PRESS RELEASE

Ascendis Pharma A/S Announces Positive Interim Results from a Phase 2 Pediatric Study of Once-Weekly TransCon Growth Hormone for the Treatment of Growth Hormone Deficiency

Interim analysis shows annualized three month height velocity is comparable to the active comparator, Genotropin®, given at an equivalent cumulative weekly dose

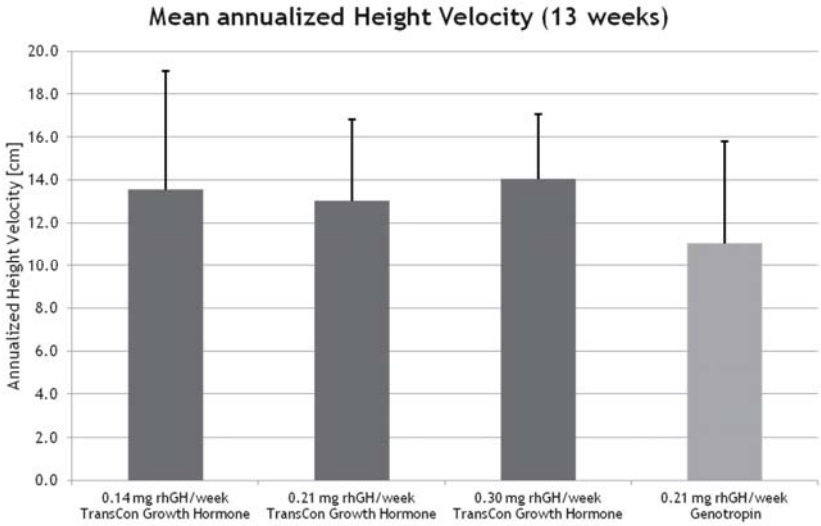
Copenhagen, Denmark, September 16, 2014 /PR Newswire/ -- Ascendis Pharma A/S, a biotechnology company that applies its innovative TransCon technology to address significant unmet medical needs, today announced positive interim results from its ongoing Phase 2 pediatric study to evaluate once-weekly TransCon Growth Hormone in children with growth hormone deficiency, or GHD. The full interim results will be presented at the 7th International Congress of the GRS and IGF Society, being held October 15-18, 2014, in Singapore.

"It has been demonstrated that patients who do not comply with the daily injection regimen of currently available growth hormone therapies suffer from suboptimal treatment outcomes," commented Paul Saenger, M.D., Professor Emeritus of Pediatrics, Albert Einstein College of Medicine. Dr. Saenger continued, "The interim results from the Phase 2 pediatric study suggest that TransCon Growth Hormone has thus far demonstrated an impressive safety and efficacy profile comparable to that of existing daily growth hormone therapies, and may enable once-weekly dosing to improve patient compliance and treatment outcomes."

Michael Beckert, M.D., Acting Chief Medical Officer of Ascendis Pharma, stated, "Following decades of collective industry effort, these interim data suggest that we may finally be able to provide patients with a long-acting growth hormone that has the efficacy and safety of daily growth hormone, but with a more convenient dosing regimen."

This interim analysis consists of 25 patients, which represents approximately 50% of the anticipated total enrollment in the study, completing three months of the total six months of treatment. Key conclusions from the interim analysis include:

- mean annualized height velocities ranged from 13.0 cm to 14.1 cm for the three weekly dose levels of TransCon Growth Hormone, which were comparable to the active comparator, daily injections of Genotropin®;
- there have been no reports of serious or unexpected adverse events;
- injection site reactions were generally mild and transient and occurred in only a few patients;
- thus far, there have been no observations of injection site nodule formation or lipoatrophy; and
- a dose-proportional increase in IGF-I levels was observed following dosing of the three TransCon Growth Hormone doses. Transient point values of IGF-I SDS > +2 have been observed in a small number of patients and only in the high-dose treatment arm.



The ongoing Phase 2 pediatric study is being conducted to investigate the safety, tolerability, pharmacokinetics, pharmacodynamics and efficacy of TransCon Growth Hormone in up to 52 treatment-naïve pre-pubertal patients with GHD, who meet internationally recognized criteria for GHD. The study is a 6-month multi-center, randomized study comparing three dose levels of TransCon Growth Hormone (0.14; 0.21; and 0.30 mg hGH/kg/week), administered once per week, to the active control Genotropin (0.21 mg hGH/kg/week), administered as 7 daily injections. The efficacy endpoint is height velocity at six months. The protocol for the Phase 2 pediatric study calls for two interim data analyses. This interim analysis reports height velocity in 50% of the evaluable patients completing the first three months of the study. In the first quarter of 2015, Ascendis Pharma plans to report six-month height velocity data from 50% of the patients completing the Phase 2 pediatric study, and top-line data for all patients in the study in mid-2015.

About Growth Hormone Deficiency

Growth hormone deficiency, or GHD, is a serious orphan disease affecting both children and adults. In children, GHD manifests with short stature, metabolic abnormalities, cognitive deficiencies and poor quality of life. Adult GHD is associated with premature mortality and neuropsychiatric-cognitive, cardiovascular, neuromuscular, metabolic and skeletal abnormalities. The market for daily injections of human growth hormone was approximately $3 billion in 2013. There are currently no long-acting growth hormone treatment options available in the United States or Europe.

The current standard of care for the treatment of GHD requires patients to receive daily injections over many years. The administrative burden of daily injections often results in poor patient compliance and can lead to suboptimal treatment outcomes.

About TransCon Growth Hormone

Ascendis Pharma is developing once-weekly TransCon Growth Hormone, an investigational new drug, to address the burden of daily injections and suboptimal treatment outcomes that can result from poor patient compliance. TransCon Growth Hormone is a prodrug that releases unmodified

growth hormone, thus maintaining the same mode of action as currently prescribed daily growth hormone therapies. Clinical studies of TransCon Growth Hormone have demonstrated a comparable efficacy, safety, tolerability and immunogenic profile to that of daily growth hormone. If approved, TransCon Growth Hormone may reduce the burden of daily treatment by requiring significantly fewer injections, which may improve patient compliance and treatment outcomes. Ascendis Pharma has successfully completed a Phase 2 study of TransCon Growth Hormone in adults with GHD and is currently enrolling children with GHD in a Phase 2 pediatric study. Ascendis Pharma expects to report interim data from its Phase 2 pediatric study in the first quarter of 2015, and top-line data in mid-2015.

About Ascendis Pharma A/S
Ascendis Pharma is applying its innovative TransCon technology, which combines the benefits of prodrug and sustained release technologies, to develop a pipeline of best-in-class therapeutics that address significant unmet medical needs. The TransCon technology can be applied to existing drug therapies, including proteins, peptides and small molecules, to create prodrugs that provide for the predictable and sustained release of an unmodified parent drug.

Ascendis Pharma has a diversified and balanced pipeline. TransCon Growth Hormone is a proprietary program that has completed a Phase 2 study in adults with growth hormone deficiency, or GHD, and is currently enrolling children with GHD in a Phase 2 study. Ascendis Pharma also plans to develop TransCon Treprostinil for the treatment of pulmonary arterial hypertension. In addition to its proprietary programs, Ascendis Pharma has formed collaborations focused on leading products in large markets that are of strategic importance to its collaborators. These collaborations are with Sanofi in diabetes and an undisclosed market leader in the field of ophthalmology.

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