Study ID 202512_GS

Name of the Principal Investigator: Guftar Shaikh
Contact details including institution/sponsor: Royal Hospital for Children, Glasgow, UK Guftar.shaikh2@nhs.scot
Coinvestigators: Evelien Gevers, London; Muhammad Yazid Jalaludin, Kuala Lumpur; Ching Chen, Glasgow; Charlotte Hoybye, Stockholm; Edna Roche, Dublin; Violeta Iotova, Varna; Ohn Nyunt, Sydney; Theresa Strong, FPWR; Jackie Lodge, PWSAUK.
Date of Approval: Jan 2026
Name of study: Assessing Clinical Hormone Intervention, Efficacy and Value in Endocrinology for PWS (ACHIEVE for PWS)
Study period Feb 2026 – Jan 2029
Summary Prader Willi Syndrome (PWS) is a rare genetic syndrome that causes poor muscle tone, developmental delay, and obesity. Growth Hormone (rhGH) therapy has been used to help individuals with PWS, in particular with growth, body composition and muscle tone. However, it is unclear whether rhGH worsens some of the co-morbidities associated with PWS, such as breathing difficulties and curvature of the spine. More long-term studies are, therefore, needed, and the current study aims to use GloBE-Reg to perform these long-term studies of safety and effectiveness of rhGH in PWS.
Inclusion criteria - Diagnosis of PWS - Age 0-25 years at the time of assessment - Eligible for or initiated on rhGH before age 16 years
Exclusion criteria No
Data to be collected for all participating cases Data will be collected both retrospectively and prospectively and will include: 1. Core Data 2. Core Data History 3. Diagnosis, Therapy, and Clinician Reported Outcome Case Report Forms: - At least one visit before the start of GH therapy - One visit every 6 months during rhGH therapy, up to 25 years of age - One visit every 6 months after treatment has stopped, if applicable, up to 25 years of age
Timepoints for Data Cut Sep 2026, Mar 2027 and then annually
Expected outputs These will be disseminated by a publication in a journal, presentation in scientific meetings and in GloBE-Reg newsletter.

Publication Plan for authorship in outputs

The investigator will adhere to the GloBE-Reg recommendations. All current and future participating centres will be invited as co- authors on all planned publications as long as they meet the ICMJE criteria. The coauthors will include those involved in study design, data provision, data extraction, data analysis and interpretation and those involved in the creation of the outputs. The support of all funders of the GloBE-Reg platform at the time the study was active will be acknowledged.

Date created: 050326
Date revised: 150626