Somatotrophin treatment patterns: Real-world data from Argentina.

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INTRODUCTION

- Currently, in Argentina the use of somatotrophin have universal coverage for growth hormone deficit (GHD) and in non-growth hormone deficient children with the following diagnosis: Turner syndrome (TS), chronic renal insufficiency (CRI), Prader-Willi syndrome (PWS), children born small for gestational age (SGA)[1].
- Argentinean MoH issued the current guidelines in 2007[1].
- The Argentinean market has eight somatotrophin brands. On average, in the last three years, Genotropin™ was used for approximately 16.7% of the patients (17.7% GHD; 15.7% SGA; 23.3% TS).
- Use in children with idiopathic short stature (ISS) is included in the label but not included in national guidelines[1]. For that reason, that indication doesn´t have universal coverage.
- The national guidelines stablish the criteria for starting treatment related to symptoms, age, height and growth rate.
- The only reasons for treatment discontinuation are the following: 1) growth velocity increases <50% from baseline in the first year 2) the patient is close to the final height and growth velocity is <2 cm of total growth in 1 year, 3) intractable problems with adherence, or 4) final height is reached.
- Treatment discontinuation for other reasons are considered suboptimal adherence and it have a negative impact in growth response[2].
- Genotropin™ patient support program (PSP) started more than 10 years ago, all patients with an on-label Genotropin prescription need to be enrolled in the PSP in order to get the injection device.
- The analysis of the program allows us to have local real-world data, vital to improve adherence and growth outcomes in Argentinean children.

METHODS

- We conducted a retrospective analysis using data from patients enrolled in the Patient Support Program (PSP) from June 2012 to October 2022. The way to get the injection device, for all patients, is to enroll in the PSP. For that reason, all patients who received a prescription have been enrolled. All the data were provided by the patients or their caregivers.
- For this analysis age cutoff was 16 years at diagnosis and adherence was defined as treatment discontinuation for non-medical reasons (delay in authorization/delivery or family/patient decision).
- Patients with diagnosis at a pediatric age who reported starting treatment in adulthood were excluded for the analysis. Also, patients with diagnosis at a pediatric age who reported starting treatment in as children but were enrolled in the PSP after the age of 18 were excluded only for adherence analysis.
- Statistical analysis was done in STATA 14.

RESULTS

- 1437 patients provided data for analysis. The mean age at diagnosis was 7.04 yrs (SD 4.3), and mean age at start of treatment was 8.9 yrs (SD 4.23) –see analysis by diagnostic group in Table 1 and Figure 1).
- Analyzing the time elapsed between diagnosis and the start of treatment in GHD and SGA patients we could observe that after the publication of local guidelines value are more homogeneous. On the other hand, the number of patients treated increases substantially (see Figures 2 and 3).

Table 1: Age distribution by diagnosis

	n	Age DX (yrs)	SD	Age of the start of treatment (yrs)	SD
GHD	506	6.8	4.2	8.4	4.5
PWS	36	1.63	3	5.6	3.9
SGA-SS	690	7.7	4	9.3	3.6
TS	160	6.7	4.6	8.8	3.7
CRI	27	4.3	4.6	9.97	3.6
Other	18	7.1	5.01	10.5	6.2

Figure 1: Age distribution by diagnosis

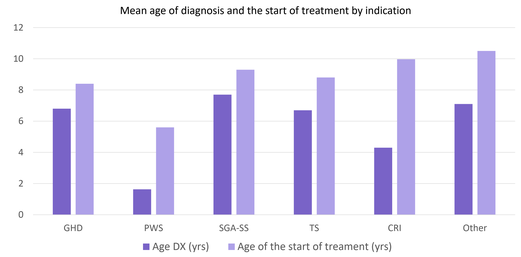

Figure 2: GHD patients

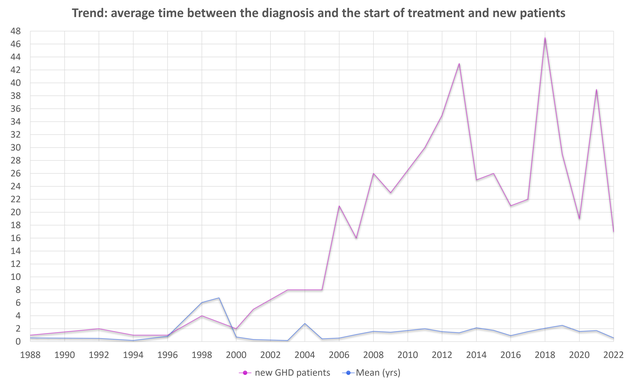

Figure 3: SGA patients

- Overall treatment adherence was 89.2% (n= 1408 after removing patients enrolled in the PSP after 18 yrs).
- Among the 149 adherence failure patients, 43.6% (n=65) discontinued treatment due to delay in the treatment authorization or delivery was and 56.4% (n=84) due to personal decision. Private health coverage was significantly associated with adherence, 91.2% vs 80.8% in the public sector (p=0.00).

OBJECTIVE

- To analyze and understand the treatment patterns and suboptimal adherence in Argentinean children prescribed Genotropin™.

CONCLUSIONS

- The observed adherence rate reported here was similar to reports in the literature[3]. However, discontinuation of treatment remains an issue, especially in the public sector. Strategies to improve adherence are therefore warranted in children treated with growth hormone.

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