Ascendis Submits U.S. NDA for TransCon CNP (Navepegritide) for the Treatment of Children with Achondroplasia

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— Data demonstrated multiple clinical benefits beyond linear growth

— NDA supported by data from three randomized, double-blind, placebo-controlled clinical trials in children with achondroplasia, with up to three years of open-label extension data

— MAA in EU on track for submission during Q3 2025

COPENHAGEN, Denmark, March 31, 2025 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND) today announced that it has submitted its New Drug Application (NDA) to the U.S. Food & Drug Administration (FDA) for TransCon CNP (navepegritide) for the treatment of children with achondroplasia. TransCon CNP is an investigational prodrug of C-type natriuretic peptide (CNP) administered once weekly and designed to treat individuals with achondroplasia by providing continuous exposure of active CNP to receptors on tissues throughout the body, including growth plates and skeletal muscle.

The filing 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.

“We are pleased to share clinical data with the FDA demonstrating that, in addition to increased growth velocity, treatment with TransCon CNP was associated with reduced health-related burden, stronger muscle function, and straightening of abnormal leg bowing for the majority of treated children,” said Aimee Shu, M.D., Executive Vice President and Chief Medical Officer at Ascendis Pharma. “In addition to once-weekly administration, these outcomes and a safety and tolerability profile comparable to placebo support TransCon CNP’s potential to be recognized as a best-in-class treatment for achondroplasia.”

Ascendis is on track to submit its Marketing Authorisation Application (MAA) to the European Medicines Agency (EMA) during Q3 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 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’ plan to submit a MAA to the EMA for TransCon CNP in Q3 2025, (ii) TransCon CNP’s potential to be recognized as a best-in-class treatment for achondroplasia, (iii) Ascendis’ ability to apply its TransCon technology platform to make a meaningful difference for patients, and (iv) Ascendis’ application of 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 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. 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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