Ascendis Pharma Announces Results of Study at ENDO 2019 Evaluating Impact of Hypoparathyroidism on Patient Quality-of-Life

March 25, 2019 at 2:00 PM EDT

Despite current treatments, 100 percent of patients experienced physical signs and symptoms, burden on daily life and psychological impact of disease

COPENHAGEN, Denmark, March 25, 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 study evaluating impact of hypoparathyroidism (HP) on patients in a poster at the Endocrine Society's annual meeting, ENDO 2019, in New Orleans. A paucity of data exists on the short-term symptoms and burden of illness experienced by patients with this condition. The study found that, despite treatment with current therapies, 100 percent of patients experienced physical impact, psychological impact, and interference with daily life, reflecting a substantial illness burden from HP.

"I have lived with HP for over 25 years and have personally experienced how this debilitating disease affects all areas of life - physical, emotional and social," said Deb Murphy, President of the HypoPARAthyroidism Association, Inc. "This study highlights that currently available therapies are still not enough. It is critical to not only increase awareness of HP, but to develop new treatment options that address all aspects of the disease and improve patients' quality-of-life."

In the study, 42 adult patients with post-surgical (diagnosis at least 6 months) or idiopathic (diagnosis at least 12 months) HP were interviewed. Patients were required to be stable on treatment (oral calcium and vitamin D supplements and/or parathyroid hormone therapy with PTH[1-84]) for at least 3 months. Over half the patients (52.4 percent) were treated with PTH(1-84). Participants were predominantly female (83.3 percent) and had post-surgical HP (85.7 percent).

Despite treatment, 71 percent of patients reported that managing their condition was difficult. Other findings included:

- All patients reported experiencing physical signs and symptoms attributable to HP, including paresthesia (tingling, numbness), muscle cramps and physical fatigue
- 86 percent reported experiencing one or more forms of cognitive dysfunction (collectively known as "brain fog"), including difficulties with memory/forgetfulness, having a conversation, being able to concentrate, fuzzy thinking and understanding information
- All patients reported that HP interfered with their daily life and caused some psychological issues, including anxiety (81 percent), frustration (64 percent) and depression (62 percent)
- For 95 percent of patients, HP also affected physical functioning and social interactions, such as the ability to exercise and be mobile, as well as the ability to participate in social activities and build/maintain relationships
- Additionally, over half the patients reported difficulty finding primary care physicians, specialists, or emergency department physicians with sufficient working knowledge about the disease.

"These results clearly show the need for a treatment option that can address all aspects of HP to help alleviate the significant quality-of-life issues these patients face every day," said Jonathan Leff, M.D., Ascendis Pharma's Chief Medical Officer. "Our endocrinology rare disease pipeline includes a potential new therapy for hypoparathyroidism, TransCon PTH, which is designed to restore PTH to physiologic levels continuously for 24 hours a day. By providing sustained levels of PTH, we hope to control and maintain serum and urinary calcium levels. In doing so, we hope to prevent many of the debilitating quality-of-life challenges highlighted in this study."

TransCon PTH is a long-acting prodrug of parathyroid hormone (PTH[1-34]), in development as a potential once-daily replacement therapy for HP. PaTH Forward is a global phase 2 trial designed to evaluate the safety, tolerability and efficacy of TransCon PTH in adult subjects with hypoparathyroidism. The trial is also evaluating a titration regimen for the complete withdrawal of standard of care (i.e., active vitamin D and calcium supplements). The company anticipates top-line results from the PaTH Forward trial in the fourth quarter of 2019.

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

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. In fact, patients with HP have an estimated 4-fold greater risk of renal disease compared to healthy controls.

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.

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 rare disease endocrinology 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) our expectation to receive top-line results from the PaTH Forward trial in the fourth quarter of 2019, (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 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, 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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Source: Ascendis Pharma A/S