New Two-Year Data from Pivotal ApproaCH Trial Showing Durable Benefits of TransCon® CNP (Navepegritide) in Children with Achondroplasia Shared at ACMG 2026

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COPENHAGEN, Denmark, March 16, 2026 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND) today announced new data from its pivotal ApproaCH Trial showing that children with achondroplasia treated with once-weekly TransCon CNP (navepegritide) maintained consistent improvements in growth through Week 104, with further improvement in body proportionality during the second year of treatment. These and other results were shared in an oral presentation by Dr. Ravi Savarirayan, M.B.B.S., M.D., Murdoch Children's Research Institute (Australia), during ACMG 2026, the Annual Clinical Genetics Meeting held March 10-14 in Baltimore, Maryland.

"Two-year data from the ApproaCH Trial demonstrated continued improvement in body proportionality and sustained increases in linear growth," said Professor Savarirayan. "In addition, children switching from placebo to TransCon CNP at Week 52 demonstrated one-year results that mirrored those previously reported in three randomized double-blind, placebo-controlled trials of TransCon CNP, highlighting its potential as a transformative once-weekly treatment option for children with achondroplasia."

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. FDA under the trade name YUVIWEL® to increase linear growth in pediatric patients 2 years of age and older with achondroplasia with open epiphyses. The Marketing Authorization Application for YUVIWEL is under review by the European Medicines Agency with a regulatory decision anticipated in the fourth quarter of 2026.

ApproaCH Trial Design
The ApproaCH Trial was a randomized, double-blind, placebo-controlled pivotal trial in 84 children with achondroplasia aged 2–11 years, investigating TransCon CNP (100 µg/kg once-weekly) versus placebo for 52 weeks, followed by an open-label extension in which all participants received TransCon CNP through Week 104.

Highlights of the Week 104 ApproaCH Trial Results

- Treatment with TransCon CNP demonstrated continued improvement in observed change from baseline in upper-to-lower body segment ratio during the second year, from -0.04 at Week 52 to -0.10 at Week 104. Children who switched from placebo to TransCon CNP at Week 52 experienced similar improvements, from -0.02 at Week 52 to -0.10 at Week 104.
- Treatment benefit in change in height Z-scores (both achondroplasia-specific and CDC-based) from Weeks 52 to 104 were consistent with that observed in TransCon CNP-treated children from Weeks 0 to 52.
- Through two years of treatment, TransCon CNP was generally well-tolerated. Most adverse events in TransCon CNP-treated children were mild or moderate, with none leading to treatment discontinuation or withdrawal from the trial. There were no occurrences of symptomatic hypotension, and the overall rate of injection-site reactions, all of which were mild, was 0.35 per person-year of exposure.
- Retention in the pivotal ApproaCH Trial was strong with 80 of 84 children enrolled completing the trial, and all 80 children enrolling into the long-term, open-label AttaCH extension 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 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 potential as a transformative once-weekly treatment option for children with achondroplasia, (ii) the anticipated timing of a regulatory decision by the European Medicines Agency, (iii) Ascendis' ability to apply its TransCon technology platform to make a meaningful difference for patients, and (iv) Ascendis' ability to apply TransCon 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 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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