

## PRESS RELEASE

# Ascendis Submits Marketing Authorisation Application to the European Medicines Agency for TransCon<sup>®</sup> CNP for Treatment of Children with Achondroplasia

**COPENHAGEN, Denmark, October 8, 2025 (GLOBE NEWSWIRE)** – Ascendis Pharma A/S (Nasdaq: ASND) today announced it has submitted a Marketing Authorisation Application (MAA) to the European Medicines Agency (EMA) for TransCon CNP (navepegritide) as a treatment for children with achondroplasia, a rare genetic condition that causes skeletal dysplasia and, for many affected individuals, significant health, physical functioning, and quality of life impacts. TransCon CNP 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, including growth plates and skeletal muscle.

The MAA is based on data from three randomized, double-blind, placebo-controlled clinical trials and up to three years of open-label extension data, including results from the pivotal ApproaCH Trial of children with achondroplasia. In these trials, TransCon CNP has been generally well tolerated, with no discontinuations related to the study drug.

“The achondroplasia community has been clear about its need for early pharmacological interventions that improve the medical, functional, and quality of life complications,” said Aimee Shu, M.D., Executive Vice President of Endocrine & Rare Disease Medical Sciences and Chief Medical Officer at Ascendis Pharma. “We look forward to engaging with the EMA during its review to bring TransCon CNP to patients in the European Union as soon as possible.”

In the United States, TransCon CNP as a treatment for children with achondroplasia is under priority review by the U.S. Food and Drug Administration, with a Prescription Drug User Fee Act (PDUFA) target date of November 30, 2025.

### **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](http://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) TransCon CNP's potential as a new treatment option for children with achondroplasia, (ii) Ascendis' plans to work with the EMA to make TransCon CNP available as quickly as possible, (iii) the PDUFA target date for the U.S. Food and Drug Administration's review of TransCon CNP for the treatment of children with achondroplasia, (iv) Ascendis' ability to apply its TransCon technology platform to make a meaningful difference for patients, and (v) 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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