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A 5-year follow-up of adults, with childhood-onset GH deficiency, treated with GENOTONORM® in France

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Disclosure Statement

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BACKGROUND

Growth hormone deficiency (GHD) is a rare condition caused mostly by pituitary disorders. Young adult patients with childhood-onset GHD whose growth hormone replacement therapy (GHRT) is discontinued exhibit negative metabolic and physiological effects, reversible through GHRT. Therefore, continuation of GH therapy without interruption is recommended for adolescents transitioning to adulthood by guidelines of the American Association of Clinical Endocrinologists and The Endocrine Society [1,2]. In GHD adults, benefits of GHRT have been shown in body composition, bone health, cardiovascular risk factors, and quality of life [2].

The aim of the present study was to report the characteristics and 5-year GHRT in adults with childhood onset GHD.

1. *Cook DM et al. Endocr Pract 2009;15 Suppl 2:1-29.*
2. *Molitch ME et al. J Clin Endocrinol Metab 2011;96:1587-609.*

PATIENTS AND METHODS

KIMS is a large international pharmacoepidemiological registry monitoring long-term safety and clinical outcomes of GHRT (Genotropin®) in hypopituitary adults with GHD.

In France, KIMS was initiated in March 2003 and is open to:

- all adults receiving Genotropin®
- not treated patients, either because reimbursement was denied by the French health insurance system, the patient refused GHRT or stopped it.

KIMS was conducted in all French centers with at least one adult treated with Genotropin®.

All patients had to provide written informed consent for recruitment in the study.

Data were collected through case report forms until December 2010 and data entry has been web-based since. Data were monitored at the country level, and in the Stockholm centralized database.

All recorded study data were collected as part of patient routine clinical care. Timing of visits, Genotropin® dose and dose titration were at each treating physician/investigator's discretion.

We report here the analysis of the 5-year follow-up of the subgroup of adults with childhood-onset GHD included between March 2003 and October 2006 in KIMS, in France.

The analysis is descriptive, median and inter quartile range are displayed.

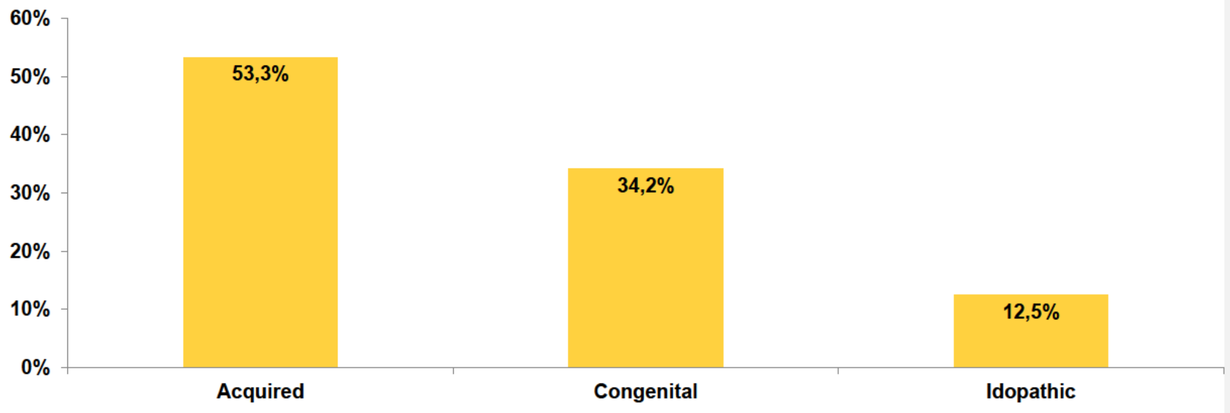
RESULTS

Overall 120 adults with childhood-onset GHD were included, by 80 centers.

Upon inclusion in KIMS

Slightly more patients were males [64 (53.3%) males]. Median age at inclusion in KIMS was 22 years (19 years; 30 years). All patients had severe GHD, associated with one or more other pituitary hormone deficiency in 104 (87%) patients.

Fig 1. Etiology of GHD



A pituitary tumor had been diagnosed in 56 (87.5%) patients out of the 64 patients with acquired GHD.

Median age at diagnosis of the pituitary disease was 7 years (5 years; 11 years) and median age at diagnosis of GHD was 12 years (6 years; 19 years).

