PRESS RELEASE

# First Infant Data from Ascendis Trial of Once-Weekly TransCon CNP (Navepegritide) Presented at ESPE 2026

- *Achondroplasia Foramen Magnum Score was stable or improved between baseline and Week 52 with TransCon CNP treatment*
- *Treatment with TransCon CNP increased linear growth in the sentinel cohort, with an improvement in ACH-specific supine length Z-score of +0.42 from baseline through Week 52*
- *Treatment with TransCon CNP was generally well tolerated, with no reports of injection site reactions over the 52-week treatment period*

**COPENHAGEN, Denmark, September 9, 2026 (GLOBE NEWSWIRE) –** Ascendis Pharma A/S (Nasdaq: ASND) today announced Week 52 data from the open-label sentinel cohort portion of the reACHin Trial (which preceded the now fully enrolled double-blinded portion of the trial) showing that treatment with once-weekly TransCon CNP (navepegritide) provided stabilization or improvement in foramen magnum stenosis (a skull bone narrowing that can lead to dangerous brain stem and spinal cord compression), as well as linear growth benefits, in infants with achondroplasia aged 0 to <2 years. The data were presented by Geneviève Baujat, M.D., clinical geneticist at Necker-Enfants Malades Hospital (Paris), during ESPE 2026, the annual meeting of the European Society for Paediatric Endocrinology.

"The impressive safety profile and data from this sentinel group show how early intervention with weekly TransCon CNP treatment could help address medical complications and growth limitations in infants with achondroplasia," said Dr. Baujat. "Of note, the stabilization or improvement in foramen magnum stenosis observed in this sentinel cohort indicate the potential for TransCon CNP to address a key concern in this vulnerable population."

ReACHin is a pivotal Phase 2, randomized, placebo-controlled trial to evaluate the safety, tolerability, and efficacy of once-weekly TransCon CNP at the 100 µg/kg/week dose in at least 66 treatment-naïve infants aged 0 to <2 years with genetically confirmed achondroplasia, followed by a 52-week open-label extension. Prior to recruitment for the 52-week double-blind period, seven infants with achondroplasia (mean age 11.7 months) were enrolled in an open-label sentinel cohort to evaluate the safety and pharmacokinetics (PK) of TransCon CNP in this age group. Additional outcome measures at Week 52 included Achondroplasia Foramen Magnum Score (AFMS, a standardized MRI-based grading system used to evaluate and classify the severity of foramen magnum stenosis), change from baseline in ACH-specific supine length Z-score, and annualized growth velocity (AGV).

**Highlights from the Week 52 reACHin Trial Sentinel Cohort Data**

- Treatment with TransCon CNP led to stable or improved cranio-cervical junction health at Week 52, as measured by:
    - AFMS was stable or improved between baseline and Week 52 in all children
    - Mean change from baseline in sagittal diameter of foramen magnum was +3.15 mm
    - There were no decompression surgeries during the 52-week period
- Treatment with TransCon CNP increased linear growth at Week 52:
    - Mean change from baseline in ACH-specific supine length Z-score was +0.42
    - Mean AGV was 9.9 cm/year
- PK was comparable to that observed in older children, supporting the 100 µg/kg/week dose
- TransCon CNP was generally well tolerated and the safety data were consistent with those previously reported in other trials:
    - No reports of injection site reactions over the 52-week treatment period
    - No deaths, fractures, bone-related safety events, or symptomatic hypotension
    - No AEs were assessed by investigators as related to treatment and no AEs led to treatment disruption or discontinuation or to trial withdrawal

"Achondroplasia can affect many aspects of a child's health and quality of life. For this reason, and not for height alone, we believe families should have broad access to as many treatment options as possible for their child," said Susana Noval, Director of Fundación ALPE Acondroplasia, an advocacy organization in Spain. "This initial infant data for TransCon CNP demonstrates a very encouraging safety and tolerability profile, and we look forward to following the study as more data emerge."

**About TransCon CNP**

TransCon CNP is a prodrug of C-type natriuretic peptide (CNP) administered once weekly, designed to provide continuous exposure of active CNP to receptors on tissues throughout the body to counteract the overactive FGFR3 signaling in achondroplasia. In February 2026, TransCon CNP was approved by the U.S. Food & Drug Administration (FDA) under the trade name YUVIWEL® to increase linear growth in pediatric patients 2 years of age and older with achondroplasia with open epiphyses. Ascendis Pharma's Marketing Authorisation Application for YUVIWEL is under review by the European Medicines Agency, with a regulatory decision anticipated in the fourth quarter of 2026.

**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 affect physical well-being and quality of life, and may be impacted by a range of individual, clinical, and social factors. Some individuals with achondroplasia require multiple procedures and surgeries to address specific functional or anatomical concerns.

**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 within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. Examples of such statements include, but are not limited to, statements relating to (i) the potential for TransCon CNP (navepegritide) to address foramen magnum stenosis and support linear growth in infants with achondroplasia, (ii) the potential safety, tolerability, and efficacy benefits of TransCon CNP in infants based on reACHin Trial sentinel cohort data, (iii) the anticipated timing of a regulatory decision from the European Medicines Agency regarding YUVIWEL, (iv) Ascendis' ability to apply its TransCon technology platform to make a meaningful difference for patients, and (v) Ascendis' use of TransCon to develop new and potentially best-in-class therapies 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 11, 2026, 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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