Ascendis Pharma Announces Orphan Drug Designation Granted for TransCon PTH as Treatment for Hypoparathyroidism

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COPENHAGEN, Denmark, June 06, 2018 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq:ASND), a biopharmaceutical company that utilizes its innovative TransCon technology to address significant unmet medical needs, today announced that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) to TransCon PTH, a long-acting prodrug of parathyroid hormone (PTH) in development for hypoparathyroidism.

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

“We are pleased about the ODD for TransCon PTH, which reflects the need for a true PTH replacement therapy that more fully addresses all aspects of hypoparathyroidism,” said Jonathan Leff, M.D., Ascendis Pharma’s Chief Medical Officer. “We have recently completed a phase 1 trial in healthy adults, which reinforced the potential of TransCon PTH to restore PTH to physiological levels for 24 hours per day. We are committed to developing innovative new therapies that improve patients’ lives and look forward to advancing TransCon PTH.”

The FDA grants orphan status to drugs that are intended for the safe and effective treatment, diagnosis, or prevention of rare diseases or disorders that affect fewer than 200,000 people in the United States. The ODD program provides a drug developer with certain benefits and incentives, including a seven-year period of U.S. marketing exclusivity from the date of marketing authorization, waiver of FDA user fees, and tax credits for clinical research. The granting of an orphan drug designation does not alter the FDA’s regulatory requirements to establish safety and effectiveness of a drug through adequate and well-controlled studies to support approval and commercialization.

TransCon PTH is designed to restore PTH to physiologic levels, normalizing blood and urinary calcium levels, serum phosphate levels and bone turnover. Ascendis Pharma plans to initiate a phase 3 program for TransCon PTH in the first quarter of 2019, pending discussions with regulatory agencies.

About Hypoparathyroidism

Hypoparathyroidism affects approximately 80,000 patients in the U.S., the majority of whom develop the condition following damage or accidental removal of the parathyroid glands during thyroid surgery.

In the short term, symptoms include weakness, muscle cramps, abnormal sensations such as tingling, burning and numbness (paresthesias), memory loss, impaired judgment and headache. Patients often experience decreased quality of life. 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.

Conventional therapy for hypoparathyroidism with active vitamin D analogs and calcium supplementation do not fully control the disease and may lead to renal disease caused by progressive calcification of the kidney and kidney stones resulting from increased calciuria. As a result, patients with hypoparathyroidism have an estimated four-fold greater risk of renal disease compared to healthy controls.

About Ascendis Pharma A/S

Ascendis Pharma is applying its innovative prodrug technology to build a leading, fully integrated rare disease company focused on making a meaningful difference in patients’ lives. The company utilizes its TransCon technology with clinically validated parent drugs to create new therapies with potential for best-in-class efficacy, safety and/or convenience.

Ascendis Pharma has a wholly-owned pipeline of three rare disease endocrinology product candidates in clinical development. These include once-weekly TransCon Growth Hormone, which is being evaluated in a phase 3 program for children with growth hormone deficiency (GHD), TransCon PTH, a long-acting prodrug of parathyroid hormone for hypoparathyroidism for which a phase 1 trial has been completed, and TransCon CNP, a long-acting prodrug of C-type natriuretic peptide, which is also in phase 1 development for achondroplasia and other FGFR-related skeletal disorders.

Additionally, Ascendis Pharma has multi-product collaborations with Sanofi in diabetes and Genentech in the field of ophthalmology.

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 our 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) our ability to apply our prodrug technology to build a leading, fully integrated rare disease company, (ii) our expectations regarding when we will initiate a phase 3 program for TransCon PTH, (iii) our expectations regarding our ability to create therapies with potential for best-in-class efficacy, safety and/or convenience and (iv) our product pipeline. We 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 we make, including the following: unforeseen safety or efficacy results in our TransCon Growth Hormone, TransCon PTH and TransCon CNP or other development programs; unforeseen expenses related to the development of TransCon Growth Hormone, TransCon PTH and TransCon CNP or other development programs, general and administrative expenses, other research and development expenses and our business generally; delays in the development of TransCon Growth Hormone, 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; and our ability to obtain additional funding, if needed, to support our business activities. 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 our business in general, see our current and future reports filed with, or submitted to, the U.S. Securities and Exchange Commission (SEC), including our Annual Report on Form 20-F for the year ended December 31, 2017, which we filed with the SEC on March 28, 2018. Forward-looking statements do not reflect the potential impact of any future in-licensing, collaborations, acquisitions, mergers, dispositions, joint ventures, or investments we may enter into or make. We do not assume any obligation to update any forward-looking statements, except as required by law.

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