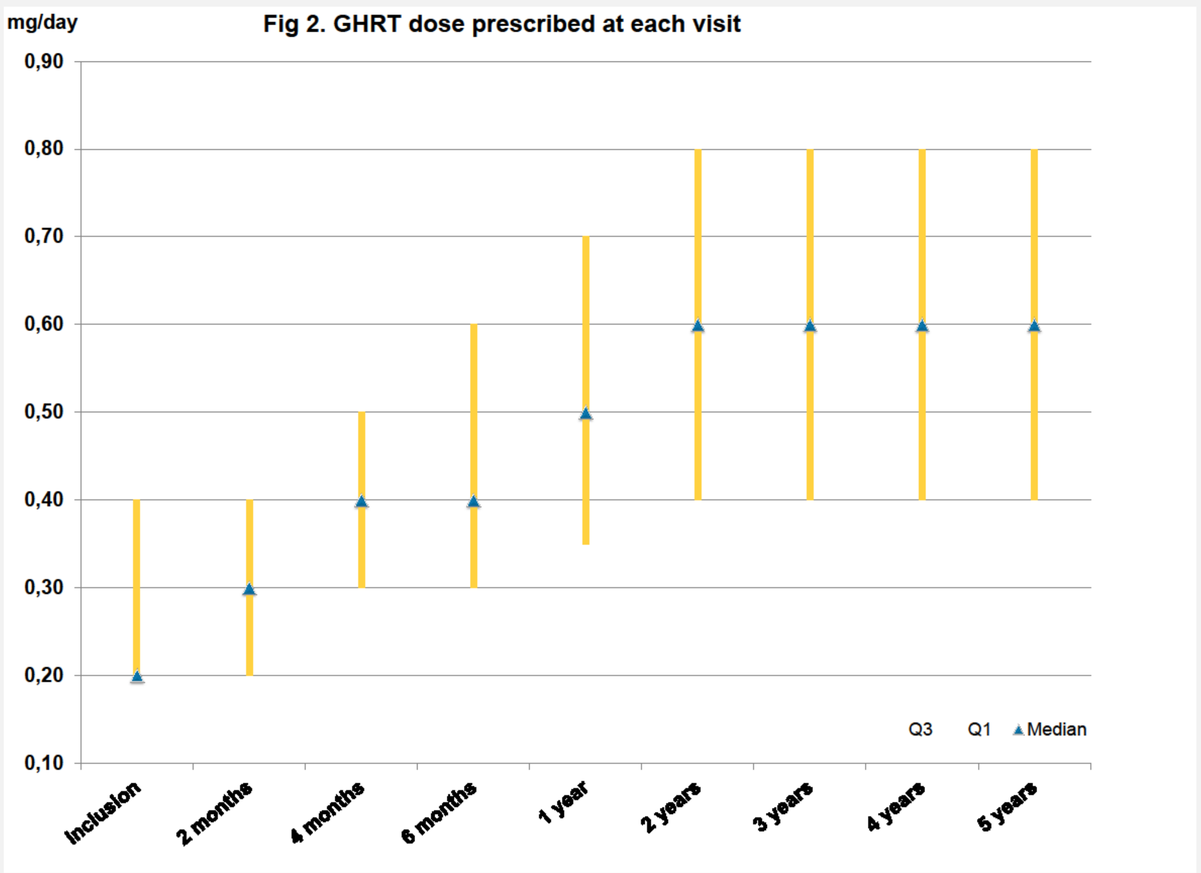
Median GHRT dose prescribed at inclusion, was 0.20 mg/day (0.20 mg/day; 0.40 mg/day). In the 95 (79%) patients, where inclusion in KIMS corresponded to adult GHRT initiation, the median Genotropin® starting dose was 0.20 mg/day (0.20 mg/day; 0.30 mg/day).

Follow-up

Overall, 23 (19%) patients were lost to follow-up.

The percentage of patients treated with GHRT decreased over time especially after 2 years and was 77% at 5 years. In 29 (24%) patients, GHRT was permanently discontinued and, at the request of the patient, in 17 (59%) cases. The prescribed GHRT dose increased up to 2 years (Fig. 2).

