PRESS RELEASE

# Once-Weekly TransCon™ CNP Achieved Primary Efficacy Objective, with Superiority at 100 µg/kg/week, in Ascendis Pharma's Phase 2 ACcomplisH Trial of Children with Achondroplasia Aged 2 to 10 Years

- *Data demonstrated that once-weekly TransCon CNP has the potential to meet patient and caregiver needs for a safe, effective, tolerable and convenient treatment*
- *The primary endpoint, annualized height velocity (AHV) at Week 52, demonstrated superiority of TransCon CNP at 100 µg/kg/week compared to placebo (p=0.0218)*
- *TransCon CNP was generally well tolerated with low frequency of injection site reactions; all 57 randomized children continued, with the longest treatment duration beyond two years*
- *Data showed robust and consistent results in prespecified analyses across age groups and dose levels, supporting continued development at the selected dose of 100 µg/kg/week*
- *Ascendis to host conference call and webcast Monday, November 14 at 8:00 a.m. Eastern time*

**COPENHAGEN, Denmark, November 14, 2022 (GLOBE NEWSWIRE) –** Ascendis Pharma A/S (Nasdaq: ASND) today announced positive topline results from the ACcomplisH Trial, its Phase 2 randomized, double-blind, placebo-controlled, dose-escalation trial evaluating the safety and efficacy of once-weekly TransCon CNP compared to placebo in children with achondroplasia aged 2 to 10 years old. TransCon CNP is an investigational long-acting prodrug of C-type natriuretic peptide (CNP), designed to provide continuous exposure of CNP at safe, therapeutic levels via a single, weekly subcutaneous dose.

The ACcomplisH Trial evaluated 57 children with achondroplasia aged 2 to 10 years old, randomized in a 3:1 ratio to receive either sequential ascending doses of once-weekly TransCon CNP or placebo for 52 weeks. All 57 randomized children completed the blinded portion of ACcomplisH and are currently continuing in the open label extension (OLE) at the 100 µg/kg/week dose.

The trial met its primary objectives, demonstrating that TransCon CNP at 100 µg/kg/week was superior to placebo on the primary efficacy endpoint of AHV at 52 weeks. A slide presentation with these data can be found on the Investor Relations & News section of the Ascendis Pharma website: https://investors.ascendispharma.com.

Key data include:

| **TransCon CNP Dose Group** (n) | **AHV (cm/year)**<br>LS Mean [95% CI] | **p-value**<br>(TransCon CNP vs. Pooled Placebo) |
|---|---|---|
| **6 µg/kg/week** (n=10) | **4.09**<br>[3.34, 4.84] | 0.6004 |
| **20 µg/kg/week** (n=11) | **4.52**,<br>[3.82, 5.22] | 0.7022 |
| **50 µg/kg/week** (n=10) | **5.16**<br>[4.43, 5.90] | 0.0849 |
| **100 µg/kg/week (**n=11) | **5.42**<br>[4.74, 6.11] | 0.0218 |
| **Pooled Placebo** (n=15) | **4.35**<br>[3.75, 4.94] | NA |

Additional highlights:

- TransCon CNP demonstrated a consistent dose-response in AHV across the four dose groups.
- Mean improvements in AHV for TransCon CNP-treated patients were consistent across age groups <5 years and ≥5 years, with dose response established.
- TransCon CNP at 100 µg/kg/week demonstrated superiority in change in ACH-specific height SDS compared to placebo.
- TransCon CNP was generally safe and well tolerated, with no discontinuations.
- No serious AEs (SAEs) related to treatment were reported; two unrelated SAEs were reported.
- Injections were generally well tolerated with low frequency of injection site reactions (ISRs):
    - 11 mild ISRs (in 8 patients) out of >2,000 injections.
- Patients treated ≥6 months at 100 µg/kg/week in the blinded or OLE period demonstrated a consistent and sustained response, with mean AHV of 5.39 cm/year (n=40).

"Development of a drug with potential to be both an accepted and a preferred treatment option for children with achondroplasia and their caregivers has a high bar related to safety, efficacy, tolerability and convenience," said Aimee Shu, M.D., Vice President, Clinical Development, Endocrine & Rare Diseases at Ascendis Pharma. "We are excited to see once-weekly TransCon CNP demonstrated statistically superior growth compared to placebo at the 100 µg/kg/week dose level, with safety and tolerability results supporting further development in children with achondroplasia."

**Conference Call and Webcast Information**

Ascendis Pharma will host a conference call and webcast Monday, November 14, at 8:00 a.m. Eastern Time (ET) to discuss the topline ACcomplisH Trial results.

Those who would like to listen to the live webcast can access it through the following link here. To access the live teleconference, register online here. Participants are encouraged to register at least 15 minutes prior to the call.

A replay of the webcast will be available on the Investors & News section of the Ascendis Pharma website at https://investors.ascendispharma.com shortly after conclusion of the event for 30 days.

**About Ascendis Pharma A/S**

Ascendis Pharma is applying its innovative TransCon platform to build a leading, fully integrated, global biopharma company focused on making a meaningful difference in patients' lives. Guided by its core values of patients, science and passion, the company uses its TransCon technologies to create new and potentially best-in-class therapies. Ascendis is headquartered in Copenhagen, Denmark, and has additional facilities in Heidelberg and Berlin, Germany; Palo Alto and Redwood City, California; and Princeton, New Jersey. Please visit www.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 provide continuous exposure of CNP at safe, therapeutic levels via a single, weekly subcutaneous dose, (ii) TransCon CNP's potential to meet patient and caregiver needs for a safe, effective, tolerable and convenient treatment and to become an accepted and preferred treatment method, (iii) Ascendis' plans to advance TransCon CNP in its new Phase 2b ApproaCH trial and to bring TransCon CNP to patients as fast as possible, (iv) Ascendis' use of its TransCon technologies and platform to create new and potentially best-in-class therapies and build a leading, fully integrated, global biopharma company and (v) 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 its development programs or on-market products; unforeseen expenses related to commercialization of any approved Ascendis products; 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, and the effects on its business from the worldwide COVID-19 pandemic and the ongoing conflict 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 March 2, 2022 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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