

Ascendis Presents TransCon[™] PTH Data at the American Thyroid Association Annual Meeting

 At Week 26, 81% of patients in Phase 3 PaTHway Trial with chronic post-surgical hypoparathyroidism, the most common cause of this disease, achieved independence from conventional therapy while maintaining normocalcemia following treatment with TransCon PTH

- Post-surgical patients treated with TransCon PTH self-reported significant improvements in disease-related symptoms, physical functioning, and daily life compared to those on placebo
- Results for post-surgical patients (n=70) comparable to overall PaTHway Trial population (n=82)

COPENHAGEN, Denmark, October 2, 2023 (**GLOBE NEWSWIRE**) – Ascendis Pharma A/S (Nasdaq: ASND) presented a poster with results of a sub-analysis of its Phase 3 PaTHway clinical trial data during the recent American Thyroid Association (ATA) annual meeting, showing that 81% of adults with chronic post-surgical hypoparathyroidism (n = 70) treated with TransCon PTH (palopegteriparatide) achieved independence from conventional calcium and active vitamin D therapy while maintaining normal serum calcium levels during the 26-week blinded portion of the trial, compared to 6% taking placebo. The results were comparable to those from the overall PaTHway Trial population (N = 82), which demonstrated consistency in improved outcomes with TransCon PTH treatment compared to conventional therapy across disease etiologies.

TransCon PTH is an investigational prodrug with sustained release of active parathyroid hormone (PTH [1-34]) administered once daily. TransCon PTH (palopegteriparatide) has received a positive CHMP opinion recommending approval in the European Union and is in development in the United States, and Japan for the treatment of adults with hypoparathyroidism.

"We are pleased to share with thyroid surgeons and thyroidologists attending ATA this data demonstrating the safety and benefits of TransCon PTH seen in clinical trial patients whose disease arose following neck surgeries," said Aimee Shu, M.D., Vice President of Clinical Development, Endocrinology Medical Sciences at Ascendis Pharma. "Our goal is to provide a treatment option that can help patients overcome the serious health and quality of life burdens associated with hypoparathyroidism, regardless of its cause. We will continue to advance TransCon PTH globally as a candidate to address the urgent needs of this patient community."

PaTHway is a Phase 3 trial with a completed 26-week randomized, double-blind, placebocontrolled period, followed by an ongoing 156-week open-label extension period, designed to evaluate the efficacy, safety, and tolerability of TransCon PTH in adults with hypoparathyroidism. A total of 84



patients were randomized 3:1 to treatment with TransCon PTH (n = 63) or placebo (n = 21), both coadministered with conventional therapy. The multi-component primary efficacy endpoint of PaTHway was defined as normocalcemia (a normal concentration of calcium in the blood; 8.3-10.6 mg/dL), independence from conventional therapy (no active vitamin D and $\leq 600 \text{ mg/day}$ calcium), and no TransCon PTH dose change within the 4 weeks prior to Week 26. Key secondary endpoints included patient-reported assessments of disease impact using the Hypoparathyroidism Patient Experience Scale (HPES) and the 36-Item Short Form Survey (SF-36 version 2). Of the 84 patients randomized, 82 received study drug. Of those, the 70 with post-surgical hypoparathyroidism (52 TransCon PTH, 18 placebo) were included in the sub-analysis population.

PaTHway Trial Sub-Analysis Highlights

Across multiple evaluated parameters in participants with post-surgical hypoparathyroidism, treatment with TransCon PTH enabled independence from conventional therapy (no active vitamin D and ≤ 600 mg/day calcium); improvement of hypoparathyroidism-specific symptoms and their impact on daily life and physical functioning; and normalization of mean 24-hour urine calcium excretion. This includes:

- At Week 26, 81% of patients with postsurgical hypoparathyroidism treated with TransCon PTH in the trial achieved independence from conventional therapy while maintaining normocalcemia compared to 6% treated with placebo.
- At Week 26, 81% (42/52) of patients with chronic post-surgical hypoparathyroidism treated with TransCon PTH demonstrated significant improvement compared to those on placebo in disease-related symptoms and the impact of hypoparathyroidism on physical functioning and daily life (all p < 0.01).
- Health-related quality of life, as measured by the SF-36 physical functioning subscale score, improved significantly with TransCon PTH treatment compared to placebo in participants with post-surgical hypoparathyroidism (p = 0.0063).
- From baseline to Week 26, patients with postsurgical hypoparathyroidism treated with TransCon PTH in the trial showed a normalization and greater reduction in urine calcium excretion than those on placebo.
- Treatment with TransCon PTH was generally safe and well-tolerated. Most treatment-emergent adverse events (TEAEs) were mild or moderate, and no patients discontinued study treatment or the trial due to a treatment-related TEAE.

About Ascendis Pharma A/S

Ascendis Pharma is applying its innovative platform technology 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, 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 Germany (Heidelberg and Munich) and the United States (Palo Alto and Redwood City, California, and Princeton, New Jersey). Please visit <u>ascendispharma.com</u> 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) the potential value of TransCon PTH, if approved, as a treatment option for adults with hypoparathyroidism; (ii) the ability of TransCon PTH to provide stable PTH levels within the physiological range 24 hours/day; (iii) Ascendis' ability to achieve its goal of providing a treatment option that can help patients overcome the serious health and quality of life burdens seen with hypoparathyroidism; (iv) Ascendis' plans to continue to advance TransCon PTH globally as a candidate to address the needs of the hypoparathyroidism patient community; (v) the potential approval of TransCon PTH in the European Union; (vi) Ascendis' ability to apply its TransCon platform technology to build a leading, fully integrated, global biopharma company; and (vii) 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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