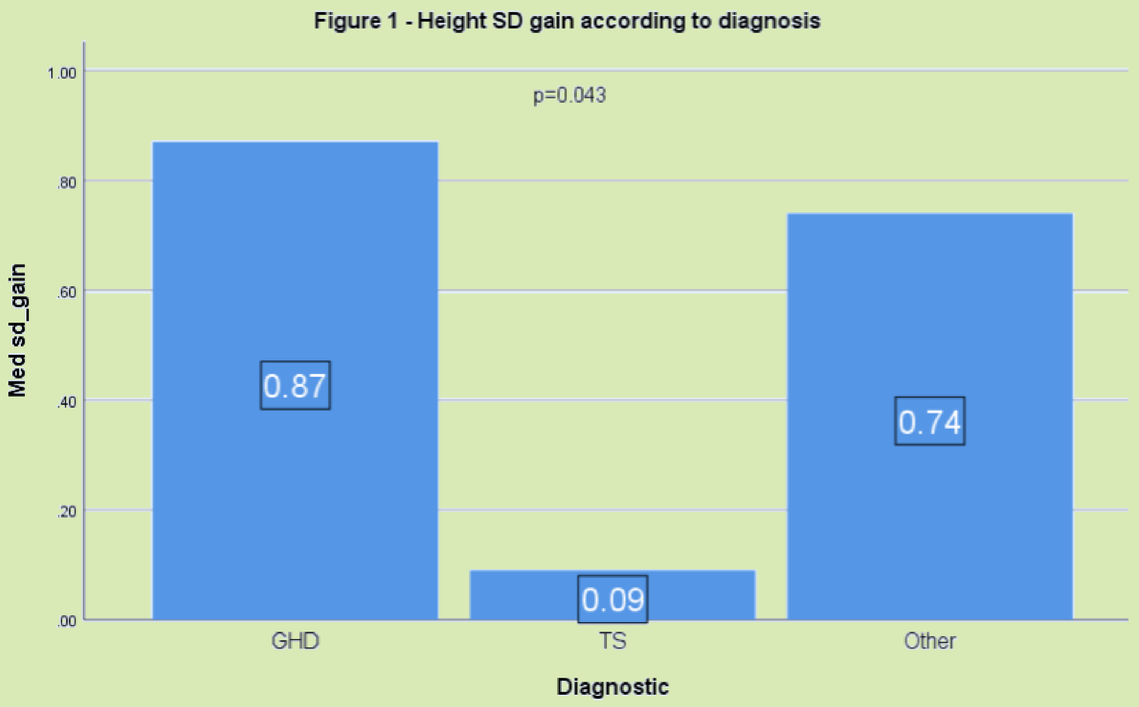
DISCUSSIONS

Height gain was smaller than that reported in other studies both for GHD (1) & TS (2), but the latter group was small & with a higher age at treatment initiation. The cost of treatment for GHD is similar to that reported by NICE, with a lower cost for TS (3). As previously demonstrated best predictors of treatment response are age at treatment initiation & HV in the first year, with the mean GH dose not a significant predictor, underlining the idea of a personalized approach to treatment (4).

CONCLUSIONS

GH treatment recommendation in Wales follows current guidelines. The response to treatment is variable, with a significant proportion of subjects not reaching the predicted final height. TS benefits the least from GH therapy.





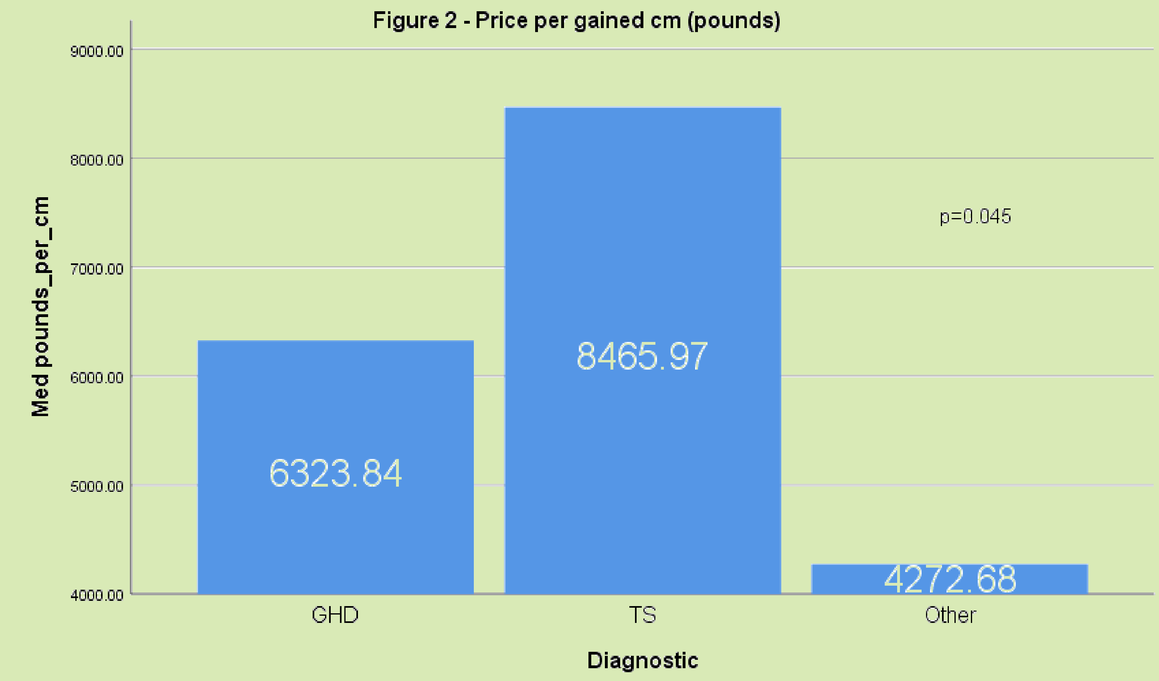


Table 1 – Linear regression results for GHD

Predictors	Beta	95.0% Confidence Interval for B		p
		Lower Bound	Upper Bound	
Sex (F=0)	0.158	-0.137	0.997	0.134
Levothyroxine	-0.249	-1.276	-0.100	0.023
Hydrocortisone	0.317	0.215	1.575	0.011
Age at treatment start	0.461	-0.007	0.429	0.057
Years on treatment	0.755	0.148	0.729	0.004
Puberty induction (Y/N)	-0.071	-0.856	0.465	0.555
Target height SDS	0.125	-0.118	0.514	0.213
Mean rHGH dose	-0.210	-0.124	0.015	0.123
Start rHGH dose	0.013	-0.059	0.066	0.909
Height velocity 1st year	0.299	0.031	0.198	0.008
Height SDS at diag.	-0.379	-0.524	-0.086	0.007

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