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 Pfizer Registry of Outcomes in Growth hormone RESearch (PROGRES): A multi-country, non-interventional, prospective, cohort study of patients receiving human growth hormone treatments under routine clinical care

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Background: Children with growth hormone deficiency (GHD) are treated with recombinant human growth hormone (rhGH), usually administered as a daily subcutaneous injection. Long-acting hGH (LAGH) treatments (approved and in development) have the potential to improve adherence and treatment outcomes. Somatrogon is a LAGH currently being developed as a once-weekly injectable treatment for GHD. The Pfizer Registry of Outcomes in Growth hormone REResearch (PROGRES) study was initiated to assess the long-term safety and effectiveness of Genotropin and other hGH formulations to treat GHD under routine clinical care.

Goals & aims: The overall goal of the registry is to increase our understanding of hGH treatments as used in a real-world setting. Enrolling children treated with different daily hGH brands as well as somatrogon (if approved in a given geography) will enable comparison between daily and LAGH, and across daily hGH brands. The primary objectives of the study are to describe and compare the safety and effectiveness of daily and LAGH treatments in children. Secondary objectives include evaluating adherence to hGH treatments and the health-related quality of life (HRQoL) and treatment experience of patients receiving hGH treatments.

Methods: For this non-interventional, prospective, phase 4 cohort study, eligible patients (male or female at any age) from >20 countries are planned for inclusion, with the aim of targeting 667 patients across the daily hGH brands in each of the three geographic regions. Patients will be enrolled from September 2021 to October 2029, with data collection planned until October 2030. Study inclusion criteria include prescription of daily Genotropin or other approved hGH treatments for GHD and the provision of informed consent/assent. If somatrogon is approved by regulatory agencies, somatrogontreated patients will also be eligible for inclusion. Patients participating in any interventional clinical trials at the time of enrollment will be excluded from the study. Patients will be followed until the end of the study, withdrawal of consent, death, switch to an excluded hGH, or loss to follow-up, whichever occurs first. In addition to demographic and clinical characteristics, information on hGH treatment brand, dose and start/end dates will be collected, along with reasons for switching or discontinuing treatment. Primary safety outcomes include adverse events (AEs), serious AEs, and AEs of special interest. Primary effectiveness outcomes include annual height velocity (HV) and change in HV standard deviation scores. Treatment adherence, compliance outcomes, HRQoL, and patient treatment experience will be assessed.

Conclusions: The findings from the PROGRES study will provide valuable insights into the use of somatrogon, Genotropin and other approved hGH treatments in real-world clinical practice, specifically with regard to the long-term safety and effectiveness of these treatments as well as treatment compliance and patient HRQoL.

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