Ascendis Pharma Announces Phase 3 heiGHt Trial Demonstrated Superior Efficacy and Comparable Safety and Tolerability of TransCon™ hGH to a Daily Growth Hormone

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- Oral presentation of results in pediatric growth hormone deficiency on behalf of investigators at ENDO 2019 -

- Patient group acknowledges importance of once-weekly therapy to address overall impact of GHD -

COPENHAGEN, Denmark, March 24, 2019 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND), a biopharmaceutical company that utilizes its innovative TransCon technology to address unmet medical needs, today announced top-line results from its pivotal phase 3 heiGHt Trial were presented at the Endocrine Society's annual meeting, ENDO 2019, in New Orleans. Trial results demonstrated that TransCon Growth Hormone (hGH) administered once-weekly to children with pediatric growth hormone deficiency (GHD) had comparable safety and tolerability to daily Genotropin®, with a significantly greater increase in annualized height velocity over the one-year study period. TransCon hGH has been designed to provide sustained release of unmodified hGH, the same growth hormone used in daily therapies, at a predictable rate over one week.

The heiGHt Trial findings were presented at ENDO 2019 by investigator Paul Thornton, M.B. B.Ch., MRCPI, a pediatric endocrinologist at Cook Children's Medical Center in Fort Worth, Texas, in an oral session.

"This is exciting news for all of those living with GHD, including our families at Cook Children's, because we have shown that a single once-weekly dose of TransCon hGH was just as safe and works as well or better than daily growth hormone," said Dr. Thornton. "Now, the heiGHt Trial has shown that children with GHD can grow effectively on one shot a week."

Pediatric GHD is a serious orphan disease characterized by short stature and metabolic abnormalities that affect overall physical and mental health. In GHD, the pituitary gland does not produce sufficient growth hormone, which is important not only for height but also for optimal bone, heart, muscle and brain development. As a result, children with GHD experience psychosocial challenges and poor quality of life, including impaired sleep and difficulty concentrating.

"Children have a short time to grow and a lifetime to live, which is why it is so important to help those with GHD have the best chance possible of growing up to achieve normal adult height and experience both good physical and mental health given the substantial psychosocial impact of the disease," said Mary Andrews, Chief Executive Officer and co-founder of the MAGIC Foundation, the global leader in endocrine health, advocacy, education, and support. "Children with GHD and their families have waited years for a long-acting growth hormone therapy that could ultimately reduce the number of injections needed to help these children thrive. We are grateful for Ascendis' commitment to developing this new treatment option for pediatric GHD and making it a reality for patients and their families in the coming years."

Currently, in the United States and Europe, the only treatment option for pediatric GHD is a daily subcutaneous injection of hGH. While daily injections of hGH improve growth and metabolic effects, they can be associated with a high treatment burden. Patients receiving daily therapy may endure thousands of injections over the course of many years, which can lead to poor adherence and reduce overall treatment outcomes.

About the heiGHt Trial

The heiGHt Trial evaluated 161 treatment-naïve children with GHD randomized in a 2:1 ratio to receive either once-weekly TransCon hGH (0.24 mg/kg/week subcutaneously) or daily Genotropin (34 µg/kg/day or 0.24 mg/kg/week subcutaneously) for 52 weeks. Top-line results showed that once-weekly TransCon hGH was superior to once-daily hGH on the primary endpoint of annualized height velocity (AHV) at 52 weeks. In the primary analysis of the intent-to-treat population using ANCOVA, TransCon hGH demonstrated an AHV of 11.2 cm/year compared to 10.3 cm/year for the daily hGH. The treatment difference was 0.86 cm/year with a 95 percent confidence interval of 0.22 to 1.50 cm/year. The AHV for TransCon hGH was significantly greater than the daily hGH (p=0.0088).

In addition, the incidence of poor responders (AHV < 8.0 cm/year) was 4 percent and 11 percent in the TransCon hGH and daily hGH arms, respectively.

In the trial, no serious adverse events related to study drug were observed in either arm. No treatment-emergent adverse events leading to discontinuation of study drug were observed in either arm. Adverse events leading to dose reduction occurred twice in the TransCon hGH arm (1.9 percent) and once in the daily hGH arm (1.8 percent). Two subjects in each treatment arm experienced mild injection site reactions that were considered adverse events. Two subjects, one from each arm, withdrew from the trial prior to the final visit.

About TransCon™ Technology

TransCon refers to "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 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) our ability to apply our platform technology to build a leading, fully integrated biopharma company, (ii) our expectations regarding our ability to create new and potentially best-in-class therapies and (iii) 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, 2017, which we filed with the SEC on March 28, 2018. 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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