

PRESS RELEASE

New 2-Year Data from Pivotal ApproaCH Trial of TransCon[®] CNP (Navepegritide) Show Pronounced Gains in Growth Outcomes in Children with Achondroplasia Aged ≥ 5 Years

COPENHAGEN, Denmark, May 6, 2026 (GLOBE NEWSWIRE) – Ascendis Pharma A/S (Nasdaq: ASND) today announced new data from a subgroup analysis showing that children with achondroplasia ≥ 5 years of age at enrollment treated with once-weekly TransCon CNP (navepegritide) in its pivotal ApproaCH Trial demonstrated significantly greater annualized growth velocity (AGV) compared to placebo at Week 52, and sustained these growth improvements through up to two years of treatment. The safety profile for this subgroup through up to two years of treatment was similar to the overall population, with a low rate of injection site reactions (ISRs, all mild), no symptomatic hypotension, and no acceleration of bone age. The data follow previously reported Week 104 results showing consistent improvements in growth and body proportionality in the overall population, and expand on data recently presented by M. Jennifer Abuzzahab, M.D. during PES 2026, the annual meeting of the Pediatric Endocrine Society.

“We are pleased to see confirmation of the expected sustained growth improvements in these children, with a consistent safety and tolerability profile,” said Aimee D. Shu, M.D., Executive Vice President of Endocrine & Rare Disease Medical Science and Chief Medical Officer at Ascendis Pharma. “These results, along with the improved skeletal alignment and body proportionality and positive changes in health-related quality of life previously reported for the overall population¹ further highlight TransCon CNP’s ability to promote healthy, proportional growth in children with achondroplasia across age groups.”

ApproaCH Trial Design

ApproaCH was a randomized, double-blind, placebo-controlled pivotal trial in 84 children with achondroplasia aged 2–11 years, investigating TransCon CNP (100 $\mu\text{g}/\text{kg}$ once-weekly) versus placebo for 52 weeks, followed by a 52-week open-label extension (OLE) period in which all participants received TransCon CNP through Week 104. Fifty-three of the 84 children were ≥ 5 years of age at the time of their enrollment in the trial.

Highlights of the ApproaCH Trial Data Through Week 104

Subgroup of children aged ≥ 5 years at enrollment

	AGV (cm/year)		
	LS Mean	Observed Mean ³	
	Week 52	Week 52	Week 104
TransCon CNP (n=36)	5.79	5.84	5.71
Placebo/TransCon CNP ¹ (n=17)	4.02	3.88	5.53
TransCon CNP vs. Placebo, Treatment Difference [95% CI]	+1.78 [1.22, 2.33] p<0.0001	+1.97² [1.37, 2.56] p<0.0001	-

	ACH-Specific Height Z-score, Change from Baseline			CDC-Based Height Z-score, Change from Baseline		
	LS Mean	Observed Mean ³		LS Mean	Observed Mean ³	
	Week 52	Week 52	Week 104	Week 52	Week 52	Week 104
TransCon CNP (n=36)	+0.38	+0.38	+0.75	+0.28	+0.26	+0.58
Placebo/TransCon CNP ¹ (n=17)	+0.07	+0.07	+0.46	-0.05	-0.02	+0.36
TransCon CNP vs. Placebo, Treatment Difference² [95% CI]	+0.31 [0.20, 0.42] p<0.0001	+0.30 [0.18, 0.42] p<0.0001	-	+0.32 [0.20, 0.44] p<0.0001	+0.29 [0.14, 0.44] p=0.0004	-

Note: The observed mean is a simple average of recorded measurements; the LS mean is a model-based estimated average that adjusts for selected variables, typically baseline patient characteristics, enabling a more balanced comparison across arms of a clinical trial.

¹ Week 104 data reflects placebo patients that crossed over to TransCon CNP treatment at Week 52

² Not presented at PES 2026; included for context

³ Treatment differences between TransCon CNP and placebo were estimated from a T-test

Through up to two years of treatment, the safety profile in children ≥ 5 years was similar to the overall population, with a low rate of ISRs (all mild), no symptomatic hypotension, and no acceleration of bone age. Most adverse events in TransCon CNP-treated children were mild or moderate, with none leading to treatment discontinuation or withdrawal from the trial.

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

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 YUWIWEL[®] 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 YUWIWEL 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, and is associated with an increased risk of muscular, neurological, and cardiorespiratory complications in addition to skeletal dysplasia. Medical complications of achondroplasia can vary from individual to individual and across different stages of life. Throughout infancy and childhood, observed complications include spinal abnormalities, enlarged brain ventricles, impaired muscle strength and reduced stamina, hearing deficits and chronic ear infections, upper airway obstructions, sleep-disordered breathing, hip problems, leg bowing, and chronic pain; some of which 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) TransCon CNP's ability to promote healthy, proportional growth in children with achondroplasia across age groups, (ii) Ascendis' ability to apply its TransCon technology platform to make a meaningful difference for patients and (iii) Ascendis' use of TransCon 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, 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.

Ascendis, Ascendis Pharma, the Ascendis Pharma logo, TransCon, and YUVIWEL[®] are trademarks owned by the Ascendis Pharma group. © May 2026 Ascendis Pharma A/S.

Investor Contact:

Chad Fugere
Ascendis Pharma
+1 (650) 519-7494

Media Contact:

Melinda Baker
Ascendis Pharma
+1 (650) 709-8875