

PRESS RELEASE

TransCon[®] hGH Boosted Treatment Benefits of TransCon[®] CNP in Children with Achondroplasia at Week 26 Interim Analysis of the Phase 2 COACH Trial

- *For the TransCon CNP treatment-naïve cohort, combination treatment resulted in mean annualized growth velocity (AGV) of 9.14 cm/year, with an improvement in mean ACH height Z-score of +0.53 over 26 weeks*
- *For the TransCon CNP-treated cohort, combination treatment resulted in mean AGV of 8.25 cm/year, with an improvement in mean ACH height Z-score of +0.44 over 26 weeks*
- *The combination of TransCon hGH and TransCon CNP demonstrated accelerated improvement in body proportionality at Week 26, aligning with the increase in linear growth*
- *Safety and tolerability data consistent with those observed for TransCon hGH and TransCon CNP monotherapies; combination treatment was generally well tolerated, with generally mild treatment-emergent adverse events (TEAEs)*
 - *Ascendis to host conference call today at 8:00 am ET*

COPENHAGEN, Denmark, June 9, 2025 (GLOBE NEWSWIRE) – Ascendis Pharma A/S (Nasdaq: ASND) today announced Week 26 interim analysis results from its ongoing COACH Trial, the first clinical trial to evaluate combination treatment with once-weekly investigational TransCon CNP (navepegritide) and once-weekly TransCon hGH (lonapegsomatropin) in children with achondroplasia. Results demonstrated that TransCon hGH boosted treatment benefits of TransCon CNP, resulting in significant growth and proportionality improvements in children with achondroplasia after 26 weeks of combination treatment, with a safety and tolerability profile consistent with those observed for TransCon hGH and TransCon CNP monotherapies.

TransCon CNP, which is under priority review as a monotherapy for children with achondroplasia by the U.S. Food & Drug Administration (FDA), is an investigational prodrug of C-type natriuretic peptide (CNP) administered once weekly, providing continuous exposure of active CNP to receptors on tissues throughout the body, including growth plates and skeletal muscle. TransCon hGH is a prodrug of somatropin administered once weekly, providing sustained release of active, unmodified somatropin. TransCon hGH is approved and marketed as SKYTROFA[®] for the treatment of pediatric growth hormone deficiency and is in development for other indications.

“TransCon CNP as a monotherapy has demonstrated the potential to transform the treatment of achondroplasia, and the COACH Trial at Week 26 demonstrates that TransCon hGH has the potential to boost treatment benefits of TransCon CNP with a safety profile consistent with monotherapies,” said Aimee Shu, M.D., Executive Vice President of Endocrine & Rare Disease Medical Sciences and Chief Medical Officer at Ascendis Pharma. “These results highlight the unique portfolio of once-weekly

TransCon CNP and once-weekly TransCon hGH, with complementary modes of action, to improve the treatment landscape for growth disorders and physical functioning.”

COACH Trial Design

The COACH Trial is an ongoing proof-of-concept prospective Phase 2 open-label trial to investigate the efficacy, safety, and tolerability of combined treatment with once-weekly TransCon CNP at 100 µg/kg/week and once-weekly TransCon hGH at 0.30 mg/kg/week in children with achondroplasia aged 2 to 11 years. The trial included a cohort of TransCon CNP treatment naïve children (N=12, mean age 4.67 years) and a cohort of TransCon CNP-treated children (N=9, mean age 7.89 years) who had received TransCon CNP (100 µg/kg/week) for a mean of 2.56 years in clinical trials. The trial population is representative of children with achondroplasia, except for the observed growth benefit in the TransCon CNP-treated cohort. The interim analysis will be followed by Week 52 data, expected in Q4 2025, and Ascendis plans to initiate a Phase 3 trial in Q4 2025.

Highlights of the Interim Topline Week 26 COACH Trial Results

- For TransCon CNP treatment-naïve children, mean annualized growth velocity (AGV) was 9.14 cm/year, representing an increase from baseline at Week 26 of 4.23 cm/year, with an improvement in mean ACH height Z-score of +0.53 over 26 weeks.
- For TransCon CNP-treated children, mean AGV was 8.25 cm/year, representing an increase from baseline at Week 26 of 3.10 cm/year, with an improvement in mean ACH height Z-score of +0.44 over 26 weeks.
- Mean AGV with TransCon CNP and TransCon hGH combination treatment exceeded the 97th percentile of average-stature children.
- Children treated with TransCon hGH and TransCon CNP demonstrated accelerated improvement in body proportionality at Week 26, aligning with the increase in linear growth.
- Bone age advanced in line with chronologic age.
- Safety and tolerability data were consistent with those observed for TransCon hGH and TransCon CNP monotherapies; combination treatment was generally well tolerated, with generally mild TEAEs.

A slide presentation with these data can be found on the Investor Relations & News section of the Ascendis Pharma website: <https://investors.ascendispharma.com>.

Conference Call and Webcast Information

Ascendis Pharma will host a conference call and webcast today at 8:00 am Eastern Time (ET) to discuss these results. Those who would like to participate may access the live webcast [here](#), or register in advance for the teleconference [here](#). The link to the live webcast will also be available on the Investors & News section of the Ascendis Pharma website at <https://investors.ascendispharma.com>. A replay of the webcast will be available on that page shortly after the conclusion of the event for 30 days.

About Achondroplasia

Achondroplasia is a rare genetic condition arising from a systemic fibroblast growth factor receptor 3 (FGFR3) variant that leads to an imbalance in the effects of the FGFR3 and CNP signaling pathways, estimated to affect more than 250,000 people worldwide. While historically considered a bone growth disorder, the FGFR3 variant seen in achondroplasia is expressed in tissues throughout the body, causing serious muscular, neurological, and cardiorespiratory complications in addition to skeletal dysplasia. Medical complications of achondroplasia vary across different stages of life. Throughout infancy and childhood, observed complications include spinal deformities, enlarged brain ventricles, impaired muscle strength and stamina, hearing deficits and chronic ear infections, upper airway obstructions, sleep-disordered breathing, hip problems, leg bowing, and chronic pain; many of these persist or worsen in adulthood. These medical complications can have detrimental effects on quality of life, physical functioning, and psychosocial function. Individuals with achondroplasia often require multiple surgeries and procedures to alleviate the condition's many complications.

About Ascendis Pharma A/S

Ascendis Pharma is a global biopharmaceutical company focused on applying our innovative TransCon technology platform to make a meaningful difference for patients. Guided by our core values of Patients, Science, and Passion, and following our algorithm for product innovation, we apply TransCon to develop new therapies that demonstrate best-in-class potential to address unmet medical needs. 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) TransCon CNP's potential to transform the treatment of achondroplasia, (ii) TransCon hGH's potential to boost treatment benefits of TransCon CNP with a safety profile consistent with monotherapies, (iii) the potential for the combination of TransCon CNP and TransCon hGH to improve the treatment landscape for growth disorders and physical functioning, (iv) the expected timing of Week 52 data from the COACH Trial and Ascendis' plans to initiate a Phase 3 trial in Q4 2025, (v) Ascendis' ability to apply its TransCon technology platform to make a meaningful difference for patients, and (vi) Ascendis' application of its TransCon technologies to develop new therapies that demonstrate best-in-class potential to address unmet medical needs. 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 tariffs and trade policies. 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 12, 2025, 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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