GROWTH HORMONE REPLACEMENT IN ADULTS

Real-world data from the ANSWER* (US) and NordiNet® IOS† (Europe) registries of 3,180 adults with GH deficiency treated with GH replacement therapy accumulated over 10 years (2002–2016)¹

- Supports the long-term effectiveness and safety of GH replacement, as prescribed in clinical practice.
- Long-term GH replacement is associated with improved body composition.
OBJECTIVE
Assess the effectiveness and safety of GH replacement therapy in real-world clinical practice.

MATERIALS AND METHODS

1. STUDY DESIGN
Two multicentre, longitudinal, non-interventional, observational cohort registries (ANSWER* and NordiNet® IOS†) collected data on GH replacement in adults. GH was prescribed according to routine practice and local regulations.

2. PARTICIPANTS

- Adults ≥18 years old;
- Diagnosis of GH deficiency;
- GH-naïve or non-naïve;
- GH therapy initiated after 18 years of age.

3. MAIN OUTCOME MEASURES

- **Effectiveness endpoints:** Change from baseline in waist circumference, BMI and bioimpedance.
- **Safety endpoints:** Treatment-related ARs, IGF-I SDS, GH exposure and non-treatment-related AEs.

RESULTS

1. PATIENT DISPOSITION AND CHARACTERISTICS

- 3,180 eligible adults with GH deficiency.‡§
- Baseline characteristics were typical of patient population. Obesity was the most prominent characteristic.
- Mean duration of GH exposure:
  - ANSWER: 3.7 years (22%), 3.7 years (24%), 5.7 years (24%), 5.2 years (1%)
  - NordiNet® IOS: 5.7 years (54%), 5.2 years (8%), 5.1 years (8%), 3.2 years (32%)

2. EFFECTIVENESS

- Mean change from baseline:
  - Waist circumference: 2.8 cm (n=403, SD: 6.38)
  - Bioimpedance: -17.4 ohm (n=239, SD: 59.19)

3. SAFETY

- Total duration of treatment exposure: 14,704 PYE (in 3,180 patients)§
- Safety profile comparable to previously reported data. No new safety signals were observed.

CONCLUSIONS

- Real-world observations accumulated from over 10 years of follow-up support the long-term effectiveness and safety of GH replacement, as prescribed in clinical practice.
- Long-term GH replacement is associated with improved body composition.
  - Effectiveness was consistent with expected body composition trends.
  - Decreases in waist circumference and bioimpedance observed.
  - Change in bioimpedance associated with dose, follow-up duration and sex.

*The American Norditropin Studies: Web-Enabled Research Program (ANSWER; NCT01009905).2
†NordiNet® International Outcome Study (NordiNet® IOS; NCT00960128).3
‡3,506 patients with GH deficiency and other diagnoses enrolled in total (NordiNet® IOS: 2,524 patients; ANSWER: 982 patients).
§FAS of this study comprised 3,180 patients from NordiNet® IOS and ANSWER combined (2,321 and 859 patients, respectively).
EAS comprised 971 patients from NordiNet® IOS and 304 from ANSWER.
ADDITIONAL SAFETY RESULTS
• The most frequent ARs were oedema, arthralgia, joint stiffness, myalgia and headache.
• ANSWER: 3 deaths (none were likely to be GH-related).
• NordiNet® IOS: 14 deaths; 12 unlikely to have been GH-related, two were possibly GH-related (one anaplastic astrocytoma, one colon cancer).

LIMITATIONS
• Some aspects of clinical practice changed over the course of follow-up (2002–2016) and differed between regions.
• Many known benefits of GH therapy were not captured by the study design.
• Interpretation of results should consider reporting bias and treatment adherence.

ABBREVIATIONS
AE, adverse event; AR, adverse reaction; BMI, body mass index; EAS, effectiveness analysis set; FAS, full analysis set; GH, growth hormone; HCP, healthcare provider; IGF-I SDS, insulin growth factor-I standard deviation score; PYE, person-years of exposure; SD, standard deviation.

REFERENCES

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