



Ascendis Pharma A/S Submits Marketing Authorisation Application (MAA) to the European Medicines Agency (EMA) for TransCon™ hGH for Treatment of Pediatric Growth Hormone Deficiency

September 8, 2020

COPENHAGEN, Denmark, Sept. 08, 2020 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND), a biopharmaceutical company that uses its novel TransCon technologies to address unmet medical needs, announced today the submission of a MAA to the EMA seeking approval for TransCon hGH (lonapegsomatropin), an investigational long-acting once-weekly prodrug of somatropin (human growth hormone or hGH), for the treatment of pediatric patients who are diagnosed with growth hormone deficiency (GHD).

"This MAA submission, which follows the approval of our Paediatric Investigation Plan (PIP) from EMA, is an important next step for extending the global reach of TransCon hGH," said Dana Pizzuti, M.D., Ascendis Pharma's Senior Vice President of Development Operations. "If approved, TransCon hGH could provide clinicians and patients with a once-weekly treatment option."

TransCon hGH is designed to release somatropin with the same mode of action and distribution as once-daily somatropin products, but with a once-weekly injection.¹ The MAA submission includes data from the clinical development program for TransCon hGH that included eight clinical trials evaluating safety and efficacy in more than 400 subjects with GHD.

A Biologics License Application seeking approval for TransCon hGH for the treatment for pediatric GHD is under review by the U.S. Food and Drug Administration (FDA), and a PDUFA date is set for June 25, 2021.

TransCon hGH is an investigational therapy that is not approved for use in any country. There is no long-acting growth hormone treatment currently approved by the EMA for the treatment of pediatric GHD. TransCon hGH has received orphan designation in both the U.S. and Europe as a treatment for GHD.

About Pediatric Growth Hormone Deficiency (GHD)²

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 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 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.

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 Ascendis' 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) Ascendis' PDUFA date of June 25, 2021 with respect to the BLA Ascendis submitted in June 2020, (ii) Ascendis' ability to apply its platform technology to build a leading, fully integrated biopharma company, (iii) Ascendis' product pipeline and expansion into additional therapeutic areas, and (iv) Ascendis' expectations regarding its ability to utilize its TransCon technologies to create new and potentially best-in-class therapies. Ascendis 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 Ascendis makes, including the following: unforeseen safety or efficacy results in its oncology programs, TransCon hGH, TransCon PTH and TransCon CNP or other development programs; unforeseen expenses related to the development and potential commercialization of its oncology programs, TransCon

hGH, TransCon PTH and TransCon CNP or other development programs, selling, general and administrative expenses, other research and development expenses and Ascendis' business generally; delays in the development of its oncology programs, 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; Ascendis' ability to obtain additional funding, if needed, to support its business activities and the effects on its 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 Ascendis' business in general, see Ascendis' prospectus supplement filed on July 9, 2020 and Ascendis' current and future reports filed with, or submitted to, the U.S. Securities and Exchange Commission (SEC), including its 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 that Ascendis may enter into or make. Ascendis does not assume any obligation to update any forward-looking statements, except as required by law.

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¹ Sprogøe K, et al. *Endocrine Connections*. 2017(6): R171-181.

² Backeljauw PF, et al. *Endocrine Disorders in Adolescents*. 2014: 292-403.



Source: Ascendis Pharma