Six-Month Interim Safety and Efficacy of Different Dose Levels of TransCon hGH Administered Once-weekly Versus Standard Daily Human Growth Hormone Replacement Therapy in Pre-Pubertal Children with Growth Hormone Deficiency (GHD)

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Background

TransCon hGH is a long-acting prodrug of recombinant human Growth Hormone (hGH) that releases fully active unmodified hGH into the blood compartment. TransCon hGH was shown in two Phase 1 studies in Healthy Volunteers and a Phase 2 study in adults with GH Deficiency (AGHD) to:

1) Be safe and well tolerated,
2) Produce dose dependent, predictable levels of growth hormone,
3) Be suitable for a once-weekly dosing regimen,
4) Provide a pharmacokinetic (PK) hGH and IGF-I pharmacodynamic (PD) response comparable to daily hGH throughout the dosing period.

This interim analysis consists of 25 patients (approximately 50 % of the total enrollment in the study) completing all six months of treatment, and demonstrates that TransCon hGH has a safety and efficacy profile comparable to daily hGH.

Results: Growth

At 6 months (Figure 2B), mean annualized height velocities among the three dosing levels administered weekly ranged from 11.9 cm for the 0.14 mg/kg/week dose to 14.5 cm for the 0.30 mg/kg/week dose, which were comparable to 11.5 cm for the active comparator, daily injections of Genotropin® at a 0.21 mg/kg/week dose.

Results: PK/PD

Maximum hGH blood concentration is comparable between equivalent weekly doses of TransCon Growth Hormone and daily hGH (Figure 4); and a dose-proportional increase in IGF-I levels (SDS) into the normal range (Figure 5) was observed following dosing of the three TransCon Growth Hormone dose levels.

Conclusion

To date, TransCon hGH has demonstrated efficacy and safety comparable to that observed with daily hGH. Injection site reactions have generally been mild and similar to what is expected with daily hGH injections, with no nodule formation or lipatrophy noted. This TransCon hGH Phase 2 study conducted in a pediatric population confirms the excellent safety and efficacy profile observed in previous clinical trials.