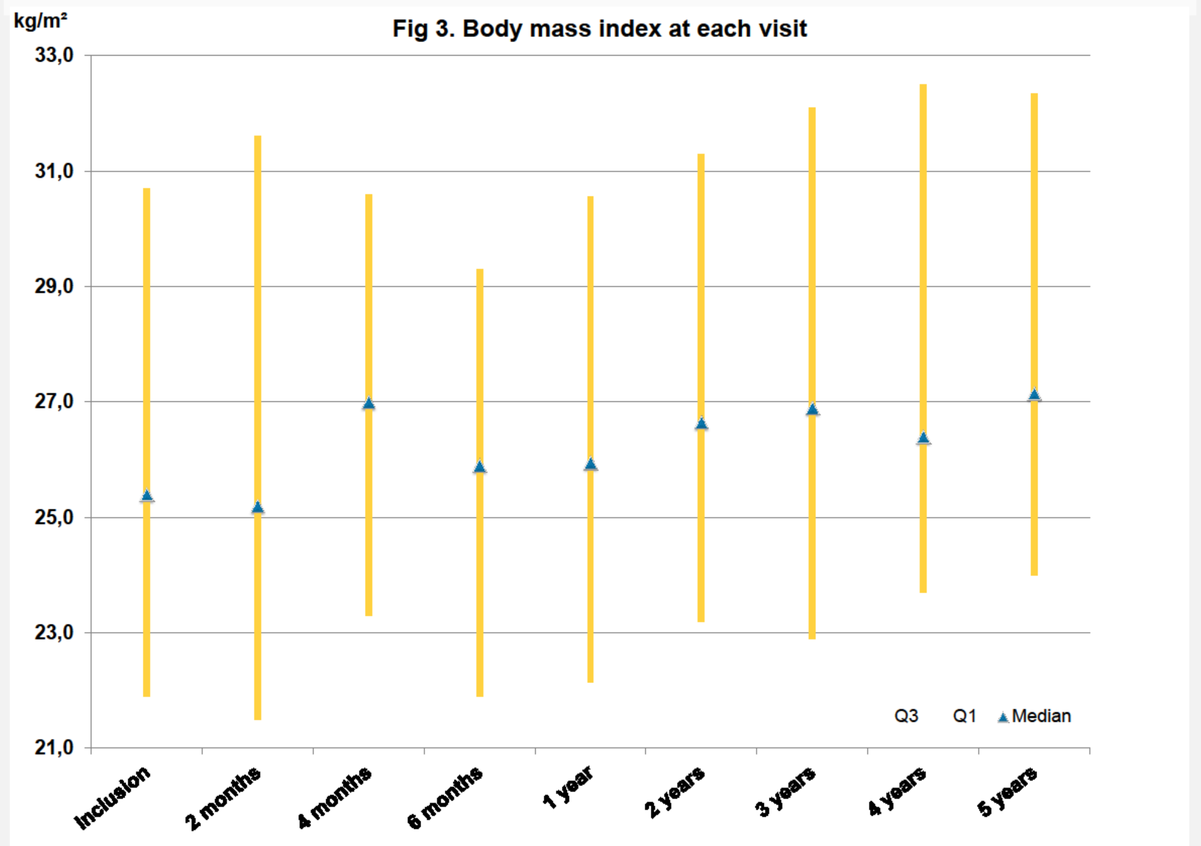
Fig 2. GHRT dose prescribed at each visit



Serum IGF-1 level was available, at each visit, in more than 80% of the patients, except at 5 years where IGF-1 level was available in 70% of the patients. Median serum IGF-1 level was 80 µg/L (40; 164) at inclusion, 199 µg/L (116; 306) after 1 year and 172 µg/L (78; 220) after 5 years.

Body mass index increased from 25.4 kg/m² (21.9;30.7) at inclusion up to 27.2 kg/m² (24.0;32.4) after 5 years (Fig.3).

Fig 3. Body mass index at each visit



Tumor size monitoring and tolerance

The percentage of patients with a Magnetic Resonance Imaging available at the visit tended to decrease over time, down to 5.1% at 5 years. Increase in pituitary tumor size was reported, 26 months after inclusion, in one female.

No new safety concern was reported.

CONCLUSION

The relatively weak prescribed doses may preclude highlighting long-term benefit of GHRT.

The authors thank all physicians and all patients for their participation in the study

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