

PRESS RELEASE

Week 52 COACH Trial Topline Results Confirm Consistent and Durable Treatment Benefits in Children with Achondroplasia (ACH)

- *Once-weekly TransCon® CNP and TransCon® hGH combination therapy showed durable growth without compromising safety or tolerability at 52 weeks*
- *Annualized growth velocity (AGV) exceeded the 97th-percentile of average stature children*
- *ACH height Z-score improvements indicated a tripling of efficacy compared to TransCon CNP monotherapy*
- *Combination therapy demonstrated benefits beyond linear growth with improvements in body proportionality and arm span, aligning with the increase in linear growth*
- *All children completed 52 weeks of treatment and remain on therapy in COACH as of today*
- *Combination therapy was generally well tolerated, with generally mild treatment-emergent adverse events (TEAEs), consistent with TransCon CNP and TransCon hGH monotherapies*
 - *Ascendis to host conference call today at 4:30pm ET*

COPENHAGEN, Denmark, January 8, 2026 (GLOBE NEWSWIRE) – Ascendis Pharma A/S (Nasdaq: ASND) today announced topline results from Week 52 of COACH, the first Phase 2 clinical trial to evaluate combination therapy with once-weekly TransCon CNP (navepegritide) and once-weekly TransCon hGH (lonapegsomatropin) in children with achondroplasia. At Week 52, combination therapy showed durable growth without compromising safety or tolerability. In addition, combination therapy demonstrated benefits beyond linear growth with improvements in body proportionality and arm span, aligning with the increase in linear growth. Safety and tolerability of combination therapy were consistent with those observed for monotherapies of TransCon CNP and TransCon hGH and was generally well-tolerated, with generally mild TEAEs. The combination data underscore the potential of TransCon CNP to become the backbone therapy for addressing the underlying biology of achondroplasia, with TransCon hGH providing complementary benefit.

“The COACH Trial has demonstrated unmatched improvements in growth and benefits beyond linear growth without compromising safety or tolerability compared to historical data.” said Dr. Ciara McDonnell, MD FRCPI, Consultant in Pediatric Endocrinology & Diabetes at Children's Health Ireland in Dublin. “Seeing the additive effects of these complementary once-weekly therapies, where TransCon CNP makes the growth plates receptive to the growth-promoting effects of TransCon hGH, suggests we may be entering an era where rational dual-agent regimens such as this become a new standard of care in achondroplasia and other growth disorders.”

TransCon CNP 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 somatotropin administered once weekly, providing sustained release of active, unmodified somatotropin. TransCon CNP is under Priority Review by the U.S. Food & Drug Administration (FDA), with a PDUFA target action date of February 28, 2026, and by the European Medicines Agency as a monotherapy for children with achondroplasia. TransCon hGH is investigational in achondroplasia and other indications and is approved and marketed as SKYTROFA[®] for the treatment of pediatric and adult growth hormone deficiency.

COACH Trial Design

COACH 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 a starting dose of 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 and the treatment benefit of TransCon CNP.

Highlights of the Topline Week 52 COACH Trial Results

- For the TransCon CNP treatment-naïve cohort, mean annualized growth velocity (AGV) was 8.80 cm/year, with an improvement in mean ACH height Z-score of +1.02 over 52 weeks, indicating a tripling of efficacy compared to TransCon CNP monotherapy.
- For the TransCon CNP-treated cohort (average treatment of 2.56 years), mean AGV was 8.42 cm/year, representing an increase from baseline at Week 52 of 3.28 cm/year, with an improvement in mean ACH height Z-score of +0.86, increasing from 1.28 to 2.15 over 52 weeks.
- After 52 weeks, children treated with combination therapy exceeded the 97th-percentile AGV of average-stature children.
- Children treated with TransCon CNP and TransCon hGH demonstrated improvements in body proportionality after 52 weeks, aligning with the increase in linear growth.
- Arm span of children treated with combination therapy improved beyond the 84th-percentile of children with achondroplasia at Week 52.
- Bone age remained consistent with chronological age at Week 52.
- Safety and tolerability were consistent with those observed for TransCon CNP and TransCon hGH monotherapies. Combination treatment was generally well-tolerated, with a low incidence of injection site reactions and generally mild TEAEs.
- All children completed 52 weeks of treatment and remain on therapy in the COACH Trial.

“The dwarfism community has long emphasized the importance of research that honors dwarf and disability pride while also deepening understanding of outcomes that matter most in achondroplasia,” said Michael Hughes, Chair of the Biotech Industry Liaison Committee at Little People of America.

“Although the COACH findings are still early, it is encouraging to see meaningful improvements in arm

span and body proportionality alongside changes in linear height, as these endpoints provide important context beyond height alone. Given the diversity of healthcare goals within the dwarfism community, data like these may help expand the range of options individuals and families consider as they weigh what matters most to them.”

“Once-weekly TransCon CNP, designed to provide continuous exposure to CNP, has demonstrated its potential to transform the treatment of achondroplasia, positioning it as a potential long-term foundation of care,” said Jan Møller Mikkelsen, President and Chief Executive Officer at Ascendis Pharma. “The Week 52 COACH Trial results show that combination therapy with TransCon CNP and TransCon hGH further unlocks the transformative potential in children with achondroplasia and may set new treatment standards with clinically meaningful improvements in growth, body proportionality, and arm span with a safety and tolerability profile essential for long-term use in children.”

In Q4 2025, Ascendis submitted a protocol and held an end of Phase 2 meeting with the FDA regarding a Phase 3 trial of TransCon CNP and TransCon hGH in pediatric achondroplasia.

A slide presentation with the COACH Trial Week 52 combination therapy data can be found on the Investors & 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 4:30 pm 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 abnormalities, 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) the potential for dual-agent regimens to become a new standard of care in achondroplasia and other growth disorders; (ii) the PDUFA target action date; (iii) the potential for once-weekly TransCon CNP to transform the treatment of achondroplasia and position itself as a long-term foundation of care; (iv) the efficacy of combination therapy with TransCon CNP and TransCon hGH in children with achondroplasia, including establishing new efficacy benchmarks; (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, without limitation: dependence on third-party manufacturers, distributors, and service providers for Ascendis' products and product candidates; risks related to regulatory review and approval, including the possibility of delays, requests for additional data or analyses, restrictions or limitations on use, approval with labeling that is more limited than expected, or failure to obtain approval in the United States, European Union, or other jurisdictions; clinical development risks, including that results from ongoing or future trials may not confirm earlier data; unforeseen safety or efficacy findings in development programs or on-market products; manufacturing, supply chain, quality, or logistics issues that could delay development or commercialization; unforeseen expenses related to commercialization of any approved Ascendis products; unforeseen research and development or selling, general and administrative expenses and other costs impacting Ascendis' business generally; market acceptance, pricing, and reimbursement challenges, including payer coverage decisions and health technology assessments; competitive developments, including new or improved therapies; intellectual property protection, freedom-to-operate, and litigation risks; Ascendis' ability to obtain additional funding, if needed, to support its business activities; cybersecurity, data privacy, and information technology disruptions; and the impact of international economic, political, legal, compliance, public health, and business factors, including tariffs, trade policies, currency fluctuations, and geopolitical events. 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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