Study ID 202509_MSE FASTEN

Name of the Principal Investigator: Meritxell Sabido Espin
Contact details including institution/sponsor: Merck KGaA, Darmstadt, Germany meritxell.sabido-espin@merckgroup.com
Coinvestigators: Jeff Muir, Merck KGaA/EMD Serono
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Name of study: Feasibility assessment of the effects of treatment with Saizen in children and adolescents with Short Stature Homeobox Gene (SHOX) deficiency, Turner Syndrome, and Noonan Syndrome (FASTEN)
Funding support: Merck KGaA
Study period Oct 2025 – Sep 2026
Summary More evidence is needed regarding the effectiveness of Saizen for SHOX deficiency, Turner Syndrome and Noonan Syndrome. To address this, the proposed study aims to collect real-world data to understand the use of Saizen for these conditions and compare its effects to other daily rhGH as well as those cases that are not on rhGH
Inclusion criteria - All those with a diagnosis of SHOX deficiency, Noonan Syndrome or Turner Syndrome - And, who receive any preparation of daily rhGH - Or, who are not receiving rhGH therapy - Aged 2-17 years at the time of the clinical encounter
Exclusion criteria – Patients receiving long-acting rhGH
Data requirement for all participating cases: Core Data Core Data History GloBE-Reg rhGH Minimum Dataset (retrospective and/or prospective) - Diagnosis Case Report Form (CRF) - Therapy CRF – One visit per 6-month period while on daily rhGH until final adult height is achieved or until the end of the study period, whichever is first. For participants not receiving rhGH, one visit per 6 month period to collect information on other hormone therapies - Clinician Reported Outcome CRF – One visit prior to rhGH initiation, one visit at rhGH start, with 6-monthly visits while on rhGH until final adult height is achieved or by the age of 17 years, whichever is first. For participants not receiving rhGH, one visit per 6 month period until final adult height is achieved or by the age of 17 years, whichever is first.

Data cut time points Data cuts shall be performed in March 2026 and September 2026
Expected outputs The data will be used to satisfy regulatory requirements and also as part of a publication in a peer- reviewed journal and presentation at scientific conferences.
Publication plan for authorship in outputs Merck KGaA intends to publish the results of this study in a peer-reviewed journal. As part of the collaboration between Merck and Globe-Reg, any Globe-Reg contributors, including those at participating centres, that fulfil the ICMJE recommendations for authorship, would be included as co-authors on any manuscripts and/or abstracts/posters summarizing data from this study.