Ascendis Pharma A/S Announces Preliminary Data from Single Arm Phase 3 fliGHt Trial Demonstrated TransCon™ hGH was Safe and Well-Tolerated in Pediatric Subjects Previously Treated with Daily Growth Hormone

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- Provides real-world experience for switching pediatric growth hormone deficiency subjects one to 17 years old from daily growth hormone to once-weekly TransCon hGH -

- Data included safety and efficacy for TransCon hGH in treatment-naïve subjects younger than three years old -

COPENHAGEN, Denmark, May 20, 2019 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND), a biopharmaceutical company that utilizes its innovative TransCon™ technologies to address unmet medical needs, today announced preliminary results from the phase 3 fliGHt Trial of TransCon Growth Hormone (hGH). The results indicated treatment with TransCon hGH was safe and well-tolerated in subjects with pediatric growth hormone deficiency (GHD) who were previously treated with commercially-available daily growth hormone therapies. The company plans to present detailed results from the fliGHt Trial at its upcoming R&D Update on Wednesday, June 26 in New York City, and at future medical conferences.

In the trial, the adverse event profile of TransCon hGH was similar to the published safety profile of daily growth hormone therapies with no drug-related discontinuations or drug-related serious adverse events, no neutralizing antibodies, and a low level of low-titer non-neutralizing antibodies. These fliGHt Trial data include new information demonstrating safety and tolerability in treatment-naïve subjects under three years of age.

Along with data from the pivotal heiGHt Trial and the enliGHten (long-term extension) Trial, these results form a safety database of approximately 300 subjects treated with TransCon hGH (approximately 300 for six months, 120 for 12 months and 45 for 24 months), consistent with input from regulatory authorities.

“With these preliminary data from the fliGHt Trial, we continue to be encouraged by the safety profile of TransCon hGH. The fliGHt data bolster our safety database and support plans for our U.S. regulatory filing in the first half of 2020,” said Jonathan Leff, M.D., Ascendis Pharma’s Chief Medical Officer. “Our goal for TransCon hGH is to help alleviate the burden of daily injections so every child has a better opportunity to achieve normal adult height and overall endocrine health. We are thankful to all those who participated in this global clinical development program to help achieve this goal.”

The phase 3, open label, 26-week single arm fliGHt Trial enrolled 143 subjects with pediatric GHD who were previously treated with commercially-available daily growth hormone therapy (average dose was 0.29 mg/kg/week reflecting typical treatment patterns in the U.S.), and three treatment-naïve subjects under three years of age. The primary objective was to evaluate safety in this population. Subjects ranged in age from one to 17 years and were administered 0.24 mg/kg/week of TransCon hGH at initiation of the trial.

As in real-world clinical practice today using daily therapies, trial investigators were able to dose titrate TransCon hGH based on clinical response and IGF-1 levels at subsequent study visits. Of the 146 subjects enrolled in the trial, 143 completed dosing and 140 rolled into the ongoing enliGHten Trial.

“The combined findings from the heiGHt and fliGHt Trials provide compelling data about the efficacy and safety of TransCon hGH in both treatment-naïve and previously treated subjects with pediatric GHD,” said Aristides K. Maniatis, M.D., FAAP, pediatric endocrinologist from Rocky Mountain Pediatric Endocrinology and an investigator in the heiGHt and fliGHt Trials. “As a clinician, it was encouraging that the pivotal heiGHt Trial data demonstrated greater annualized height velocity for TransCon hGH as compared to daily. The preliminary fliGHt Trial data also demonstrate a reassuring safety profile. I believe these two trials combined show that TransCon hGH once-weekly therapy is a potential option for both treatment-naïve and previously treated patients, and that TransCon hGH has the potential to lead a paradigm shift in treating pediatric GHD.”

The TransCon hGH phase 3 program includes the heiGHt, fliGHt and enliGHten Trials and has enrolled over 300 subjects with pediatric GHD. Top-line data for the pivotal heiGHt Trial demonstrated that TransCon hGH administered once-weekly to children with pediatric GHD had comparable safety and tolerability to daily Genotropin®, with a significantly greater annualized height velocity over the one-year study period.

Ascendis plans a clinical database lock for the TransCon hGH phase 3 program in the third quarter of 2019. Subsequently, the company intends to submit a Biologics License Application (BLA) with the U.S. Food and Drug Administration for TransCon hGH to treat pediatric GHD in the first half of 2020.

