

PRESS RELEASE

FDA Approves Once-Weekly YUWIWEL[®] (navepegritide) for Children with Achondroplasia Aged 2 Years and Older

- *The first and only approved achondroplasia therapy to provide continuous systemic exposure to CNP over the weekly dosing interval*
 - *Commercial availability expected during early part of Q2 2026*
- *Rare Pediatric Disease Priority Review Voucher granted in connection with approval*
 - *Ascendis to host investor conference call Monday, March 2, at 8:00 am ET*

COPENHAGEN, Denmark, February 27, 2026 (GLOBE NEWSWIRE) – Ascendis Pharma A/S (Nasdaq: ASND) today announced that the U.S. Food & Drug Administration (FDA) has granted approval under the FDA’s Accelerated Approval Program for YUWIWEL[®] (navepegritide; developed as TransCon[®] CNP), the first and only once-weekly treatment indicated to increase linear growth in children 2 years of age and older with achondroplasia with open epiphyses and the only one to provide continuous systemic exposure to CNP over the weekly dosing interval. 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).

Achondroplasia is a rare genetic condition causing skeletal dysplasia and, for many affected individuals, an increased risk of muscular, neurological, and cardiorespiratory complications. 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.

“The approval of once-weekly YUWIWEL is a major step forward in the treatment of children with achondroplasia, giving physicians for the first time the option of prescribing a once-weekly medicine backed by compelling efficacy and excellent tolerability data from three randomized, double-blind, placebo-controlled clinical trials,” said Carlos A. Bacino, MD, FACMG, Professor of Molecular and Human Genetics, Baylor College of Medicine and Texas Children’s Hospital. “My goal is to help children and parents develop care plans tailored to their individual needs and objectives, and I look forward to adding YUWIWEL to my discussions with them.”

“Little People of America, the largest national advocacy and support organization for people with dwarfism, is committed to ensuring that the voices of people with dwarfism remain central in conversations about research and medical options such as YUWIWEL,” said the Board of Directors of Little People of America. “We champion dwarf and disability pride, advocate for inclusion and respect, and foster open dialogue across diverse perspectives. Our goal is to empower individuals and families to make healthcare decisions that reflect their own values and experiences, while pushing for research

efforts and new treatment options such as this that could have the potential to support outcomes that truly matter to our community.”

The FDA based its approval of YUWIWEL on their review of the clinical package for TransCon CNP submitted with the Company’s New Drug Application, which included safety and efficacy data from three randomized, double-blind, placebo-controlled clinical trials and up to three years of open-label extension data. The pivotal ApproaCH Trial data is available in *JAMA Pediatrics*.¹

“We are confident in YUWIWEL’s potential to transform the treatment of achondroplasia and are deeply grateful to patients, clinicians, and advocates for their many contributions to this important milestone,” said Jan Mikkelsen, President and Chief Executive Officer at Ascendis Pharma. “We have listened to advocacy groups for people with dwarfism to ensure we address what the community actually cares about. This reflects our ongoing commitment to pursue outcomes that patient communities have told us are important to them, and gives the achondroplasia community a new way to look at the promise of pharmacological treatment options.”

Ascendis expects to make YUWIWEL available through prescribing physicians in the United States during the early part of the second quarter of 2026. Ascendis plans to offer a suite of patient services for YUWIWEL through its U.S. Ascendis Signature Access Program (A.S.A.P.), including support navigating the treatment journey and financial assistance programs for eligible patients.

With this approval, the FDA also issued a Rare Pediatric Disease Priority Review Voucher (PRV), which confers priority review to a subsequent drug application that would not otherwise qualify for priority review. This program is designed to encourage development of new drugs and biologics for the prevention or treatment of rare pediatric diseases.

Conference Call and Webcast Information

Ascendis will host a call to review the FDA approval of YUWIWEL on Monday, March 2, 2026, at 8:00 am ET. Those who would like to participate may access the live webcast [here](#) or register in advance [here](#). The link to the live webcast and slides will also be available on the Investors & News section of the Ascendis Pharma website at <https://investors.ascendispharma.com>. A replay of the webcast will be available on this section of the Ascendis Pharma website shortly after conclusion of the event for 30 days.

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 an 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 YUWIWEL. Call your doctor for medical advice about side effects. **You are encouraged to report side effects to FDA at 1-800-FDA-1088 or at www.fda.gov/medwatch.** You may also report side effects to Ascendis Pharma at 1-844-442-7236.

Please see full [Prescribing Information](#) for YUWIWEL.

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 quality of life, physical well-being, influenced by a range of clinical, social, and individual 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 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 expected timing for the commercial availability of YUWIWEL, (ii) the continued approval for the indication, (iii) the suite of patient services Ascendis plans to offer for YUWIWEL, (iv) YUWIWEL's potential to transform the treatment of achondroplasia, (v) Ascendis' ability to apply its TransCon technology platform to make a meaningful difference for patients' lives and (vi) Ascendis' use of TransCon to create new and potentially best-in-class therapies. 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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¹ Savarirayan R, McDonnell C, Bacino CA, et al. *JAMA Pediatr* 2026;180(1):18–25. Published online November 17, 2025.
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