PRESS RELEASE

# Ascendis Pharma Presents Results from Long-Term enliGHten Trial of TransCon™ hGH in Pediatric Growth Hormone Deficiency

- *The majority of children and adolescents treated once weekly with TransCon hGH (lonapegsomatropin) met or exceeded average parental height SDS at time of treatment completion or last visit*

- *Trial demonstrated the long-term safety of TransCon hGH in patients treated up to 6 years*

**COPENHAGEN, Denmark, September 23, 2023 (GLOBE NEWSWIRE) –** Ascendis Pharma A/S (Nasdaq: ASND) today announced results from enliGHten, the Company's open-label extension trial evaluating the long-term safety and efficacy of TransCon hGH as a once-weekly treatment for children and adolescents with growth hormone deficiency (GHD). Results were shared today in Poster LB-17 at ESPE 2023, the annual meeting of the European Society for Paediatric Endocrinology being held in The Hague, September 21-23.

The enliGHten trial enrolled 298 participants (mean age 10.3 years) from the Phase 3 heiGHt Trial of treatment-naïve pediatric GHD patients and the Phase 3 fliGHt Trial of pediatric GHD patients switching from daily somatropin treatment. Patients in these trials received a total of up to 6 years of treatment with TransCon hGH.

At the time of the enliGHten Trial conclusion, 81 participants were designated as treatment completers, based on their physician's determination that treatment for pediatric GHD was no longer required. Of these treatment completers, 59% met or exceeded their average parental height SDS, with mean TransCon hGH treatment duration of 3.2 years. Treatment completers' baseline mean height standard deviation score (SDS) at the beginning of the open-label extension trial was -1.6, compared to mean height SDS of -0.4 (achieving height similar to their parents') at their final study visit. At the time of final visit, all treatment completers were Tanner stage IV or V, a categorization of physical development during puberty.

"As an investigator in the enliGHten Trial, I am pleased to see results confirming that treated children and adolescents have continued to grow well, achieving statures in line with those of their parents," said Aristides K. Maniatis, M.D., F.A.A.P., pediatrician and endocrinologist at Rocky Mountain Pediatric Endocrinology. "Additionally, these results demonstrate that long-term treatment goals can be safely reached with TransCon hGH administered once weekly."

TransCon hGH was generally safe and well-tolerated. The most commonly reported adverse events over the course of the trial were categorized as infections, injury, and respiratory/thoracic/medical disorders.

The majority of adverse events were mild in severity and unrelated to treatment. No adverse events led to discontinuation of the study treatment.

**About TransCon hGH (Lonapegsomatropin)**
TransCon hGH (lonapegsomatropin) is a prodrug of somatropin administered once weekly, designed to provide sustained release of active, unmodified somatropin. The unmodified, unbound somatropin released from lonapegsomatropin has the identical 191 amino-acid sequence and size (22 kDa) as endogenous growth hormone. TransCon hGH is approved and marketed as SKYTROFA® (lonapegsomatropin-tcgd) in the United States and as SKYTROFA® (lonapegsomatropin) in the European Union as a once-weekly treatment for children and adolescents with GHD.

**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 Germany (Heidelberg and Munich) and the United States (Palo Alto and Redwood City, California, and Princeton, New Jersey). 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) TransCon hGH's ability to provide sustained release of active, unmodified somatropin; (ii) Ascendis' ability to apply its TransCon platform technology to build a leading, fully integrated, global 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, 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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