Ascendis Pharma A/S Receives Orphan Designation for TransCon™ PTH for Treatment of Hypoparathyroidism in Europe

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COPENHAGEN, Denmark, Oct. 23, 2020 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND), a biopharmaceutical company that utilizes its innovative TransCon technologies to address unmet medical needs, today announced the European Commission (EC) has granted Orphan Designation to TransCon PTH for the treatment of hypoparathyroidism (HP).

TransCon PTH is an investigational once-daily long-acting prodrug of parathyroid hormone (PTH) in development for the treatment of adult HP, which is designed to replace PTH at physiologic levels for 24 hours each day to address both the short-term symptoms and long-term complications of HP. Currently, the standard of care for HP is calcium and active vitamin D (calcitriol or alfacalcidol).[1]

"This designation from the EC for TransCon PTH acknowledges the need in Europe for a hormone replacement therapy that can improve symptoms, long-term complications and quality of life for people with HP," said Dana Pizzuti, M.D., Ascendis Pharma's Senior Vice President of Development Operations. "We recently filed an IND amendment for the phase 3 PaTHway Trial of TransCon PTH based on feedback from U.S. and European regulatory authorities and will submit European clinical trial applications later this year. These important steps underscore our commitment to developing novel therapies that address unmet medical needs and improve peoples' lives worldwide."

Orphan Designation is granted to therapies aimed at the treatment, prevention or diagnosis of a disease that is life-threatening or chronically debilitating, affects no more than five in 10,000 persons in the European Union (EU) and for which no satisfactory therapy is available. The medicine must also be expected to provide significant benefit to those affected by the condition. Orphan medicines have 10 years of market exclusivity after they receive marketing authorization in the EU and the designation supports future reimbursement and access to new therapies. Under certain conditions, market exclusivity for pediatric indications may be extended for an additional two years.

TransCon PTH was granted Orphan Drug Designation (ODD) from the U.S. Food and Drug Administration in June 2018.

About Hypoparathyroidism (HP)[2,3,4,5,6,7]

Hypoparathyroidism (HP) is a rare endocrine disorder characterized by insufficient levels of parathyroid hormone (PTH), resulting in low calcium and elevated phosphate levels in the blood. HP affects approximately 200,000 patients in the United States, Europe, Japan and South Korea, the majority of whom develop the condition following damage or accidental removal of the parathyroid glands during thyroid surgery. Patients often experience decreased quality of life. In the short term, symptoms include weakness, severe muscle cramps (tetany), abnormal sensations such as tingling, burning and numbness (paresthesia), memory loss, impaired judgment and headache. Over the long term, this complex disorder can increase risk of major complications, such as extraskeletal calcium depositions occurring within the brain, lens of the eye, and kidneys, which can lead to impaired renal function.

Until recently, HP remained among the few hormonal insufficiency states not treated by replacement of the missing hormone. Standard of care with active vitamin D analogs and calcium supplementation do not fully control the disease and may contribute to risk of renal disease. As a result, patients with HP have an estimated 4-fold to 8-fold greater risk of renal disease compared to healthy controls.

About TransCon™ Technology Platform

TransCon refers to "transient conjugation." The proprietary TransCon platform is an innovative technology to create new therapies that are designed to potentially optimize therapeutic effect, including efficacy, safety and dosing frequency. TransCon molecules have three components: an unmodified parent drug, an inert carrier that protects it, and a linker that temporarily binds the two. When bound, the carrier inactivates and shields the parent drug from clearance. When injected into the body, physiologic conditions (e.g., pH and temperature) initiate the release of the active, unmodified parent drug in a predictable manner. Because the parent drug is unmodified, its original mode of action is expected to be maintained. TransCon technologies can be applied broadly to a protein, peptide or small molecule in multiple therapeutic areas, and can be used systemically or locally.

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 utilizes its TransCon™ technologies to create new and potentially best-in-class therapies.

Ascendis Pharma currently has a pipeline of three independent endocrinology rare disease product candidates in clinical development and is advancing oncology as its second therapeutic area of focus. The company continues to expand into additional therapeutic areas to address unmet patient needs.

Ascendis is headquartered in Copenhagen, Denmark, with additional offices in Heidelberg and Berlin, Germany, and in Palo Alto and Redwood City, California.

For more information, please visit **www.ascendispharma.com**.

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' plans to begin its phase 3 PaTHway Trial with TransCon PTH this year, (ii) Ascendis' ability to apply its platform technology to build a leading, fully integrated biopharma company, (iii) Ascendis' product pipeline and expansion into additional therapeutic areas, and (iv) Ascendis' expectations regarding its ability to utilize 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: unforeseen safety or efficacy results in its oncology programs, TransCon hGH, TransCon PTH and TransCon CNP or other development programs; unforeseen expenses related to the development and potential commercialization of its oncology programs, TransCon hGH, TransCon PTH and TransCon CNP or other development programs, selling, general and administrative expenses, other research and development expenses and Ascendis' business generally; delays in the development of its oncology programs, TransCon hGH, TransCon PTH and TransCon CNP or other development programs related to manufacturing, regulatory requirements, speed of patient recruitment or other unforeseen delays; dependence on third party manufacturers to supply study drug for planned clinical studies; Ascendis' ability to obtain additional funding, if needed, to support its business activities and the effects on its business of the worldwide COVID-19 pandemic. 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' prospectus supplement filed on July 9, 2020 and Ascendis' current and future reports filed with, or submitted to, the U.S. Securities and Exchange Commission (SEC), including its Annual Report on Form 20-F filed with the SEC on April 3, 2020. Forward-looking statements do not reflect the potential impact of any future in-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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Investor contact:
Patti Bank
Westwicke Partners
(415) 513-1284
patti.bank@westwicke.com
ir@ascendispharma.com

Media contact:
Ron Rogers
Ascendis Pharma
(650) 650-507-5208
rrs@ascendispharma.com

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