Results of Pivotal ApproaCH Trial of TransCon® CNP (Navepegritide) in Children with Achondroplasia Published in JAMA Pediatrics

November 17, 2025 at 4:01 PM EST

COPENHAGEN, Denmark, Nov. 17, 2025 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND) today announced that pivotal Week 52 results from its randomized double-blind, placebo-controlled ApproaCH Trial of investigational once-weekly TransCon® CNP (navepegritide) in children with achondroplasia have been published in *JAMA Pediatrics,* a journal of the American Medical Association. In the publication, titled "Once-Weekly Navepegritide in Children with Achondroplasia: The ApproaCH Randomized Clinical Trial," the authors report that treatment with TransCon CNP led to significantly higher annualized growth velocity (AGV) at Week 52 compared to placebo (primary endpoint), as well as improved lower-limb alignment and body proportionality and positive changes in health-related quality of life, with a safety and tolerability profile similar to placebo. The abstract can be accessed on the JAMA Pediatrics website.

"Children randomized to navepegritide had significantly better growth and improvements in important health outcomes compared with placebo," said Ravi Savarirayan, MBBS, M.D., Group Leader of Molecular Therapies at Murdoch Children's Research Institute in Melbourne, Australia. "These findings show that navepegritide is a promising potential new treatment option to reduce the medical burden of this condition, with once-weekly dosing and a low rate of injection site reactions."

"To help guide their healthcare decision, families want information beyond changes in height to understand how an intervention may affect the potential medical challenges of achondroplasia," said Michael Hughes, Chair of the Biotech Industry Liaison Committee at Little People of America. "Including these endpoints in blinded, controlled studies, as done in ApproaCH begins to fill that gap, and our community looks forward to seeing more research to deepen understanding in these areas."

TransCon CNP (navepegritide) is an investigational prodrug of C-type natriuretic peptide (CNP) administered once weekly, designed for continuous inhibition of the overactive FGFR3 pathway in achondroplasia by providing continuous exposure of active CNP to receptors on tissues throughout the body.

ApproaCH was a randomized, double-blind, placebo-controlled trial evaluating TransCon CNP in 84 children with achondroplasia ages 2-11, randomized 2:1 to receive TransCon CNP at the 100 µg/kg/week dose or placebo in the 52-week double-blind period, followed by an open-label extension through Week 104.

In addition to the key primary endpoint of annualized growth velocity (AGV) superior to placebo, favorable impacts on body proportionality and leg bowing were reported at Week 52. These analyses showed treatment with TransCon CNP decreased upper-to-lower body segment ratio from baseline to Week 52 and improved tibial-femoral angle (TFA), mechanical axis deviation (MAD), and fibula-to-tibia length ratio from baseline to Week 52 compared to placebo.

In the trial, treatment with TransCon CNP resulted in numerical improvements in health-related quality of life compared to placebo, as measured across several Achondroplasia Child Experience Measure (ACEM) domains. The benefits of TransCon CNP were achieved without accelerating bone age or negatively affecting spinal curvature. In the trial, TransCon CNP demonstrated a safety and tolerability profile similar to placebo, with the majority of adverse events (AEs) mild or moderate. Injection site reaction rates were low, and no observed symptomatic hypotension or bone fractures were reported.

"Across our development programs for TransCon CNP, we strive to demonstrate benefits that the achondroplasia community have told us are important to them," said Aimee Shu, M.D., Executive Vice President of Endocrine & Rare Disease Medical Science and Chief Medical Officer at Ascendis Pharma. "We are therefore especially pleased to see these overall results of our pivotal trial for TransCon CNP, including results that go beyond linear growth, published in this prestigious journal."

TransCon CNP as a potential treatment for children with achondroplasia is under Priority Review by the U.S. Food & Drug Administration (Prescription Drug User Fee Act target date November 30, 2025) and is also under review by the European Medicines Agency.

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 individuals 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 of TransCon CNP (navepegritide) as a new treatment option and its potential to reduce medical burden; (ii) the Prescription Drug User Fee Act target date; (iii) Ascendis' ability to apply its TransCon technology platform to make a meaningful difference for patients; and (iv) 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; and 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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Source: Ascendis Pharma