Ascendis Pharma A/S Receives Positive Opinion from the Paediatric Committee of the European Medicines Agency on the Agreement of a Paediatric Investigation Plan

July 6, 2020

– Supports the investigation of TransCon hGH for treatment of growth hormone deficiency in the pediatric population from 6 months to less than 18 years of age –

– Company on track to submit European marketing application in third quarter 2020 –

– No additional studies required –

COPENHAGEN, Denmark, July 06, 2020 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND), a biopharmaceutical company that utilizes its innovative TransCon technologies to address unmet medical needs, today announced that it received a positive opinion from the Paediatric Committee (PDCO) of the European Medicines Agency (EMA) on its agreement with the proposed Paediatric Investigation Plan (PIP) for TransCon hGH (lonapegsomatropin), an investigational long-acting once-weekly prodrug of somatropin (human growth hormone or hGH) in development for treatment of growth hormone deficiency (GHD). The positive opinion is based on the non-clinical safety program, as well as data from the three phase 3 clinical trials provided to PDCO from the ongoing TransCon hGH development program, which will be included in the upcoming Marketing Authorisation Application (MAA).

The PIP opinion from PDCO endorsed the TransCon hGH program as acceptable for assessment of safety and efficacy for the use of TransCon hGH as a treatment for growth hormone deficiency in children from 6 months to less than 18 years of age, mirroring the population covered by the studies conducted in the program.

“We are delighted to receive the positive opinion from PDCO regarding our PIP, which is based on a comprehensive data package from our non-clinical and clinical development program for TransCon hGH. As planned, we will move forward with our marketing application submission in Europe this quarter,” said Dana Pizzuti, M.D., Ascendis Pharma’s Senior Vice President of Development Operations. “We believe this opinion recognizes the unique attributes of TransCon hGH and how our TransCon technology enables the long-acting release of unmodified somatropin which provides a predictable mode of action without changing the expected safety profile. We are very pleased with our PDCO discussions as the approval of our PIP reflects to our knowledge the first time PDCO has concluded that a clinical development program for a long acting growth hormone treatment is appropriate to support the clinical development in children.”

Ascendis submitted a Biologics License Application for TransCon hGH for the treatment of pediatric GHD to the U.S. Food and Drug Administration in June 2020, and the company recently conducted productive meetings with European regulatory personnel, including a pre-submission meeting with EMA and meetings with the rapporteur and co-rapporteur.

About the Paediatric Committee (PDCO)

The Paediatric Committee (PDCO) is the European Medicines Agency's (EMA) scientific committee responsible for activities on medicines for children and to support the development of such medicines in the European Union by providing scientific expertise and defining paediatric needs.

About the Paediatric Investigation Plan (PIP)

A paediatric investigation plan (PIP) is a development plan aimed at ensuring that the necessary data are obtained through studies in children, to support the authorisation of a medicine for children. All applications for marketing authorisation for new medicines have to include the results of studies as described in an agreed PIP, unless the medicine is exempt because of a deferral or waiver. This requirement also applies when a marketing-authorisation holder wants to add a new indication, pharmaceutical form or route of administration for a medicine that is already authorised and covered by intellectual property rights.

About TransCon™ Technology

TransCon refers to “transient conjugation.” The proprietary TransCon platform is an innovative technology designed 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 conditions (e.g., pH and temperature) initiate the release of the active, unmodified parent drug in a predictable manner. Because the parent drug is unmodified, its original mode of action may be maintained. TransCon technology is designed to be applied broadly to a protein, peptide or small molecule in multiple therapeutic areas, and to 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 may experience metabolic abnormalities, psychosocial challenges and poor quality of life.

For decades, the standard of care for GHD has been a daily subcutaneous injection of hGH, which improves growth and overall endocrine health.

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 endocrinology rare disease product candidates in clinical development and is advancing oncology as its second therapeutic area of focus. The company continues to expand into additional therapeutic areas to address unmet patient needs.

Ascendis is headquartered in Copenhagen, Denmark, with additional offices in Heidelberg and Berlin, Germany, and in Palo Alto and Redwood City, 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 plans to submit a MAA for TransCon hGH to the EMA in the third quarter of 2020, (ii) our ability to apply our platform technology to build a leading, fully integrated biopharma company, (iii) our expectations regarding our ability to create potentially best-in-class therapies and (iv) 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 and potential commercialization 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; our ability to obtain additional funding, if needed, to support our business activities and the effects on our business of the worldwide COVID-19 pandemic. 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 filed with the SEC on April 3, 2020. 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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