Ascendis Pharma A/S Announces Expansion of Global Clinical Reach for TransCon™ PTH with Filing of the Clinical Trial Notification for Phase 3 Clinical Trial in Adults with Hypoparathyroidism in Japan

May 12, 2021 at 4:28 PM EDT

– Marks next step to bring the potential first ever hormone replacement therapy for hypoparathyroidism to Japan –

COPENHAGEN, Denmark, May 12, 2021 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND), a biopharmaceutical company that utilizes its innovative TransCon™ technologies to create product candidates that address unmet medical needs, today announced that it has filed a Clinical Trial Notification (CTN) with the Pharmaceuticals and Medical Devices Agency (PDMA) in Japan to initiate the company's phase 3 clinical trial of TransCon PTH in adult subjects with hypoparathyroidism (HP), the PaTHway Japan Trial.

"The CTN filing for TransCon PTH represents another milestone as we continue to lay the groundwork to make this important therapy available globally to address a significant unmet need for patients with hypoparathyroidism worldwide. The phase 3 PaTHway Japan Trial, which has been the subject of careful negotiations with the PDMA, will enroll a predetermined cohort of patients so that the combined global program will support submission of a marketing authorization application in Japan. This study, along with long-term data from our phase 2 PaTH Forward Trial and data from our phase 3 PaTHway trial, should potentially enable rapid access to an important hormone replacement therapy for patients living with HP in Japan," said Jan Mikkelsen, President and CEO of Ascendis Pharma.

"An objective of our Vision 3x3 is to obtain global clinical reach for our endocrinology rare disease pipeline. Starting first with North America and Europe, then expanding to Greater China through our investment in VISEN Pharmaceuticals, our recent expansion of our clinical reach into Japan with TransCon hGH and TransCon PTH represents an important milestone in realizing this vision," continued Mr. Mikkelsen.

The phase 3 PaTHway Japan Trial is designed to be a single arm study with the objective to evaluate the safety and efficacy of TransCon PTH in adults with hypoparathyroidism.

About TransCon PTH[1]

TransCon PTH is an investigational once-daily long-acting prodrug of parathyroid hormone (PTH[1-34]) in development as a treatment for adult hypoparathyroidism (HP) designed to restore PTH at physiologic levels for 24 hours each day to address both the short-term symptoms and long-term complications of the disease. TransCon PTH was granted Orphan Drug Designation (ODD) by the U.S. Food and Drug Administration (FDA) in June 2018, and in October 2020 was granted Orphan Designation by the European Commission for the treatment of hypoparathyroidism.

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.

HP remains among the few hormonal insufficiency states without a replacement therapy that restores the missing hormone at physiologic levels. 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 TransCon technologies to build a leading, fully integrated biopharmaceutical 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 and one oncology product candidate in clinical development. 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, Palo Alto and Redwood City, California, and Princeton, New Jersey.

Please visit www.ascendispharma.com (for global information) or www.ascendispharma.us (for U.S. information.)

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' expectations regarding Ascendis' potential submission of a marketing authorization application in Japan, (ii) Ascendis' expectations regarding the phase 3 PaTHway Japan Trial, along with other

data, enabling rapid access to a hormone replacement therapy for patients living with HP in Japan, (iii) Ascendis' Vision 3x3 (iv) Ascendis' ability to apply its platform technology to build a leading, fully integrated biopharmaceutical company, (v) Ascendis' product pipeline and expansion into additional therapeutic areas and (vi) 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 from 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' Annual Report on Form 20-F filed with the U.S. Securities and Exchange Commission (SEC) on March 10, 2021 and Ascendis' other future reports filed with, or submitted to, the SEC. 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.

Investor contacts:
Tim Lee
Ascendis Pharma
(650) 374-6343
tle@ascendispharma.com

Patti Bank
Westwicke Partners
(415) 513-1284
patti.bank@westwicke.com
ir@ascendispharma.com

Media contact:
Ami Knoefler
Ascendis Pharma
(650) 739-9952
ack@ascendispharma.com

[1] Karpf DB, et al. *J Bone Miner Res.* 2020; x:1-11.
[2] Mannstadt M, et al. *Nature Reviews* 2017, 3: 17055
[3] Ascendis Pharma HP Patient Experience Research.
[4] Hadker N, et al. *Endo Pract.* 2014, 20(7);671-679.
[5] Powers J, et al. *J Bone Miner Res* 2013, 28: 2570-2576.
[6] Mitchell DM, et al. *J Clin Endocrinol Metab* 2012, 97(12): 4507-4514
[7] Underbjerg L, et al. *J Bone Miner Res* 2013, 28: 2277-2285

Source: Ascendis Pharma