FDA Accepts Ascendis Pharma's Supplemental Biologics License Application for TransCon™ hGH for the Treatment of Adults with Growth Hormone Deficiency

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PDUFA goal date is July 27, 2025

COPENHAGEN, Denmark, Dec. 12, 2024 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND) today announced that the U.S. Food & Drug Administration (FDA) has accepted for review its supplemental Biologics License Application (sBLA) in adult growth hormone deficiency (GHD) for TransCon hGH (lonapegsomatropin-tcgd; marketed as SKYTROFA® for pediatric GHD). The FDA set a Prescription Drug User Fee Act (PDUFA) goal date of July 27, 2025.

"This marks another step towards achieving our objective to expand SKYTROFA's label beyond pediatric GHD and expand its reach to address new groups of patients," said Jan Mikkelsen, Ascendis Pharma's President and Chief Executive Officer. "Adult GHD is an undertreated condition associated with significant comorbidities and higher annual healthcare costs compared to the 5-10% of patients who receive treatment, indicative of the high unmet need."

The sBLA submission is based on results from foresiGHt, a Phase 3 randomized, parallel-arm, placebo-controlled (double-blind) and active-controlled (open-label) trial that compared the efficacy and safety of weekly TransCon hGH with weekly placebo and daily human growth hormone (hGH) in adults with GHD. The trial evaluated 259 adults with GHD aged 23 to 80 years old, randomized 1:1:1, titrated to receive a target fixed dose of TransCon hGH, placebo, or daily hGH based on age and oral estrogen intake with approximately equivalent hGH mg/week for TransCon hGH and daily hGH. TransCon hGH demonstrated superiority on its primary efficacy and key secondary efficacy endpoints at Week 38, with TransCon hGH-treated participants showing a statistically significant reduction from baseline in trunk fat and increase in total body lean mass at Week 38 compared to placebo. In the trial, TransCon hGH was generally safe and well tolerated, with no discontinuations related to study drug and with comparable safety and tolerability to daily hGH treatment.

About Adult Growth Hormone Deficiency
Growth hormone plays an essential role in the health of children and adults, promoting normal growth in children and maintenance of normal body composition and cardiometabolic health throughout adulthood. In adults, growth hormone boosts protein production, promotes fat utilization, enhances muscle mass, and helps regulate blood sugar levels. Adult GHD is a condition in which an individual's body does not produce enough growth hormone. Symptoms and morbidity can include central obesity, metabolic syndrome, decreased bone density, alterations in lipid profile and markers of cardiovascular risk, fatigue, general weakness, lack of muscle tone, and psychological symptoms such as cognitive impairment, social isolation, lack of motivation, and depression.[1]

About Ascendis Pharma A/S
Ascendis Pharma is applying its innovative TransCon technology platform 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, Ascendis uses its TransCon technologies to create new and potentially best-in-class therapies. Ascendis is headquartered in Copenhagen, Denmark and has additional facilities in Europe and the United States. Please visit 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 PDUFA goal date for SKYTROFA, (ii) Ascendis' objective to expand SKYTROFA's label and reach to address new groups of patients, (iii) Ascendis' ability to apply its TransCon technology platform to build a leading, fully integrated biopharma company, and (iv) 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, distributors and service providers for Ascendis' products and product candidates; unforeseen safety or efficacy results in Ascendis' development programs or on-market products; unforeseen expenses related to commercialization of any approved Ascendis products; unforeseen expenses related to Ascendis' development programs; unforeseen selling, general and administrative expenses, other research and development expenses and Ascendis' business generally; delays in the development of its programs related to manufacturing, regulatory requirements, speed of patient recruitment or other unforeseen delays; Ascendis' ability to obtain additional funding, if needed, to support its business activities; the impact of international economic, political, legal, compliance, social and business factors. 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 September 20, 2024 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 February 7, 2024. 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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Investor Contacts:
Scott Smith
Ascendis Pharma
ir@ascendispharma.com

Patti Bank
ICR Westwicke
+1 (415) 513-1284
patti.bank@icrhealthcare.com

Media Contact:
Melinda Baker
Ascendis Pharma
+1 (650) 709-8875
media@ascendispharma.com

1. Hoffman AR, Mathison T, Andrews D, Murray K, Kelepouris N, Fleseriu M. Adult Growth Hormone Deficiency: Diagnostic and Treatment Journeys From the Patients' Perspective. J Endocr Soc. 2022;6(7):bvac077. Published 2022 May 12. doi:10.1210/jendso/bvac077

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