

## PRESS RELEASE

### **New InsiGHTS Trial of TransCon™ hGH (Lonapegsomatropin) in Turner Syndrome Achieved Primary Objective at Week 26**

- Results for all three TransCon hGH starting dose cohorts, in first clinical trial of an indication outside of growth hormone deficiency, showed a safety and tolerability profile comparable to daily somatropin
- Annualized height velocity was similar at Week 26 in once-weekly TransCon hGH-treated and daily somatropin-treated children

**COPENHAGEN, Denmark, December 16, 2024 (GLOBE NEWSWIRE)** – Ascendis Pharma A/S (Nasdaq: ASND) today announced positive Week 26 topline results from New InsiGHTS, its Phase 2 randomized, open-label, active-controlled trial in the U.S. to investigate the safety, tolerability, and efficacy of once-weekly TransCon hGH (lonapegsomatropin; approved for pediatric growth hormone deficiency) compared to daily somatropin in prepubertal children with Turner syndrome.

New InsiGHTS randomized and dosed 49 children with Turner syndrome aged 1 to 10 years old into one of four treatment groups 1:1:1:1 – one of three starting doses of TransCon hGH (0.24, 0.30, or 0.36 mg/kg/week) or an active comparator of daily somatropin with a starting dose of 0.35 mg/kg/week. Doses were individualized based on IGF-1.

- On the primary endpoint of annualized height velocity and secondary endpoint of change from baseline in height SDS, children treated with TransCon hGH demonstrated improved growth similar to daily somatropin at Week 26, independent of starting dose.
- 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 somatropin.

“Week 26 topline data from the New InsiGHTS Trial showed that once-weekly TransCon hGH provided comparable safety and endocrine benefits to those seen with the established use of daily somatropin in Turner syndrome,” said Jan Mikkelsen, Ascendis Pharma’s President and Chief Executive Officer. “These positive results lay the foundation for our planned basket trial in 2025 to support label expansion.”

Ascendis expects Week 52 results for lonapegsomatropin-treated children compared to daily somatropin-treated children in 2025.

#### **About Turner Syndrome**

Turner syndrome is the most common congenital sex chromosomal condition in females, with an estimated prevalence of 1 out of every 2,000 to 2,500 live female births. Short stature, associated with short stature homeobox-containing gene (SHOX) haploinsufficiency, is the most common clinical feature of Turner syndrome. Clinical manifestations of Turner syndrome affect multiple organ systems and are

associated with significant and potentially life-threatening complications including cardiovascular disease, ovarian dysfunction, endocrine disease, renal malformation, liver disease, sensorineural hearing loss, and varied neuropsychological manifestations.

### **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](https://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 timing of Week 52 results for lonapegsomatropin-treated patients compared to daily somatropin-treated patients, (ii) Ascendis' plans for a basket trial in 2025 and SKYTROFA's potential label expansion, (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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