PRESS RELEASE

FDA Accepts TransCon® CNP NDA for Priority Review

– Once-weekly TransCon CNP demonstrated significantly higher annualized growth velocity, the primary endpoint, compared to placebo

– Multiple benefits beyond linear growth were observed compared to placebo, with a safety and tolerability profile similar to placebo

– The FDA grants Priority Review designation for the evaluation of medicines that, if approved, would provide a significant improvement in the safety or effectiveness of the treatment, prevention, or diagnosis of a serious condition[1]

COPENHAGEN, Denmark, June 2, 2025 -- Ascendis Pharma A/S (Nasdaq: ASND) today announced that the U.S. Food & Drug Administration (FDA) has accepted for priority review its New Drug Application (NDA) for TransCon CNP (navepegritide) for the treatment of children with achondroplasia and has set a Prescription Drug User Fee Act (PDUFA) goal date of November 30, 2025 to complete its review. The FDA also informed Ascendis that they are not currently planning to hold an advisory committee meeting to discuss this application. TransCon CNP is an investigational prodrug of C-type natriuretic peptide (CNP) administered once weekly and designed to treat people living with achondroplasia by providing continuous exposure of active CNP to receptors on tissues throughout the body, including growth plates and skeletal muscle.

"Too many profound medical needs endure for people living with achondroplasia," said Chandler Crews, Founder of The Chandler Project. "Therapies that could address some of the underlying, serious complications of achondroplasia offer welcome potential to improve health outcomes beyond what currently approved therapies and interventions offer."

"TransCon CNP is designed to provide sustained exposure to CNP, resulting in continuous inhibition of the fibroblast growth factor receptor 3 (FGFR3) pathway that is overactive in achondroplasia. In clinical trials, these pharmacological effects have been associated with improvements in lower limb alignment, spinal canal dimensions, muscle strength, and growth compared to placebo," said Janet Legare, M.D., Professor of Pediatrics at the University of Wisconsin School of Medicine and Public Health. "As a practicing physician, I am encouraged to see the FDA designating priority review for TransCon CNP as a potential new treatment option for children with achondroplasia."

"Our clinical trials of TransCon CNP are the first ever to demonstrate improvements beyond linear growth at 52 weeks compared to placebo," said Aimee Shu, M.D., Executive Vice President of Endocrine & Rare Disease Medical Sciences and Chief Medical Officer at Ascendis Pharma. "People living with achondroplasia and their physicians have expressed an urgent need for a meaningful treatment option to

address the complications of achondroplasia. We look forward to working with the FDA during its review to make TransCon CNP available as quickly as possible."

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) the PDUFA goal date for the FDA's review of TransCon CNP for the treatment of children with achondroplasia, (ii) the potential for therapies that could address underlying, serious complications of achondroplasia to improve health outcomes compared to currently approved therapies, (iii) TransCon CNP's potential as a new treatment option for children with achondroplasia, (iv) Ascendis' plans to work with the FDA to make TransCon CNP available as quickly as possible, (v) Ascendis' ability to apply its TransCon technology platform to make a meaningful difference for patients, and (vi) 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.

Investor Contacts:
Sarada Weerasinghe
Ascendis Pharma
ir@ascendispharma.com

Patti Bank
ICR Healthcare
+1 (415) 513-1284
patti.bank@icrhealthcare.com

Media Contact:
Melinda Baker
Ascendis Pharma
media@ascendispharma.com

[1] U.S. Food & Drug Administration website: https://www.fda.gov/patients/fast-track-breakthrough-therapy-accelerated-approval-priority-review/priority-review. Accessed 2 June 2025