**INTRODUCTION AND OBJECTIVES**

Growth Hormone Deficiency (GHD) in children is characterised by low growth velocity, sometimes after a period of normal growth, and short stature relative to the child's chronological age, sex, and pubertal stage.

GHD is managed with growth hormone (GH) therapy, with the aims of achieving optimal height development and expected adult height.

Nutropin® Aq (Iqen Pharma, France) is a solution, administered daily by subcutaneous (SC) injection, containing 10 mg (30 International Units (IU)) of somatropin, a recombinant form of human growth hormone produced by recombinant DNA technology with an identical sequence of 191 amino acids to endogenous human GH of pluriplastic origin.

In the paediatric population, Nutropin® Aq is indicated for:

- Long-term treatment of children with GHD.
- Treatment of patients with chronic renal insufficiency.
- Treatment of patients with other chronic conditions associated with GH deficiency.
- Treatment of paediatric patients with growth failure associated with chronic renal insufficiency, Turner’s syndrome, and chronic liver disease.
- Treatment of adult patients with GHD.
- Treatment of patients with GH deficiency associated with Turner’s syndrome.
- Treatment of patients with other chronic conditions associated with GH deficiency.

The iNCGS (International Cooperative Growth Study) – an international, open-label, non-interventional study conducted in Europe – was initiated in 2005 to collect long-term safety and effectiveness data regarding Nutropin® Aq during treatment of paediatric indications (NCT00457572).

Here we report year 1 effectiveness and safety data from the iNCGS registry.

**METHODS**

- **Patient selection**: Data were selected from iNCGS participating centres in seven European countries (Germany, France, Spain, Italy, UK, Austria, and Romania) between October 2005 and December 2009.
- **Children with growth disorders**: For which GH therapy was initiated, and who were initiated into the study and treated for at least 56 weeks and up to 36 months.
- **The iNCGS**: Was initiated in 2005 to collect long-term safety and effectiveness data regarding Nutropin® Aq during treatment of paediatric indications.

**RESULTS**

- A total of 3,493 patients were screened for the study, 684 were not enrolled due to lack of patient consent or non-availability of data.
- Patient populations and disposition: Of 3,277 patients who were enrolled, 2,752 patients were who were fully informed about the study, gave written informed consent to participate, and had data available (Figure 2).
- Registry population: 714 patients who were enrolled, completed at least one follow-up visit and received at least one Nutropin® Aq injection.
- Safety population: 1,413 patients who received at least one Nutropin® Aq injection and with at least one follow-up visit, or follow-up safety data.

**Statistical analysis**

- Statistical analysis was performed using Statistical Analysis System (SAS®) Version 9.4 (SAS Institute Incorporated, Cary, NC, USA).
- Descriptive statistics of patients characteristics, dose and duration of exposure were evaluated.
- Height and weight velocity at one year were compared using ANOVA.
- Differences between GH treatments were evaluated by ANOVA. Differences were considered statistically significant if the p-value was <0.05.

- **Effectiveness parameters at 1 year**: Height at baseline, height velocity at one year, and height velocity change from baseline.
- **Safety parameters**: Adverse events (TEAEs), serious TEAEs, serious adverse drug reactions (SADR), and their relationship with study treatment.

**Conclusions**

- After 1 year of treatment with Nutropin® Aq, the mean height SDS as measured by the mean height SDS improved in patients with GHD and was well tolerated.
- The median duration of treatment was 3.2 years. Duration of treatment by visits (Per routine clinical practice)

**Figure 1. Study scheme for iNCGS**

- **Figure 2. Patient disposition for enrolled population in iNCGS**

**Figure 3. Changes in height SDS at 1 year by aetiology**

- **Table 1. Baseline characteristics – enrolled population**

**Figure 4. Height velocity at baseline and at 1 year by aetiology**

- **Table 2. Exposure to Nutropin® Aq – enrolled population**

**Figure 5. Change in height SDS at 1 year by aetiology**

- **Figure 6. Height velocity at one year by aetiology:**
  - a) treatment-naïve patients b) treatment-naïve prepubertal patients

**Figure 7. Effectiveness at one year by baseline height SDS:**

- a) change from baseline in height SDS, b) height velocity

**Error bars represent a Standard Deviation**

- **Safety**
  - A total of 610 patients (17.5%) in the safety population had at least one non-serious TEAE related to therapy.
  - The most frequent non-serious TEAEs were infections and infestations, which were reported in 241 patients (7.0%).
  - 205 patients (5.9%) experienced at least one serious TEAE, and 30 of these TEAEs in 27 patients (0.8%) were considered to be related to Nutropin® Aq.
  - Serious treatment-related TEAEs in 3 patients were: a) chronic renal failure and connective tissue disorder, b) 10 events in 9 patients (0.3%), c) infections and infestations, d) 2 events in 2 patients (0.1%).
  - 14 of these patients had a prior history of nephropathy.
  - AKI leading to death occurred in seven patients during the study, all events were considered by the investigators as not related to study treatment.

**References**


**Disclosures**

- All investigators participated on advisory boards for and/or have received consulting fees from Ipsen, Pfizer, Novo Nordisk and Sanofi. A research investigator for Ipsen, Sarolea and Pfizer, and a speaker for Lilly and Sanofi. Kellum has received consulting fees from Ipsen, has been a research investigator for Bayer, Lilly, and Merck and a speaker for Lilly, Merck, Pfizer, and Sanofi. Kellum has been a research investigator for Sanofi, and a speaker for Sanofi. The senior author is a research investigator for Sanofi and Pfizer, and has participated in corporate-sponsored research for Pfizer and Sanofi. Kellum is a research investigator for Bayer and Pfizer. He has consulted on behalf of Sanofi and Pfizer. Kellum has received consulting fees from Sanofi, and Pfizer. Iakova has consulted on behalf of Sanofi and Pfizer. Kellum has received consulting fees from Sanofi and Pfizer. Iakova has consulted on behalf of Sanofi and Pfizer. Kellum has received consulting fees from Sanofi and Pfizer.

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**Poster title:**

- Nutropin® Aq® and Other Growth Hormone Products: Treatment-naïve Prepubertal Patients with Growth Failure of Unknown Etiology

**This poster was presented at**

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