

Pharmacoepidemiological Study on the Use of Growth Hormone Therapy in Portuguese Paediatric Patients

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BACKGROUND AND OBJECTIVE:

- Short stature is the most common cause of referral for paediatric endocrinology visits. It can be due to several reasons, from lack of growth hormone to genetic syndromes such as Prader-Willi or Turner syndrome.
- Recombinant growth hormone (rGH) is used in its treatment, but adherence to rGH therapy has been reported suboptimal in a large proportion of paediatric patients, which can lead to suboptimal clinical outcomes. (1,2,3)
- We aimed to characterize and to analyse adherence and treatment duration with rGH therapy, through National Health Service (NHS) hospitals electronic prescription databases from 2011 to 2020.

METHODS:

- This study used data from electronic prescription databases of 9 Portugal’s NHS hospitals.
- Patients under 18 years old with at least one dispensing registry of GH therapy were included.
- For adherence and treatment duration analysis, the sample was restricted to new patients, defined as those with a first dispensing registry at least three months after the start of each hospital database.
- To evaluate adherence the continuous measure of adherence (CMA) was calculated. (4) (Table 1) Those patients with a CMA > 80% were considered adherent. (5)
- Treatment duration was estimated using Kaplan-Meier survival curves.

Table 1. Adherence to therapy outcomes.

Outcome	Definition	Formula
CMA	Percentage of days with medication over the therapy period	$\frac{\text{Number of days with medication within dispensing dates}}{\text{Number of days within dispensing dates}}$

CMA, continuous measure of adherence.

RESULTS:

- The study identified an overall of 1,272 patients, from which 738 were defined as new patients. On average, those patients started treatment at 10.5 years of age. More details are described in Table 2.
- The average treatment duration was 5 years, with 53% of patients being considered adherent. However, those not achieving the 80% landmark also had a relatively high CMA, with the populational average achieving 79%.

- CMA increased over the years, being 71% after 1 year of treatment, 79% after 3 years and 93% after 9 years (Table 3).
- Kaplan Meier analysis curve showed that 11% of patients will remain on treatment for 9 years (Figure 1).

Table 2. Patients’ characterization.

Patients on therapy with GH, n	1,272
Male patients, n (%)	787 (62%)
Age at the 1 st dispensed prescription registry, mean ± SD, years	10.5 ± 4.0
Female	10.0 ± 3.7
Male	10.8 ± 4.1
Age at the last dispensed prescription registry, mean ± SD, years	13.7 ± 3.9
Female	13.2 ± 3.5
Male	14.0 ± 4.1

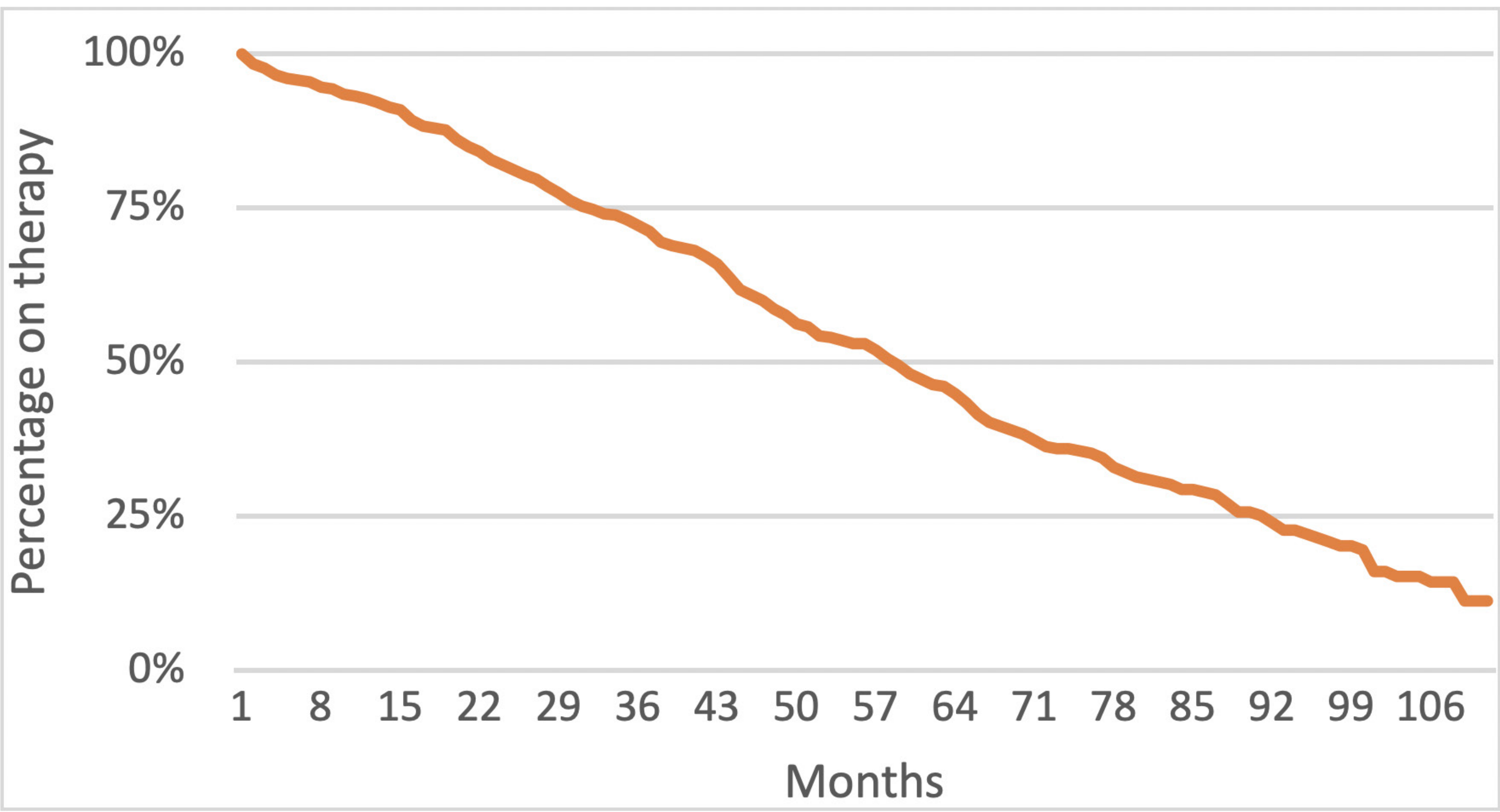
GH, growth hormone; IQR, range interquartile; SD, standard deviation

Table 3. Refill adherence (CMA).

Years on therapy	CMA (%)
< 1	74
1	71
2	74
3	79
4	84
5	79
6	81
7	78
8	81
9	93

CMA, continuous measure of adherence.

Figure 1. KM curve of time on treatment, patients de novo (n=738).



KM, Kaplan-Meier.

CONCLUSIONS:

- This study provided real-world adherence to rGH therapy estimate in a Portuguese paediatric population.
- About half of the children presented a good adherence to treatment.
- Adherence increased with therapy duration, attaining 93% after 9 years of treatment.

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