Ascendis Pharma A/S Announces Positive Preliminary Phase 1 Data for TransCon CNP

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- TransCon CNP delivered continuous exposure of CNP at target levels for seven days with a single subcutaneous administration -

- TransCon CNP phase 1 results reinforce preclinical data and support target product profile -

COPENHAGEN, Denmark, Nov. 28, 2018 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND), a biopharmaceutical company that utilizes its innovative TransCon ™ technology to address significant unmet medical needs, today announced positive preliminary results from a phase 1 trial of TransCon CNP. These results showed TransCon CNP provided continuous exposure to C-type natriuretic peptide (CNP) with a pharmacokinetic profile designed to maximize efficacy with once-weekly dosing. Additionally, the mean resting blood pressure and heart rate were unchanged from predose levels at all time points in all cohorts. Based upon these results, the company plans to initiate a phase 2 program of TransCon CNP in pediatric subjects with achondroplasia in mid-2019.

A presentation of the phase 1 results may be viewed on the Ascendis Pharma website at: https://ascendispharma.gcs-web.com/events-and-presentations/upcoming-events

“The TransCon CNP data presented for the first time today indicate that our product candidate delivered continuous exposure of CNP at target levels over seven days, supporting once-weekly administration,” said Jan Mikkelsen, Ascendis Pharma’s President and Chief Executive Officer. “This is an important step forward for patients. We believe that TransCon CNP is well-suited to address the challenges of people living with achondroplasia, and offers a potential new therapeutic option that looks beyond height to address the associated comorbidities. We look forward to advancing this program into clinical trials for children living with achondroplasia next year.”

In this phase 1, double-blind, randomized, placebo-controlled trial, 45 healthy adult subjects were enrolled. Five doses of TransCon CNP were tested sequentially, beginning with the lowest dose: 3.0, 10, 25, 75 and 150 microgram/kg. Up to 10 subjects in each dose cohort were randomized to receive TransCon CNP or placebo in a 4:1 ratio. After each cohort completed dosing, a Data Safety Monitoring Board (DSMB) reviewed the blinded data to approve escalation to the next higher dose. The primary endpoint was frequency of adverse events after administration of TransCon CNP. Secondary endpoints included additional safety parameters, tolerability and pharmacokinetics.

TransCon CNP is a long-acting prodrug of CNP comprised of an unmodified parent drug transiently bound to an inert carrier molecule via a proprietary linker. Following subcutaneous administration, TransCon CNP is designed to allow the body’s physiologic pH and temperature to release and activate CNP in a controlled manner over the course of one week. The carrier and linker are then cleared through the kidneys. Because the parent CNP drug is unmodified, its mode of action is expected to be maintained. TransCon CNP is in development as a treatment for achondroplasia and has potential as a treatment for other growth disorders, including fibroblast growth factor receptor (FGFR)-related skeletal disorders.

About Achondroplasia

Achondroplasia is the most common form of dwarfism, affecting approximately 250,000 people worldwide. Achondroplasia results in severe skeletal complications and comorbidities, including narrowing of the foramen magnum, sleep apnea and chronic ear infections. Individuals with achondroplasia often face multiple surgeries and procedures to alleviate its many complications.

The condition is caused by an autosomal dominant activating mutation in the fibroblast growth factor receptor 3 (FGFR3) gene that leads to an imbalance in the effects of the FGFR3 and CNP signaling pathways. Preclinical and clinical data show that the CNP pathway stimulates growth. Increased CNP counteracts the effects of the FGFR3 mutation downstream, thus promoting bone growth.

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 ™ technology 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. 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 plans to initiate our phase 2 TransCon CNP trial in pediatric subjects with achondroplasia in mid-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 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 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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