

PRESS RELEASE

Online Enrollment Now Open for Physicians Requesting Expanded Access to Ascendis Pharma's TransCon™ PTH for Eligible U.S. Adult Patients with Hypoparathyroidism

- *The Expanded Access Program (EAP) allows physicians to request access to TransCon PTH, the company's investigational parathyroid hormone replacement therapy, for eligible patients in the United States*
- *TransCon PTH is under Priority Review by the FDA, with a target PDUFA date of April 30, 2023*

COPENHAGEN, Denmark, January 4, 2023 (GLOBE NEWSWIRE) – Ascendis Pharma A/S (Nasdaq: ASND) today announced that the online portal is now open for physicians wanting to request access to TransCon PTH (palopegteriparatide), the Company's investigational parathyroid hormone replacement therapy, through the U.S. Expanded Access Program (EAP). Requests for access must be made by the patient's treating physician through the program administrator, Clinigen Healthcare LTD, at [Palopegteriparatide - Clinigen \(clinigengroup.com\)](https://www.clinigengroup.com), or by searching "palopegteriparatide" on the Clinigen Direct homepage: <https://www.clinigengroup.com/direct/en/>.

To qualify, patients must be adults diagnosed with hypoparathyroidism who live in the U.S., have prior PTH treatment experience, and meet other criteria outlined below and in the full treatment protocol.

Inclusion Criteria:

- Diagnosis of hypoparathyroidism;
- Patients with previous PTH-treatment experience;
- Serum albumin-adjusted calcium level ≥ 7.8 mg/dL and 25(OH) vitamin D in the normal range within 2 weeks before first dose;
- Male or female at least 18 years of age;
- Body mass index (BMI) 17–40 kg/m²; and
- Be willing and able to give written informed consent by signing an Institutional Review Board (IRB)-approved Informed Consent Form (ICF).

Exclusion Criteria:

- Diagnosis of pseudohypoparathyroidism;
- Currently enrolled in an investigational drug or device study or has used an investigational drug or device within 30 days or 5 half-lives (whichever is longer) of Day 1;

- Severe renal impairment (estimated glomerular filtration rate <30 mL/min/1.73m²);
- Increased risk for osteosarcoma, such as those with Paget's disease of bone or unexplained elevations of alkaline phosphatase, hereditary disorders predisposing to osteosarcoma, or with a prior history of substantial external beam or implant radiation therapy involving the skeleton;
- Active malignancy within past 2 years excluding successfully resected thyroid carcinoma and non-melanoma skin cancer;
- Severe or decompensated cardiac disease within 26 weeks, including but not limited to class IV or Stage D heart failure, unstable angina, myocardial infarction or uncontrolled arrhythmias;
- Pregnant or lactating females, or females intending to become pregnant; or
- Known allergy and/or sensitivity to palopegteriparatide or its excipients or prior PTH therapy.

“We are pleased to have worked with the FDA to make TransCon PTH available for eligible patients in the U.S.,” said Edward Trott, M.D., Vice President, Global Medical Affairs at Ascendis Pharma. “Recognizing the needs expressed by the hypopara community, we will continue to work with the FDA and others to make TransCon PTH, if approved, more broadly available as quickly as possible.”

Clinigen representatives are also available to U.S. healthcare professionals by phone toll-free at 1 (877) 768-4303.

Full details of Ascendis Pharma’s TransCon PTH US EAP program (NCT05654701) are listed on <https://clinicaltrials.gov/ct2/show/NCT05654701> and navigator.reaganudall.org.

TransCon PTH (palopegteriparatide) is an investigational prodrug in development for adult patients with hypoparathyroidism. The safety and efficacy of TransCon PTH have not been established and TransCon PTH is not currently approved by the FDA. TransCon PTH is currently under Priority Review by the FDA, with a Prescription Drug User Fee Act (PDUFA) target action date of April 30, 2023.

About Expanded Access

Expanded access is per FDA regulation a potential pathway for a patient with a serious or immediately life-threatening disease or condition to gain access to an investigational medical product (drug, biologic, or medical device) for treatment outside of clinical trials when no comparable or satisfactory alternative therapy options are available. Please visit the [FDA’s Expanded Access website](#) for more information.

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 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) Ascendis' PDUFA date of April 30, 2023 with respect to the FDA's Priority Review of TransCon PTH, (ii) Ascendis' ability to make TransCon PTH more broadly available as quickly as possible, (iii) Ascendis' ability to apply its platform technology to build a leading, fully integrated global 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 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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