Data from Phase 2 ACcomplisH Trial of TransCon CNP in Children with Achondroplasia Presented at ICCBH 2024

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• Data demonstrate significant improvements in well-being and physical functioning compared to placebo in patients treated for 1 year with TransCon CNP at the pivotal 100µg/kg/week dose

• Improvements could not be explained by changes in linear growth only, supporting a potential additional direct treatment effect of TransCon CNP beyond linear growth

COPENHAGEN, Denmark, June 24, 2024 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND) today announced the oral presentation of data from its Phase 2 ACcomplisH Trial of TransCon CNP (navepegritide) in children aged 2 to 10 years old with achondroplasia by Ravi Savarirayan, M.D., Murdoch Children's Research Center (Australia), during ICCBH 2024, the International Conference on Children's Bone Health being held in Salzburg, Austria.

The data showed that patients treated for one year at the pivotal 100µg/kg/week dose demonstrated significant improvements in well-being and physical functioning compared to placebo. These improvements could not be explained by changes in linear growth only, supporting a potential additional direct treatment effect of TransCon CNP beyond linear growth.

"Once-weekly TransCon CNP has demonstrated a positive impact on linear growth with a favorable safety profile, as well as benefits beyond growth," said Dr. Savarirayan. "As the first investigational pharmaceutical treatment for achondroplasia to demonstrate statistically significant improvements compared to placebo in health-related quality of life measures, TransCon CNP at the pivotal dose has potential to meet the need for a treatment addressing the health and quality-of-life complications of this condition."

TransCon CNP is an investigational prodrug of C-type natriuretic peptide (CNP) administered once weekly, designed to provide sustained release of active CNP. ACcomplisH is a Phase 2, multicenter, randomized, double-blind, placebo-controlled, dose-escalation trial of once-weekly TransCon CNP versus placebo in 57 children with achondroplasia (aged 2-10 years old). Patients were randomized 3:1 to receive TransCon CNP across 4 dose-escalation cohorts or placebo for 52 weeks, after which participants could receive TransCon CNP in an ongoing open-label extension at the 100µg/kg/week dose. Health-related quality of life assessments compared 52-week results from patients whose initial TransCon CNP dose was 100µg/kg/week (the pivotal trial dose) with results from the pooled placebo group in the randomized period. Of the 57 participants, 56 continue with open-label treatment.

Statistically significant improvements were observed for various exploratory endpoints including Daily Living Functioning (p=0.047; n=16; 13 [treated;placebo]) and Emotional Well-Being (p=0.045; n=13;9) domains of the Achondroplasia Child Experience Measure-Impact (ACEM) assessment, and the SF-10 Physical Summary among participants 5 years of age or older (p=0.002; n=9;5). Growth across the full trial population (n=57) on TransCon CNP at the 100µg/kg/week dose for 52 weeks was consistent with results from this dose cohort during the randomized period.

No new safety signals were observed, and no serious adverse events related to the study drug were reported. Most treatment-emergent adverse events (TEAEs) were Grade 1-2, with a low frequency of injection site reactions.

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 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) improvements in well-being and physical functioning supporting a potential additional direct treatment effect of TransCon CNP, (ii) TransCon CNP's potential to meet the need for a treatment addressing the health and quality-of-life complications of achondroplasia, (iii) Ascendis' ability to apply its TransCon technology platform to build a leading, fully integrated biopharma company, and (iv) 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. 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 7, 2024, 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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