Ascendis Pharma Expands TransCon™ PTH Phase 2 PaTH Forward Clinical Trial to Expedite Enrollment of Subjects Previously Treated with NATPARA® in the United States

November 14, 2019

- Reflects company values and commitment to patients living with hypoparathyroidism -

- Top-line data from expanded PaTH Forward Trial expected in first quarter of 2020 -

- Company expects to exceed targeted enrollment of 40 subjects -

COPENHAGEN, Denmark, Nov. 14, 2019 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND), a biopharmaceutical company that utilizes its innovative TransCon technology to address unmet medical needs, today announced a protocol addendum designed to facilitate enrollment of subjects previously treated with NATPARA (parathyroid hormone) for Injection in the United States (US) in PaTH Forward, a global phase 2 trial evaluating the safety, tolerability and efficacy of TransCon PTH in adult subjects with hypoparathyroidism (HP). TransCon PTH is an investigational long-acting prodrug of parathyroid hormone (PTH) in development as a potential once-daily replacement therapy for HP.

Previously, patients treated with NATPARA were required to undergo a long washout period prior to entering screening in PaTH Forward. In response to the recent recall of NATPARA in the US, Ascendis has been evaluating pathways to help enroll patients affected by the recall. Under the protocol addendum, patients previously treated with NATPARA in the US will now have an expedited pathway to enroll in PaTH Forward. As a result, the company expects to exceed targeted enrollment of 40 subjects in the trial.

“This has been one of the most challenging times in the history of the hypoparathyroidism community, as we have navigated both the emotional and physical impact of the only PTH replacement therapy available being recalled in the US,” said Deb Murphy, President and Vice Chair of the Board of Trustees of the HypoPARAthyroidism Association, Inc. “We are grateful and very excited about Ascendis Pharma’s efforts to expedite participation in the PaTH Forward Trial for additional patients.”

Patients interested in participating in PaTH Forward in the US should discuss it with their physician, or visit pathforwardtrial.com, where they may contact a clinical representative who will refer them to a participating clinical investigator.

“The unexpected recall of NATPARA in the US has had a major impact on my patients who have not been optimally controlled on standard of care with vitamin D and calcium supplements,” said Mishaela Rubin, M.D., a PaTH Forward investigator. “Patients living with hypoparathyroidism have an acute need to manage both their short-term symptoms and reduce risk of long-term complications. The PaTH Forward Trial is an opportunity for patients to participate in evaluation of a new potential treatment option for this debilitating disease.”

PaTH Forward is a global, phase 2, randomized, double-blind, placebo-controlled, parallel group trial that will evaluate safety and efficacy of three fixed doses of TransCon PTH. The goal of PaTH Forward is to evaluate TransCon PTH control of serum and urinary calcium, and identify a titration regimen for complete withdrawal of standard of care (i.e., active vitamin D and calcium supplements). The trial will include adult subjects with HP who are currently receiving standard of care or were previously treated with parathyroid hormone therapies at up to 40 sites worldwide. The PaTH Forward Trial will introduce a ready-to-use pre-filled pen device and assess disease-specific patient-reported outcomes. After four weeks of dosing, all subjects may enter an open-label extension period with the opportunity to receive TransCon PTH to evaluate long-term safety and efficacy.

About TransCon™ Technology

TransCon refers to “transient conjugation.” The proprietary TransCon platform is an innovative technology to create new therapies that 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 pH and temperature conditions initiate the release of the active, unmodified parent drug in a predictable release manner. Because the parent drug is unmodified, its original mode of action is expected to be maintained. TransCon technology can be applied broadly to a protein, peptide or small molecule in multiple therapeutic areas, and can be used systemically or locally.

About Hypoparathyroidism (HP)

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 80,000 patients in the United States, 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 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 has established oncology as its second therapeutic area of focus. Additionally, Ascendis Pharma has multi-product collaborations with Sanofi in diabetes and Genentech in the field of ophthalmology and continues to expand into additional therapeutic areas for both internal and external development.

Ascendis is headquartered in Copenhagen, Denmark, with offices in Heidelberg, Germany and Palo Alto, 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 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) the timing of the topline data from the PaTH Forward Trial, (ii) our ability to apply our platform technology to build a leading, fully integrated biopharma company, (iii) our expectations regarding our ability to create new and potentially best-in-class therapies 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 hGH, TransCon PTH and TransCon CNP or other development programs; unforeseen expenses related to the development and potential commercialization of TransCon hGH, 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 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 and potential commercial sale, if approved; 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, 2018, which we filed with the SEC on April 3, 2019. 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.

Internal contact:
Scott T. Smith
Chief Financial Officer
(650) 352-8389
ir@ascendispharma.com

Media contact:
Ami Knoefler
Head of Global Communications
(650) 739-9952
ack@ascendispharma.com

Investor contact:
Patti Bank
Westwicke Partners
(415) 513-1284
patti.bank@westwicke.com

Source: Ascendis Pharma