

## PRESS RELEASE

# TransCon™ hGH (lonapegsomatropin) Achieved Statistically Superior Outcomes Compared to Placebo in Phase 3 foresiGHt Trial of Adults with Growth Hormone Deficiency

- *On primary efficacy and key secondary endpoints, TransCon hGH demonstrated statistically significant reduction from baseline in trunk fat and increase in total body lean mass at Week 38 compared to placebo*
- *TransCon hGH was generally safe and well tolerated, similar to daily hGH, with no discontinuations related to study drug*
- *Exploratory post-hoc analysis suggests the same treatment effects on target tissues as daily hGH*

**COPENHAGEN, Denmark, December 19, 2023 (GLOBE NEWSWIRE)** – Ascendis Pharma A/S (Nasdaq: ASND) today announced positive topline results from foresiGHt, its Phase 3 randomized, parallel-arm, placebo-controlled (double-blind) and active-controlled (open-label) trial to compare the efficacy and safety of TransCon hGH (lonapegsomatropin) with placebo and daily hGH (human growth hormone, somatropin) in adults with growth hormone deficiency (GHD).

The foresiGHt 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 endpoint at Week 38:
  - Change from baseline in trunk percent fat as measured by dual x-ray absorptiometry (TransCon hGH -1.67% vs. placebo +0.37%, LS mean difference = -2.04%,  $p < 0.0001$ )
- TransCon hGH demonstrated superiority on its key secondary efficacy endpoints at Week 38:
  - Change from baseline in total body lean mass (TransCon hGH +1.60 kg vs placebo -0.10 kg, LS mean difference = 1.70 kg,  $p < 0.0001$ )
  - Change from baseline in trunk fat mass (TransCon hGH -0.48 kg vs placebo +0.22 kg, LS mean difference = -0.70 kg,  $p = 0.0053$ )
- Exploratory post-hoc analysis at Week 38 demonstrated comparable treatment effect of TransCon hGH and daily hGH on target tissues. For patients with IGF-1 SDS levels  $\leq 1.75$  at Week 38:
  - Change from baseline in trunk percent fat (TransCon hGH -2.42% vs. daily hGH -2.59%)
  - Change from baseline in total body lean mass (TransCon hGH +1.70 kg vs daily hGH +1.37 kg)
  - Change from baseline in trunk fat mass (TransCon hGH -0.90 kg vs daily hGH -0.94 kg)
- TransCon hGH was generally safe and well tolerated, with no discontinuations related to study drug and with comparable safety and tolerability to daily hGH.

“TransCon hGH, our once-weekly growth hormone, is designed to deliver unmodified somatropin, thereby preserving the same overall endocrine benefits inherent in endogenous and daily hGH,” said Jan Mikkelsen, Ascendis Pharma’s President and CEO. “Next year, we plan to submit a supplemental Biologics License Application to the U.S. FDA for the adult GHD indication and look forward to reporting data from our Phase 2 program in Turner syndrome.”

### **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.<sup>1</sup>

### **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 Germany (Heidelberg and Munich) and the United States (Palo Alto and Redwood City, California, and Princeton, New Jersey). Please visit [ascendispharma.com](http://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) TransCon hGH’s ability to preserve the same overall endocrine benefits inherent in endogenous and daily somatropin, (ii) Ascendis’ plan to submit a supplemental Biologics License Application to the U.S. FDA next year, (iii) Ascendis’ plan to report data from its Phase 2 program in Turner syndrome, (iv) Ascendis’ ability to apply its TransCon technology platform to build a leading, fully integrated global biopharma company, and (v) 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, including inflation, the effects on its business from the worldwide COVID-19 pandemic and ongoing conflicts such as that in the region surrounding Ukraine and Russia. 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 February 16, 2023 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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<sup>1</sup>: 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