

PRESS RELEASE

Significant Health and Quality of Life Improvements Achieved in Children with Achondroplasia Treated for One Year with TransCon™ CNP (Navepegritide) at 100 µg/kg/week

- *TransCon CNP is the first investigational product to demonstrate improvements in health-related quality of life and disease impacts in children with achondroplasia*

COPENHAGEN, Denmark, December 20, 2023 (GLOBE NEWSWIRE) – Ascendis Pharma A/S (Nasdaq: ASND) today announced new analyses from the blinded and ongoing open-label extension (OLE) portions of ACcomplisH, the Company’s Phase 2 randomized, double-blind, placebo-controlled, dose-escalation trial of TransCon CNP in children ages 2-10 years with achondroplasia. In the trial, all 57 patients have now completed one year of treatment with TransCon CNP (navepegritide) at 100 µg/kg/week, the dose agreed with regulatory agencies for the active arm in the pivotal ApproaCH Trial.

Ascendis analyzed available data for patients who only received TransCon CNP at the 100 µg/kg/week dose in either blinded or OLE part and were treated for one year (n=19), compared to those administered placebo for one year (n=15). Results showed that these TransCon CNP treated patients (data available for 9-16 patients) showed significant improvements in health-related quality of life and disease impacts compared to those receiving placebo (data available for 5-13 patients).

Assessments were performed with the SF-10 and Achondroplasia Child Experience Measure (ACEM), with statistically significant improved outcome in TransCon CNP treated versus placebo for:

- SF-10 Physical Summary (p=0.002, ages 5 years and older)
- ACEM Daily Living Function (p=0.047)
- ACEM Emotional Well-being (p=0.045)

The 46 children switching from placebo or a lower dose of TransCon CNP to the 100 µg/kg/week dose in the OLE demonstrated improved growth after one year of treatment, similar to the growth benefits seen in the 11 children treated with 100 µg/kg/week in the one-year randomized, double-blind period of ACcomplisH.

“These data support our aspirations for TransCon CNP, the first investigational product to go beyond height by improving the signs and symptoms of achondroplasia. We believe that the observed treatment benefits are likely contributing to the continued strong patient retention in our trials,” said Jan Mikkelsen, President and Chief Executive Officer at Ascendis Pharma. “We remain confident that TransCon CNP has the potential to deliver meaningful benefits to children with achondroplasia, and we will explore effects of TransCon CNP in adults with achondroplasia in the years ahead.”

TransCon CNP (navepegritide) is an investigational prodrug of CNP, administered once weekly and designed to provide sustained release of active CNP supporting continuous exposure for the treatment of children with achondroplasia.

Further details of the results will be shared early next year.

About Ascendis Pharma A/S

Ascendis Pharma is applying its innovative TransCon technology platform to build a leading, fully integrated biopharma company focused on making a meaningful difference in patients' lives. Guided by its core values of patients, science and passion, Ascendis uses its TransCon technologies to create new and potentially best-in-class therapies. Ascendis is headquartered in Copenhagen, Denmark and has additional facilities in Germany (Heidelberg and Munich) and the United States (Palo Alto and Redwood City, California, and Princeton, New Jersey). 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) TransCon CNP's ability to go beyond height by improving the signs and symptoms of achondroplasia, (ii) Ascendis' belief that the treatment benefits of TransCon CNP contributed to the continued strong patient retention in Ascendis' trials, (iii) TransCon CNP's ability to deliver meaningful benefits to children with achondroplasia, (iv) Ascendis' intent to explore the effects of TransCon CNP in adults with achondroplasia in the years ahead, (v) TransCon CNP's ability to provide sustained release of active CNP thereby supporting continuous exposure of CNP for the treatment of children with achondroplasia, (vi) the timing for sharing further details of the results of the ACcomplisH Trial, (vii) Ascendis' ability to apply its TransCon technology platform to build a leading, fully integrated global biopharma company, and (viii) Ascendis' use of its TransCon technologies 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 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 inflation, the effects on its business from the worldwide COVID-19 pandemic and ongoing conflicts such as that in the region surrounding Ukraine and Russia. 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 16, 2023 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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