

PRESS RELEASE

New Data Shows Improvements in Growth and Bone Morphometry in Children with Achondroplasia Treated with TransCon[®] CNP (Navepegritide)

COPENHAGEN, Denmark, May 13, 2025 (GLOBE NEWSWIRE) – Ascendis Pharma A/S (Nasdaq: ASND) today announced new data showing improvements in growth and bone morphometry from Week 52 of its pivotal ApproaCH Trial of TransCon CNP (navepegritide) in children with achondroplasia. 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.

The bone morphometry data were shared in an oral presentation on May 12 by Leanne Ward, M.D., Professor of Pediatrics in the Faculty of Medicine at the University of Ottawa (Canada) and the growth data were shared in an oral presentation on May 13 by Hanne B. Hove, M.D., DMSc., Chief Consultant in the Department of Pediatric & Adolescent Medicine at Copenhagen University Hospital Rigshospitalet (Denmark), during ESPE & ESE 2025, the joint congress of the European Society for Paediatric Endocrinology (ESPE) and the European Society of Endocrinology (ESE).

The double-blind placebo-controlled ApproaCH Trial included 84 children with achondroplasia (aged 2-11 years) randomized 2:1 (TransCon CNP:placebo) for 52 weeks, followed by an open-label extension period. At Week 52 of the trial, TransCon CNP demonstrated superiority over placebo in annualized growth velocity (AGV), with a safety and tolerability profile comparable to placebo that included a low rate of injection site reactions, no treatment-related serious adverse events (SAEs), no cases of symptomatic hypotension, no fractures, and no acceleration of bone age versus chronological age.

Analyses also showed that TransCon CNP improved aspects of bone morphometry at Week 52. This included improvement in lower limb alignment and proportional growth, as well as increases in spinal canal dimensions, versus placebo.

“The observed improvements in growth and bone morphometry seen in this trial support our goal to deliver benefits beyond linear growth,” said Aimee Shu, M.D., Executive Vice President of Endocrine & Rare Disease Medical Science and Chief Medical Officer at Ascendis Pharma. “We look forward to continuing to work with our investigators and the broader achondroplasia community to better understand how changing the trajectory of skeletal dysplasia in childhood could potentially reduce future complications associated with this condition, such as pain, impaired mobility, or the need for surgery.”

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 deformities, 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) Ascendis' goal to deliver benefits beyond linear growth, (ii) Ascendis' plans to continue to work with investigators and the broader achondroplasia community to better understand how changing the trajectory of skeletal dysplasia in childhood could potentially reduce future complications associated with the condition, (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; 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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