Design and Rationale for the Height Trial, a Phase 3 Transcon GH Study in Children with Growth Hormone Deficiency

Number: SUN 464
Category: Neuroendocrinology and Pituitary

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Body

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Background: TransCon Growth Hormone (GH) is a novel sustained-release recombinant human GH (somatropin) prodrug in development for children with growth hormone deficiency (GHD) intended to provide comparable efficacy, safety, tolerability, and immunogenicity to daily GH and enhanced compliance/adherence with once-weekly dosing.
Objective: Based on results from a Phase 2 trial, which demonstrated comparable safety, efficacy, immunogenicity, and tolerability of once-weekly TransCon GH to daily GH therapy in children with GHD, a Phase 3 trial has been initiated.

Design and Methods: A global Phase 3 randomized open-label active-controlled parallel-group trial will be conducted investigating the safety, tolerability, and efficacy of weekly TransCon GH versus standard daily GH over 52 weeks in prepubertal children with GHD. Approximately 100 sites in 20 countries in North America, Europe, the Middle East, North Africa, and Oceania have been selected with plans to enroll approximately 150 treatment-naïve prepubertal children with GHD. Subjects will be randomized in a 2:1 ratio and receive either once-weekly TransCon GH 0.24 mg/kg/week or dose equivalent once-daily somatropin for 52 weeks. Endpoints include efficacy (height SDS, annualized height velocity with a non-inferiority margin of 2.0 cm, and changes in IGF-1), safety (adverse events, local tolerability, incidence of anti-GH antibodies), pharmacokinetics (serum GH), and pharmacodynamics (insulin-like growth factor-1, IGF-1).

Comments: The Phase 2 trial demonstrated safety and efficacy of TransCon GH 0.14, 0.21, and 0.30 mg/kg/week. In the Phase 3 trial, TransCon GH 0.24 mg/kg/week will be used to accommodate global dosing practices. The length of the trial is 12 months to qualify as a pivotal trial. Following completion of the trial, subjects will be invited to participate in an extension trial to evaluate long-term safety and efficacy. During the extension trial, subjects will be switched from TransCon GH in vials to an auto-injector.

Nothing to Disclose: MB, DBK, EM, JM, EDC, JAL

Sessions

SUN 422-465 Acromegaly and GH

Sunday, Apr 02 1:00 PM

OCCC - West Hall B (EXPO Hall)

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