Ascendis Pharma A/S Showcases New Data on Rare Disease Product Pipeline at ENDO 2017

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- TransCon Growth Hormone Data Support Ongoing Phase 3 heiGHt Trial -

- Late-Breaking Posters on TransCon PTH and TransCon CNP Summarize Potential "Best-in-Class" Product Profiles -

COPENHAGEN, Denmark, April 3, 2017 /PRNewswire/ -- 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 announced data supporting its product pipeline presented at ENDO 2017, the annual meeting of the Endocrine Society in Orlando, Florida.

"We are delighted to show the breadth of our growing pipeline at this year's ENDO conference, including three potentially differentiated treatments for rare endocrine diseases," said Jonathan Leff, M.D., Chief Medical Officer at Ascendis Pharma. "Our pipeline of three unique, wholly-owned product candidates leverages our TransCon technology platform and product development approach, which may lead to higher success rates than traditional drug development."

TransCon Growth Hormone: Phase 3 Program On Track

TransCon Growth Hormone, with convenient weekly dosing, is designed to release unmodified growth hormone that diffuses freely into the body's tissues, maximizing its ability to carry out the systemic effects of endogenous and daily growth hormone. It is currently in Phase 3 development as a potential therapy for children with growth hormone deficiency (GHD). At ENDO:

- Two posters described the clinical development program for TransCon Growth Hormone, including results of the pediatric GHD Phase 2 trial, which provided the foundation for the ongoing Phase 3 program.
- For the first time, Ascendis researchers also presented data regarding the company's proprietary auto-injector device, showing that data collected from >70 patients, caregivers and healthcare professionals found the auto-injector easy-to-use. With multiple features designed to create a patient-centric experience, the auto-injector will be used in the extension phase of the heiGHt Trial and is planned to be available for launch of TransCon Growth Hormone.

"There is a significant need for a safe and effective long-acting growth hormone therapy that will help improve compliance in pediatric patients with GHD," said Aristides K. Maniatis, MD, FAAP, pediatric endocrinologist from Rocky Mountain Pediatric Endocrinology and an investigator in the heiGHt Trial. "The Phase 2 data demonstrated similar efficacy, safety and tolerability of TransCon Growth Hormone compared to daily growth hormone therapy, and I am pleased to be participating in this important Phase 3 study."

TransCon Growth Hormone is being evaluated in the Phase 3 heiGHt Trial, which compares its safety, efficacy and tolerability to daily growth hormone therapy. Ascendis Pharma expects to complete enrollment of the study in the fourth quarter of 2017.

TransCon PTH: A True Potential PTH Replacement Therapy

TransCon PTH is a sustained-release prodrug of parathyroid hormone [PTH(1-34)] for the treatment of hypoparathyroidism, a rare and serious endocrine deficiency disorder that can lead to a variety of neuromuscular, cardiovascular, and other symptoms. It is designed to maintain a steady PTH level within the physiological range over a 24-hour period, helping to address limitations of available therapies.

Two posters at ENDO reviewed the pharmacokinetics and pharmacodynamics of TransCon PTH in animal models:

- Preclinical data in two separate models demonstrated the infusion-like profile of TransCon PTH following once-daily administration. Studies in healthy monkeys also showed TransCon PTH increased serum calcium while reducing urinary calcium excretion.
- In the late-breaking poster, a study in rats who were PTH-deficient following thyroparathyroidectomy showed TransCon PTH normalized serum calcium and phosphorus. This was compared to a higher daily dose of PTH(1-84), a currently available PTH replacement therapy, which did not. Additionally, the data suggested positive effects on bone turnover at evaluated doses.

"We are excited about the potential of TransCon PTH, which may more fully control all aspects of hypoparathyroidism and its underlying disease pathophysiology," said David B. Karpf, M.D., Ascendis Pharma's Vice President of Clinical Development and a practicing endocrinologist at Stanford University. "Clinical trials have demonstrated that an infusion-like profile is superior to once or twice daily injections of PTH(1-34). TransCon PTH has the potential to become a true PTH replacement therapy as it is designed to maintain physiologic PTH levels around the clock, potentially offering patients a significant treatment advance."

Ascendis Pharma plans to initiate a Phase 1 clinical trial of TransCon PTH in the third quarter of 2017.

TransCon CNP Aims to Improve Efficacy and Safety in a Novel Treatment for Achondroplasia

Ascendis scientists presented two posters on TransCon CNP, a sustained-release prodrug of C-Type Natriuretic Peptide, for the treatment of

achondroplasia, the most common form of dwarfism:

- One presentation summarized the pharmacokinetics and lack of adverse hemodynamic effects of TransCon CNP, allowing for the administration of high doses to facilitate optimal efficacy.
- A late-breaking poster showed a dose-dependent effect on long bone (tibia) growth in juvenile monkeys with weekly TransCon CNP. Following two-month data previously presented at the company's recent R&D Update, these results demonstrated growth effects continued through six months.
- The poster also summarized the effects of TransCon CNP in a mouse model of achondroplasia, including bone growth and the potential to ameliorate some of the more disabling achondroplasia traits, including stenosis of the foramen magnum.

"The encouraging TransCon CNP preclinical data support the hypothesis that a CNP analogue can be an effective treatment for achondroplasia without dose-limiting hypotension," said Kennett Sprogøe, Ph.D., Senior Vice President of Product Innovation at Ascendis Pharma. "Patients with achondroplasia have no approved drug therapies, and we are pleased to advance a novel product candidate in this area of significant unmet medical need."

Ascendis Pharma plans to submit an investigational new drug application or equivalent for TransCon CNP in the fourth quarter of 2017.

All eight poster presentations from ENDO 2017 are available on the Publications page in the Pipeline section of the company's website at www.ascendispharma.com.

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 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 the Phase 3 heiGHt Trial for children with growth hormone deficiency (GHD), TransCon PTH, a long-acting prodrug of parathyroid hormone for hypoparathyroidism, 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 expectations of completing enrollment of our Phase 3 heiGHt Trial in the fourth quarter of 2017; (ii) whether our proprietary auto-injector device will be available at the launch of TransCon Growth Hormone; (iii) whether TransCon PTH has the potential to become a true PTH replacement therapy; (iv) our expectations of initiating a Phase 1 clinical trial of TransCon PTH in the third quarter of 2017; (vi) our plans to submit an investigational new drug application or equivalent for TransCon CNP in the fourth quarter of 2017 (vi) our ability to apply the TransCon technology platform to build a leading rare disease commercial company and (vii) our expectations regarding our ability to create therapies with potential for best-in-class efficacy, safety and/or convenience. 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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