

## PRESS RELEASE

### **Ascendis Pharma Submits Marketing Authorisation Application to the European Medicines Agency for TransCon™ PTH in Adult Patients with Hypoparathyroidism**

- *TransCon PTH is designed to be the first parathyroid hormone replacement therapy, addressing an area of major unmet medical need*

**COPENHAGEN, Denmark, November 14, 2022 (GLOBE NEWSWIRE)** – Ascendis Pharma A/S (Nasdaq: ASND) today announced it has submitted a Marketing Authorisation Application (MAA) to the European Medicines Agency (EMA) for TransCon PTH (palopegteriparatide) in adult patients with hypoparathyroidism. TransCon PTH is an investigational prodrug designed to restore parathyroid hormone (PTH [1-34]) to physiological levels over 24 hours in adult patients with hypoparathyroidism. Hypoparathyroidism is a rare disease that affects an estimated 85,000-223,000 patients in Europe, causing significant health and economic impacts on individual patients, families, and society.

“With a growing body of data showing durable, meaningful clinical and quality of life responses in the majority of treated patients, we believe TransCon PTH could, if approved, become the first treatment to address the underlying cause of hypoparathyroidism,” said Birgitte Volck, M.D., Ph.D., Senior Vice President, Head of Clinical Development & Medical Affairs, Endocrinology Rare Diseases, at Ascendis Pharma. “Our MAA submission is an important step in advancing our goal to bring TransCon PTH, if approved, to the substantial patient population in Europe who seek a treatment to address the short-term symptoms and long-term complications of their disease and support a normal daily life.”

The MAA is based on data from the global Phase 3 PaTHway Trial and the Phase 2 PaTH Forward Trial of TransCon PTH in adult patients with hypoparathyroidism. In the open-label portion of the Phase 2 trial, which includes patients treated beyond two years, 57 out of 59 patients continue. And in the open-label portion of the Phase 3 trial, 77 out of 79 patients continue, both as of September 30, 2022. In these studies, TransCon PTH has been generally well tolerated, with no discontinuations related to study drug.

The Company’s New Drug Application for TransCon PTH for adult patients with hypoparathyroidism is under Priority Review by the U.S. Food and Drug Administration, with a PDUFA target action date of April 30, 2023. TransCon PTH has been granted orphan designation in the United States and European Union.

#### **About Hypoparathyroidism** <sup>1, 2, 3, 4, 5, 6</sup>

Hypoparathyroidism is a rare endocrine disorder characterized by insufficient levels of parathyroid hormone (PTH), resulting in low calcium and elevated phosphate levels in the blood. Hypoparathyroidism

affects approximately 200,000 patients in the United States, Europe, and Japan, most of whom develop the condition following damage to or accidental removal of the parathyroid glands during thyroid surgery. Conventional therapy with calcium supplements and active vitamin D (also called calcitriol) does not effectively address the short-term symptoms, long-term complications, or quality-of-life impacts of hypoparathyroidism.

Short-term symptoms include weakness, severe muscle cramps (tetany), abnormal sensations such as tingling, burning and numbness (paresthesia), memory loss, impaired judgment, and headache. Patients often experience decreased quality of life, and, over the long term, this complex disorder can increase risk of major complications, such as calcium deposits in the brain, blood vessels, eye, and other soft tissues – including the kidneys, which can lead to impaired renal function.

Hypoparathyroidism remains among the few hormonal insufficiency states without a replacement therapy to restore the missing hormone at physiologic levels. Conventional therapy with high doses of calcium and active vitamin D does not fully control the disease or address its underlying cause and may contribute to risk of renal disease. Patients with hypoparathyroidism have an estimated 4- to 8-fold greater risk of renal disease compared to healthy populations. Hypoparathyroidism is also associated with a 2-fold increased risk of depression or bipolar disorder compared to healthy populations.

#### **About Ascendis Pharma A/S**

Ascendis Pharma is applying its innovative TransCon technology 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](http://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 PTH's ability to restore parathyroid hormone (PTH [1-34]) to physiological levels over 24 hours in adult patients with hypoparathyroidism and help restore patients their normal daily lives, (ii) TransCon PTH's potential, if approved, to become the first parathyroid hormone replacement therapy and treatment to address the underlying cause of hypoparathyroidism, (iii) Ascendis' ability to make its TransCon PTH product candidate available to a substantial population in Europe, (iv) Ascendis' expectations regarding the PDUFA target action date of April 30, 2023, (v) Ascendis' ability to apply its TransCon technology to build a leading, fully integrated global biopharmaceutical company, and (vi) 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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<sup>1</sup> Mannstadt M, et al. *Nature Reviews* 2017, 3: 17055

<sup>2</sup> Ascendis Pharma HP Patient Experience Research.

<sup>3</sup> Hadker N, et al. *Endo Pract.* 2014, 20(7);671-679

<sup>4</sup> Powers J, et al. *J Bone Miner Res* 2013, 28: 2570-2576.

<sup>5</sup> Mitchell DM, et al. *J Clin Endocrinol Metab* 2012, 97(12): 4507-4514

<sup>6</sup> Underbjerg L, et al. *J Bone Miner Res* 2013, 28: 2277-2285