Introduction

- Rambunctious human growth hormone (GH), somatropin has been used for many years to treat growth disorders in children, but concerns remain about its long-term safety.
- Omnitrope® is a r-hGH approved by the European Medicines Agency in 2000, with approval based on the results of multiple clinical trials, safety and efficacy to the reference product (Somatropin®, Pfizer®).
- Omnitrope® has been approved in the following indications:
  - growth hormone deficiency (GHD) - Turner syndrome (TS)
  - chronic renal insufficiency (CR)
  - born small for gestational age (SGA)
  - Poor PK/SWD syndrome (PWSD)

Objectives

- The main objective of PATRO Children is to assess the long-term safety of Omnitrope®, particularly in terms of the diastolic blood pressure of normotensive children, the risk of malignancies and potential risks of GHD in children with HVS.
- The long-term efficacy of Omnitrope® is analyzed as a secondary objective through changes in height parameters.
- Here, we present an interim analysis of safety and efficacy data up to 3 years after the start of treatment with Omnitrope®.

Methods

- History of patients, piscine, longitudinal, non-interventional study, currently being conducted across 14 different countries (Study P001011).
- In brief, infants, children and adolescents who require GH treatment and receive at least 60% of the dose of Omnitrope® are enrolled. Patients who have been previously treated with another somatropin product can also be included.
- Omnitrope® is administered as part of usual clinical practice in the centers involved and doses are given according to country-specific prescribing information. All patient data are captured in an electronic case report form.

Safety assessments

- All adverse events are recorded at each visit for the complete duration of the study period.
- Efficacy assessments: All adverse events are recorded at each visit for the complete duration of the study period.
- The local data of all patients are presented in Table 1.
- The mean age for the total population is 9.2 years and there were slightly more males (58.1%) than females (41.9%) patients included.
- In total, 275 patients (17.0% had been previously treated with another r-hGH before study entry and were randomized to Omnitrope® (Table 1).
- The mean (SD) study duration of Omnitrope® was 26.6 (21.0) months.
- The mean (SD) daily dose of Omnitrope® was 0.054 (0.016) μg/kg/day.

Safety

- A total of 1278 patients have been included. The most common reasons for patients discontinuing treatment was reaching final height and bone maturation (27.4%), with very few (2.9%) discontinuing due to AEs (Table 2).
- The reason for discontinuation was unknown or not documented in 11.4% of patients.
- Overall, 1475 patients (33.5%) experienced AEs, most of which were mild to moderate in intensity (Table 3).
- GH treatment was initiated in 159 patients (2.1%) and the GH dose was reduced in 28 patients (0.5%).
- In total, 191 patients (4.0%) have reported AEs, which have been noted by the study (2.0%)

Results and treatment

- To date, 4379 patients have been enrolled; all patients have been included in the safety set.
- The base characteristics of all patients are presented in Table 1. The mean age for the total population is 9.2 years and there were slightly more males (58.1%) than females (41.9%) patients included.
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Table 1: Patient characteristics

<table>
<thead>
<tr>
<th>Indication</th>
<th>Total (n)</th>
<th>Male/ Female (%)</th>
<th>Mean/SD age (years)</th>
<th>Mean age (years)</th>
<th>GH (μg/kg/day)</th>
<th>Mean/SD dose (μg/kg/day)</th>
<th>Mean/SD SDS (SDS)</th>
<th>Mean/SD SDS (HD)</th>
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<td>1551</td>
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Conclusions

- The results of this 3-year analysis demonstrate that Omnitrope® treatment remains effective and well tolerated in the majority of children-treated.
- Across all the indices examined, no statistically significant differences were noted compared to the previously published data for GH treatment during Omnitrope® treatment.

Acknowledgements and disclosures

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References