Ascendis Pharma A/S Receives Positive CHMP Opinion for TransCon™ hGH for Patients with Pediatric Growth Hormone Deficiency

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– TransCon hGH is a once-weekly prodrug of somatropin designed to reduce the treatment burden for patients with growth hormone deficiency.

– Final European Commission decision on TransCon hGH MAA for pediatric growth hormone deficiency expected within 67 days after positive opinion.

COPENHAGEN, Denmark, Nov. 12, 2021 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND) today announced that the European Medicine Agency's Committee for Medicinal Products for Human Use (CHMP) has adopted a positive opinion recommending the granting of a marketing authorization for Lonapegsomatropin Ascendis Pharma (TransCon hGH). TransCon hGH is a long-acting once-weekly, transiently pegylated somatropin that in the body releases somatropin (also called human growth hormone, or hGH), indicated for growth failure in children and adolescents aged from 3 years up to 18 years due to insufficient endogenous growth hormone secretion (growth hormone deficiency, or GHD). The European Commission final decision on the Company's Marketing Authorisation Application (MAA) for TransCon hGH is expected within 67 days after the positive opinion, or by the end of January 2022.

"We are delighted to receive a positive CHMP opinion for once-weekly TransCon hGH," said Dana Pizzuti, M.D., Ascendis Pharma's Chief Medical Officer and Senior Vice President of Development Operations. "This milestone brings us a step closer to realizing our goal of being able to offer a once-weekly treatment of unmodified somatropin to patients in Europe."

The CHMP opinion is based on results submitted in the MAA from the Company's Phase 3 heiGHt, fliGHt and enliGHten trials, which collectively treated more than 300 pediatric patients diagnosed with GHD. In 2020, Ascendis Pharma received a positive opinion from the Paediatric Committee of the European Medicines Agency for its TransCon hGH Paediatric Investigation Plan, based on the non-clinical safety program as well as data from the three Phase 3 clinical trials included in the MAA.

"We are honored that, through this positive opinion, the CHMP recognizes the value of the thorough clinical and non-clinical studies we conducted to support the MAA for TransCon hGH," said Vibeke Miller Breinholt, Ascendis Pharma's Senior Vice President of Nonclinical Development & Bioanalysis. "Science is a core value at Ascendis and that translates into our commitment to doing all we can to ensure our medicines are as safe and effective as possible."

Earlier this year, the U.S. Food & Drug Administration approved TransCon hGH – marketed in the U.S. under the brand name SKYTROFA® (lonapegsomatropin-tcgd) – for the treatment of pediatric patients one year and older who weigh at least 11.5 kg (25.4 lb.) and have growth failure due to inadequate secretion of endogenous growth hormone. Ascendis Pharma is also developing TransCon hGH in Japan, and separately in China through VISEN Pharmaceuticals.

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; they also may experience metabolic abnormalities, psychosocial challenges, and an overall poor quality of life. For decades, the standard of care for GHD has been a daily subcutaneous injection of hGH to improve 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 uses its TransCon technologies to create new and potentially best-in-class therapies. Ascendis is headquartered in Copenhagen, Denmark, and has additional facilities in Heidelberg and Berlin, Germany; Palo Alto and Redwood City, California; and Princeton, New Jersey. Please visit www.ascendispharma.com to learn more.

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) the expected timing of the European Commission final decision on the MAA for TransCon hGH, (ii) Ascendis' ability to apply its platform technology to build a leading, fully integrated biopharma company, and (iii) Ascendis' use of 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: dependence on third party manufacturers and distributors to supply TransCon hGH, the SKYTROFA® Auto-Injector and other study drug for commercial sales in the U.S. and clinical studies; unforeseen safety or efficacy results in its oncology programs, TransCon hGH, TransCon PTH and TransCon CNP or other development programs; unforeseen expenses related to commercialization of lonapegsomatropin-tcgd in the U.S., the co-pay program, and the further development of TransCon hGH, 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 from

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' Annual Report on Form 20-F filed with the U.S. Securities and Exchange Commission (SEC) on March 10, 2021 and Ascendis' other future reports filed with, or submitted to, the SEC. Forward-looking statements do not reflect the potential impact of any future 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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