Ascendis Pharma A/S Announces Presentations for TransCon™ PTH and Hypoparathyroidism at American Society for Bone and Mineral Research Annual Meeting

September 10, 2020

COPENHAGEN, Denmark, Sept. 10, 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 three presentations for its TransCon PTH program for hypoparathyroidism (HP) at the American Society for Bone and Mineral Research (ASBMR) 2020 Annual Meeting Virtual Event, taking place September 11–15.

In addition, results from the randomized, double-blind phase 2 PaTH Forward Trial, which are being presented in a scientific forum for the first time by Aliya Khan, M.D., were selected for presentation at the "*Highlights of the ASBMR Meeting*" by John Bilezikian, M.D., on Friday, September 11 at 11:00 a.m. ET.

"We are excited that ASBMR has chosen to highlight results from the 4-week, fixed dose double-blinded portion of the PaTH Forward Trial. The study demonstrated that TransCon PTH eliminated standard of care (i.e. off active vitamin D and ≤ 500 mg per day of calcium supplements) in 82 percent of subjects across all dosage arms,[1]" said Dr. Khan, primary investigator and Clinical Professor, Department of Medicine, Divisions of Endocrinology and Metabolism and Geriatric Medicine at McMaster University, Ontario, Canada. "We also found that TransCon PTH demonstrated a statistically-signficiant improvement in physical and mental wellbeing as assessed by the validated SF-36® Health Survey[1] and importantly, the results were consistent with a clinically meaningful improvement in functional health and wellbeing for subjects receiving TransCon PTH as compared with placebo after four weeks.[2]"

Also being presented at ASBMR are the results from the *Voices of Hypara Survey*, which was done in collaboration with the HypoPARAthyroidism Association (HPA). A key finding of the survey is that more than two-thirds of patients experienced calcium crashes in the last year and almost half of all participants visited the emergency room or urgent care to address their HP symptoms, despite treatment with standard of care.[3]

"Our survey illustrates how challenging it is for people with HP due to all causes, including diagnosis, managing the daily symptoms, quality-of-life and long-term complications of HP[3]," said Bob Sanders, Chairman of Board of the HypoPARAthyroidism Association. "We urgently need more effective treatment options that improve the overall health outcomes and quality-of-life for people with HP[3]. We look forward to partnering with the medical community to raise awareness of HP and ensure that patients have access to treatments."

Presentation Details

ASBMR 2020 Annual Meeting Virtual Event

Oral Presentation

Title	**Date/Time**
Results of the PaTH Forward Phase 2 Trial Demonstrating Potential of TransCon PTH as a Replacement Therapy for Hypoparathyroidism	Tuesday, September 15, 2020 11 am – 12:15 pm (ET)

Poster Presentations

Title	**Date/Time**
Validation of the Hypoparathyroidism Patient Experience Scale (HPES): Understanding and Assessing Burden of Disease	Friday, September 11 –Tuesday, September 15
The Voices of Hypopara Survey: Journey of Patients Living with Hypoparathyroidism	

The posters will be available on the Ascendis website under Selected Publications in the Pipeline section: https://ascendispharma.com/product-pipeline/publications/. If you are a healthcare provider who would like more information, please contact: medicalaffairs@ascendispharma.com.

About TransCon PTH

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 replace 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) from the U.S. Food and Drug Administration (FDA) in June 2018.

About Hypoparathyroidism (HP)[4,5,6,7,8,9]

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

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 technology 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.

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’ ability to apply its platform technology to build a leading, fully integrated biopharma company, (ii) Ascendis’ product pipeline and expansion into additional therapeutic areas, and (iii) 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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[1] Khan A, et al. American Society for Bone and Mineral Research Annual Meeting. Sept. 2020. Oral Presentation.

[2] Maruish, M. E. (Ed.). *User’s manual for the SF-36v2 Health Survey* (3rd ed.). Lincoln, RI: QualityMetric Incorporated.

[3] Astolfi D, et al. American Society for Bone and Mineral Research Annual Meeting. Sept. 2020. Abstract and poster.

[4] Mannstadt M, et al. *Nature Reviews* 2017, 3: 17055

[5] Ascendis Pharma HP Patient Experience Research.

[6] Hadker N, et al. *Endo Pract.* 2014, 20(7);671-679.

[7] Powers J, et al. *J Bone Miner Res* 2013, 28: 2570-2576.

[8] Mitchell DM, et al. *J Clin Endocrinol Metab* 2012, 97(12): 4507-4514

[9] Underbjerg L, et al. *J Bone Miner Res* 2013, 28: 2277-2285.

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