Ascendis Pharma Provides Update on Rare Disease Endocrinology Pipeline and Presents Initial Phase 1 Data for TransCon PTH

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COPENHAGEN, Denmark, Jan. 08, 2018 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq:ASND), a biopharmaceutical company that utilizes its innovative TransCon technology to address significant unmet medical needs in rare diseases, today provided an update on its rare disease endocrinology pipeline and presented initial phase 1 data for TransCon PTH, which is in development as a potential treatment for hypoparathyroidism (HP). The company is reviewing the status of, and milestones for, its three wholly-owned product candidates during a presentation today at the 36th Annual J.P. Morgan Healthcare Conference.

"We look forward to significant progress during 2018 for our rare disease endocrinology pipeline," said Jan Mikkelsen, Ascendis Pharma's President and Chief Executive Officer. "We have exceeded our target enrollment in the TransCon Growth Hormone pivotal trial and we anticipate announcing top-line results in the first quarter of next year. Our initial phase 1 data for TransCon PTH support our target product profile of TransCon PTH as a true PTH replacement therapy in HP. During the coming year, we also plan to initiate a phase 1 healthy volunteer trial and present top-line data for our TransCon CNP program in achondroplasia. We are making tremendous progress as we work to bring new therapies to patients with rare diseases."

Updates on the company's three pipeline programs include:

- ***TransCon Growth Hormone***: The company exceeded its target enrollment in the phase 3 heiGHt Trial of TransCon Growth Hormone for pediatric growth hormone deficiency. Given strong enthusiasm for the trial from investigators and subjects, the company has randomized over 160 subjects. Based on one-year follow-up, top-line results are anticipated in the first quarter of 2019.

  During the third quarter of 2018, Ascendis also plans to complete enrollment in the fliGHt Trial to evaluate TransCon Growth Hormone in subjects who have previously been treated with daily growth hormone therapy.

- ***TransCon PTH***: Pharmacokinetic data from an ongoing phase 1 trial of TransCon PTH in healthy volunteers demonstrated a half-life of approximately 60 hours, supporting an infusion-like profile with daily administration. Additionally, single ascending doses of TransCon PTH led to sustained and dose-dependent elevations of serum calcium lasting more than 72 hours with low inter-subject variability. This low variability supports the ability to titrate and individualize dosing in patients. TransCon PTH also demonstrated the expected effects on renal calcium reabsorption and down regulation of endogenous PTH(1-84). The company anticipates data from the ongoing phase 1 trial to be presented throughout 2018. The company plans to advance TransCon PTH directly into phase 3 development in the first quarter of 2019.

- ***TransCon CNP***: The company recently initiated the regulatory process in Australia to enable its first-in-human phase 1 trial for TransCon CNP. Dosing in the phase 1 trial in healthy volunteers is expected to begin in the second quarter of 2018, with top-line data to be announced in the fourth quarter of 2018. There are currently no FDA-approved treatments for achondroplasia.

Slides from the J.P. Morgan presentation may be viewed on the Ascendis Pharma website at: http://phx.corporate-ir.net/phoenix.zhtml?c=242088&p=irol-calendar

A live audio webcast of the company's presentation at 3:00 p.m. Pacific Time will also be available in the Investors and News section of the Ascendis Pharma website at www.ascendispharma.com. A webcast replay will also be available on this website shortly after conclusion of the event for 30 days.

About Ascendis Pharma A/S

Ascendis Pharma is applying the TransCon technology platform to build a leading rare disease commercial company. The company utilizes its innovative TransCon technology to address significant unmet medical needs in rare diseases by potentially improving clinically validated parent drugs and creating therapies with potential for best-in-class efficacy, safety and/or convenience.

Ascendis Pharma has a wholly-owned pipeline of rare disease endocrinology programs, including once-weekly TransCon Growth Hormone, which is currently 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 currently in a phase 1 trial, and TransCon CNP, a long-acting prodrug of C-type Natriuretic Peptide for achondroplasia. 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 the TransCon technology platform to build a leading rare disease commercial company, (ii) our expectations regarding when we will announce top-line results from the phase 3 heiGHt trial, (iii) our expectations regarding we will initiate a phase 1 healthy volunteer trial and release top-line data for our TransCon CNP program in achondroplasia, (iv) our expectations regarding when we will complete enrollment in the fliGHt Trial, (v) our expectations regarding when we will present data from the phase 1 trial of TransCon PTH, (vi) our ability to move TransCon PTH directly into phase 3 development, (vii) our expectations regarding our ability to create therapies with potential for best-in-class efficacy, safety and/or convenience and (viii) 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, 2016, which we filed with the SEC on March 22, 2017. 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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