TransCon hGH is designed to deliver unmodified hGH, the same growth hormone used in daily therapies, at a predictable rate over one week. Currently in the U.S. and Europe, the only GHD treatment option for patients and their families is daily hGH injections. Patients receiving daily therapy endure thousands of injections over the course of many years, a burden that often leads to missed doses and patients who fail to meet expected outcomes.

About TransCon™ Technology

TransCon is short for “transient conjugation.” The proprietary TransCon platform is an innovative technology to create new therapies that optimize therapeutic effect, including efficacy, safety and dosing frequency. TransCon molecules have three components: an unmodified parent drug, an inert carrier that protects it, and a linker that temporarily binds the two. When bound, the carrier inactivates and shields the parent drug from clearance. When injected into the body, physiologic pH and temperature conditions initiate the release of the active, unmodified parent drug in a predictable release manner. Because the parent drug is unmodified, its original mode of action is expected to be maintained. TransCon technology can be applied broadly to a protein, peptide or small molecule in multiple therapeutic areas, and can be used systemically or locally.

About Pediatric Growth Hormone Deficiency (GHD)

Pediatric GHD is a serious orphan disease caused when the pituitary gland does not produce enough growth hormone. Children with GHD are not only characterized by short stature, but they also experience metabolic abnormalities, psychosocial challenges, cognitive deficiencies and reduced quality of life.

For decades, the standard of care for GHD has been a daily subcutaneous injection of hGH, which improves growth and metabolic effects. For caregivers and patients, the treatment burden with daily injections is high, which leads to poor adherence and reduced overall treatment outcomes.

About Ascendis Pharma A/S

Ascendis Pharma is applying its innovative platform technology to build a leading, fully integrated biopharma company focused on making a meaningful difference in patients' lives. Guided by its core values of patients, science and passion, the company utilizes its TransCon ™ technologies to create new and potentially best-in-class therapies.

Ascendis Pharma currently has a pipeline of three independent rare disease endocrinology product candidates in clinical development and has established oncology as its second therapeutic area of focus. Additionally, Ascendis Pharma has multi-product collaborations with Sanofi in diabetes and Genentech in the field of ophthalmology and continues to expand into additional therapeutic areas for both internal and external development.

Ascendis is headquartered in Copenhagen, Denmark, with offices in Heidelberg, Germany and Palo Alto, California.
For more information, please visit www.ascendispharma.com.

Forward-Looking Statements

This press release contains forward-looking statements that involve substantial risks and uncertainties. All statements, other than statements of historical facts, included in this press release regarding our future operations, plans and objectives of management are forward-looking statements. Examples of such statements include, but are not limited to, statements relating to (i) the timing of our BLA for TransCon hGH for the treatment of pediatric GHD, (ii) the safety database and timing of the database lock for TransCon hGH, (iii) our ability to apply our platform technology to build a leading, fully integrated biopharma company, (iv) our expectations regarding our ability to create new and potentially best-in-class therapies and (v) our product pipeline. We may not actually achieve the plans, carry out the intentions or meet the expectations or projections disclosed in the forward-looking statements and you should not place undue reliance on these forward-looking statements. Actual results or events could differ materially from the plans, intentions, expectations and projections disclosed in the forward-looking statements. Various important factors could cause actual results or events to differ materially from the forward-looking statements that we make, including the following: unforeseen safety or efficacy results in our TransCon hGH, TransCon PTH and TransCon CNP or other development programs; unforeseen expenses related to the development of TransCon hGH, TransCon PTH and TransCon CNP or other development programs, general and administrative expenses, other research and development expenses and our business generally; delays in the development of TransCon hGH, TransCon PTH and TransCon CNP or other development programs related to manufacturing, regulatory requirements, speed of patient recruitment or other unforeseen delays; dependence on third party manufacturers to supply study drug for planned clinical studies and potential commercial sale, if approved; and our ability to obtain additional funding, if needed, to support our business activities. For a further description of the risks and uncertainties that could cause actual results to differ from those expressed in these forward-looking statements, as well as risks relating to our business in general, see our current and future reports filed with, or submitted to, the U.S. Securities and Exchange Commission (SEC), including our Annual Report on Form 20-F for the year ended December 31, 2018, which we filed with the SEC on April 3, 2019. Forward-looking statements do not reflect the potential impact of any future in-licensing, collaborations, acquisitions, mergers, dispositions, joint ventures, or investments we may enter into or make. We do not assume any obligation to update any forward-looking statements, except as required by law.

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