

## PRESS RELEASE

### **Children with Achondroplasia Treated with TransCon CNP Showed Continued Improvements in Lower Extremity Alignment at Week 104 of the Pivotal ApproaCH Trial**

- *Data presented during ICCBH 2026 showed that improvements in tibial-femoral angle (TFA), a measure of lower extremity alignment, continued through Week 104*
- *Greater improvements observed in children with preexisting genu varum (leg bowing)*

COPENHAGEN, Denmark, June 30, 2026 – Ascendis Pharma A/S (Nasdaq: ASND) today announced new radiographic data from Week 104 from the completed pivotal ApproaCH Trial of once-weekly TransCon CNP (navepegritide) in children with achondroplasia. In the trial, TransCon CNP-treated children demonstrated continued improvements in lower extremity alignment through up to two years of treatment, including improvements in tibial-femoral angle (TFA). As previously reported, improvements in annualized growth velocity were maintained and ACH-specific height Z-score increased with TransCon CNP treatment through Week 104. The data were presented by Leanne M. Ward, M.D., FRCPC, Professor of Pediatrics at the University of Ottawa and Children's Hospital of Eastern Ontario, during the 12<sup>th</sup> International Conference on Children's Bone Health (ICCBH 2026) held in Montreal, Canada.

“These results show that continuous exposure to active C-type natriuretic peptide (CNP) provided by once-weekly TransCon CNP positively affected skeletal growth and lower limb alignment in children with achondroplasia, with the potential to address serious complications of skeletal dysplasia that contribute to chronic pain, altered mobility, and need for surgical intervention,” said Dr. Ward. “These outcomes reinforce previously reported data showing TransCon CNP’s ability to support healthy and proportional growth, further highlighting its potential to advance pharmacological treatment for achondroplasia.”

#### **ApproaCH Trial Design**

ApproaCH was a randomized, double-blind, placebo-controlled pivotal trial in 84 children with achondroplasia aged 2–11 years, investigating TransCon CNP (100 µg/kg once-weekly) versus placebo for 52 weeks, followed by an open-label extension (OLE) in which all participants received TransCon CNP through Week 104. Radiographic assessments of lower extremity alignment were conducted at baseline, Week 52, and Week 104.

#### **Highlights of Radiographic Assessments at Week 104 of the Pivotal ApproaCH Trial**

- Improvements in TFA and TFA Z-scores continued through Week 104 and were greater in children with baseline TFA  $\geq 5^\circ$ :

- The average TFA in all children treated with TransCon CNP during the double-blind period was 9.1° at baseline, decreasing to 7.7° at Week 52 and 6.9° at Week 104, reflecting a mean absolute change of -2.2 degrees over the two-year treatment period. In the subgroup of children with baseline TFA  $\geq 5^\circ$  (reflecting children with preexisting genu varum), the average TFA was 13.4° at baseline, decreasing to 11.3° at Week 52 and 9.6° at Week 104, reflecting a mean absolute change of -3.8 degrees over the two-year treatment period.
  - The average TFA in children switching from placebo to TransCon CNP treatment at Week 52 was 11.5° at baseline, increasing to 11.8° at Week 52 and decreasing with TransCon CNP treatment to 10.1° at Week 104, reflecting a mean absolute change of -1.7 degrees during the OLE period. In the subgroup of children with baseline TFA  $\geq 5^\circ$ , the average TFA was 18.2° at baseline, increasing to 18.7° at Week 52 and decreasing with treatment to 14.9° at Week 104, reflecting a mean absolute change of -3.8 degrees during the OLE period.
  - In children treated with TransCon CNP in the double-blind period, mean TFA Z-score was 3.62 at baseline and decreased to 3.15 at Week 52 and 2.96 at Week 104, reflecting a mean absolute change of -0.66 over the two-year treatment period. In the subgroup of children with baseline TFA  $\geq 5^\circ$ , mean TFA Z-score was 5.40 at baseline and decreased to 4.67 at Week 52 and 4.36 at Week 104, reflecting a mean absolute change of -1.04 over the two-year treatment period.
  - In children switching from placebo to TransCon CNP at Week 52, mean TFA Z-score was 4.08 at baseline, increased to 4.76 at Week 52, and decreased to 4.28 at Week 104, reflecting a mean absolute change of -0.48 from Week 52 to Week 104. In the subgroup of children with baseline TFA  $\geq 5^\circ$ , mean TFA Z-score was 6.13 at baseline, increased to 7.56 at Week 52, and decreased to 6.35 at Week 104, reflecting a mean absolute change of -1.21 from Week 52 to Week 104.
- Results also showed that fibula-to-tibia length ratio remained stable in the overall clinical trial population during the open-label extension, reflecting proportional growth of the lower leg.

Through up to two years of treatment, TransCon CNP was generally well tolerated, with a low rate of ISRs (all mild), no symptomatic hypotension, and no acceleration of bone age. Most adverse events in TransCon CNP-treated children were mild or moderate, with none leading to treatment discontinuation or withdrawal from the trial.

A slide presentation with these data will be made available on the Investors & News section of the Ascendis Pharma website: <https://investors.ascendispharma.com>.

### **About TransCon CNP**

TransCon CNP 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. In February 2026, TransCon CNP was approved by the U.S. Food & Drug Administration (FDA) under the trade name YUVIWEL<sup>®</sup> to increase linear growth in pediatric patients 2 years of age and older with achondroplasia with open epiphyses. Ascendis

Pharma's Marketing Authorisation Application for YUVIWEL is under review by the European Medicines Agency, with a regulatory decision anticipated in the fourth quarter of 2026.

### **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) TransCon CNP's ability to address serious complications of skeletal dysplasia, including those that contribute to chronic pain, altered mobility, and need for surgical intervention, (ii) TransCon CNP's ability to deliver healthy and proportional growth and its potential to advance pharmacological treatment of achondroplasia, (iii) the anticipated timing of a regulatory decision by the European Medicines Agency regarding YUVIWEL, (iv) Ascendis' ability to apply its TransCon technology platform to make a meaningful difference for patients and (v) 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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