

## PRESS RELEASE

### **Ascendis Receives Orphan Drug Exclusivity and Launches YUWIWEL® (Navepegritide) in the United States**

- *YUWIWEL was granted orphan drug exclusivity by U.S. FDA to increase linear growth in pediatric patients 2 years of age and older with achondroplasia with open epiphyses*
- *YUWIWEL is now commercially available in the U.S. with revenue now recognized following approval of first patients and initiation of therapy*

**COPENHAGEN, Denmark, April 6, 2026 (GLOBE NEWSWIRE)** – Ascendis Pharma A/S (Nasdaq: ASND) today announced that YUWIWEL (navepegritide; developed as TransCon® CNP) was granted orphan drug exclusivity by the U.S. Food & Drug Administration (FDA) and that YUWIWEL is now commercially available in the United States. YUWIWEL is the first and only once-weekly treatment approved by FDA to increase linear growth in children 2 years of age and older with achondroplasia with open epiphyses and is the only one to provide continuous systemic exposure to CNP over the weekly dosing interval.

Multiple YUWIWEL prescriptions have already been approved for reimbursement for children in the U.S. enrolled through the Ascendis Signature Access Program® (A.S.A.P.), with revenue now recognized following approval of first patients and initiation of therapy. Ascendis has established A.S.A.P. to provide support to children in the U.S. prescribed YUWIWEL and their caregivers throughout the treatment journey, including a dedicated Patient Access Liaison, programs designed to help navigate each step of the treatment process, and information about co-pay and other assistance programs for eligible patients. The program also provides resources for healthcare providers, including clinical education and assistance with prior authorization and appeals. Information about the A.S.A.P. program for YUWIWEL can be found at <https://yuwiwel.com/support-services/>.

“Our daughter has gained new independence and confidence with her growth, following treatment with YUWIWEL in a clinical trial,” said Brandi, mother of an 11-year-old child living with achondroplasia. “It is satisfying to know that this once-weekly treatment is now available to others in the achondroplasia community seeking pharmacological options to mitigate the potential impacts of this condition.”

“We’re excited to see the FDA grant orphan drug exclusivity for YUWIWEL, acknowledging it as a highly differentiated treatment option,” said Jan Mikkelsen, President and Chief Executive Officer of Ascendis Pharma. “We have now delivered and commercialized our third FDA-approved TransCon product in a row, and remain on track to fulfill our objective of being the leader in Growth Disorders.”

YUWIWEL (navepegritide) received Orphan Drug Designation from the U.S. FDA in February 2019. The FDA grants orphan designation to drugs that are intended for the safe and effective treatment, diagnosis,

or prevention of rare diseases or disorders that affect fewer than 200,000 people in the United States, and that potentially may be safer or more effective than already approved products. Subject to certain exceptions, orphan designation provides a drug developer with certain benefits and incentives, including a seven-year period of U.S. marketing exclusivity upon approval of the product in the orphan-designated indication, waiver of FDA user fees, and tax credits for clinical research. The subsequent orphan drug exclusivity granted to YUWIWEL will run through February 27, 2033.

YUWIWEL is a prodrug of C-type natriuretic peptide (CNP), administered once weekly, designed to provide continuous exposure of active CNP to receptors on tissues throughout the body to counteract the overactive FGFR3 signaling in achondroplasia. It was approved by the FDA on February 27, 2026 under the agency's Accelerated Approval Program. Continued approval for this indication, which was based on an improvement in annualized growth velocity (AGV), may be contingent upon verification and description of clinical benefit in confirmatory trial(s).

The following information is intended for the U.S. audience only:

## **USE AND IMPORTANT SAFETY INFORMATION**

### **What is YUWIWEL?**

YUWIWEL is a prescription medicine used to increase linear growth in children 2 years and older with achondroplasia with open growth plates (epiphyses).

YUWIWEL is approved under accelerated approval based on improvement in annualized growth velocity. Continued approval may be contingent upon verification and description of clinical benefit in confirmatory trials.

## **IMPORTANT SAFETY INFORMATION**

### **What are the possible side effects of YUWIWEL?**

**YUWIWEL may cause serious side effects, including risk of low blood pressure.** If your child experiences a decrease in blood pressure or symptoms of low blood pressure (dizziness, feeling tired, and/or nausea) while being treated with YUWIWEL, call your child's healthcare provider.

**The most common side effects of YUWIWEL include** injection site reactions (redness, itching, skin discoloration, bleeding, swelling, bruising, pain, and blistering).

**Before you give YUWIWEL to your child, tell the healthcare provider about all of your child's medical conditions, including if they:**

- have kidney problems
- are pregnant or plan to become pregnant. It is not known if YUWIWEL will harm the unborn baby
- are breastfeeding or plan to breastfeed. It is not known if YUWIWEL passes into breast milk

**Tell the healthcare provider about all the medicines your child takes**, including prescription and over-the-counter medicines, vitamins, and herbal supplements.

These are not all of the possible side effects of YUVIWEL. Call your doctor for medical advice about side effects. **You are encouraged to report side effects to FDA at 1-(800) FDA-1088 or [www.fda.gov/medwatch](http://www.fda.gov/medwatch). You may also report side effects to Ascendis Pharma at 1-844-442-7236.**

Please see full [Prescribing Information](#) for YUVIWEL in the United States.

### **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, and is associated with an increased risk of muscular, neurological, and cardiorespiratory complications in addition to skeletal dysplasia. Medical complications of achondroplasia can vary from individual to individual and across different stages of life. Throughout infancy and childhood, observed complications include spinal abnormalities, enlarged brain ventricles, impaired muscle strength and reduced stamina, hearing deficits and chronic ear infections, upper airway obstructions, sleep-disordered breathing, hip problems, leg bowing, and chronic pain; some of which persist or worsen in adulthood. These medical complications can affect physical well-being and quality of life, and may be impacted by a range of individual, clinical, and social factors. Some individuals with achondroplasia require multiple procedures and surgeries to address specific functional or anatomical concerns.

### **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 within the meaning of Section 27A of the Securities Act of 1933, as amended, and Section 21E of the Securities Exchange Act of 1934, as amended. Examples of such statements include, but are not limited to, statements relating to (i) the potential for YUVIWEL to address the medical complications of achondroplasia in new ways, (ii) the anticipated timing of the subsequent orphan drug exclusivity granted to YUVIWEL, (iii) Ascendis' ability to apply its TransCon technology platform to make a meaningful difference for patients, and (iv) Ascendis' ability to apply 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, without limitation: dependence on third-party manufacturers, distributors, and service providers for Ascendis' products and product candidates; risks related to regulatory review and approval, including the possibility of delays, requests for additional data or analyses, restrictions or limitations on use, approval with labeling that is more limited than expected, or failure to obtain approval in the United States, European Union, or other jurisdictions; clinical development risks, including that results from ongoing or future trials may not confirm earlier data; unforeseen safety or efficacy findings in development programs or on-market products; manufacturing, supply chain, quality, or logistics issues that could delay development or commercialization; unforeseen expenses related to commercialization of any approved Ascendis products; unforeseen research and development or selling, general and administrative expenses and other costs impacting Ascendis' business generally; market acceptance, pricing, and reimbursement challenges, including payer coverage decisions and health technology assessments; competitive developments, including new or improved therapies; intellectual property protection, freedom-to-operate, and litigation risks; Ascendis' ability to obtain additional funding, if needed, to support its business activities; cybersecurity, data privacy, and information technology disruptions; and the impact of international economic, political, legal, compliance, public health, and business factors, including tariffs, trade policies, currency fluctuations, and geopolitical events. 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 11, 2026, 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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