Ascendis Pharma Announces Orphan Drug Designation Granted for TransCon CNP as Treatment for Achondroplasia

March 1, 2019

COPENHAGEN, Denmark, Feb. 28, 2019 (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 that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) to TransCon CNP, a long-acting prodrug of C-type natriuretic peptide (CNP) in development for children with achondroplasia.

Achondroplasia is the most common form of dwarfism and there are no FDA-approved therapies for this condition. Individuals living with achondroplasia may experience severe skeletal complications and comorbidities, including narrowing of the foramen magnum, sleep apnea and chronic ear infections.

"We are pleased to receive Orphan Drug Designation for TransCon CNP in achondroplasia. Our TransCon CNP candidate is designed to address not only height, but the debilitating comorbidities of the condition," said Jonathan Leff, M.D., Ascendis Pharma's Chief Medical Officer. "In our phase 1 trial in healthy subjects, TransCon CNP delivered continuous exposure of CNP at target levels over seven days, supporting once-weekly dosing with a well-tolerated safety profile. Based on these preliminary results, we expect to initiate a phase 2 trial in children with achondroplasia in the third quarter of this year. In addition, as part of our commitment to the achondroplasia community, we are currently conducting the ACHieve Study, which we believe will provide important observational insights into the experience of children living with achondroplasia."

The FDA grants orphan status to drugs that are intended for the safe and effective treatment, diagnosis, or prevention of rare diseases or disorders that affect fewer than 200,000 people in the United States. The ODD program provides a drug developer with certain benefits and incentives, including a seven-year period of U.S. marketing exclusivity from the date of marketing authorization, waiver of FDA user fees, and tax credits for clinical research. The granting of an orphan drug designation does not alter the FDA's regulatory requirements to establish safety and effectiveness of a drug through adequate and well-controlled studies to support approval and commercialization.

TransCon CNP is designed to address all aspects of achondroplasia by providing continuous exposure to CNP at safe, therapeutic levels in a single, weekly subcutaneous dose.

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 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 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 plans to initiate a phase 2 trial of TransCon CNP in children with achondroplasia in the third quarter of this year, (ii) our ability to apply our platform technology to build a leading, fully integrated biopharma company, (iii) our expectations regarding our ability to create therapies with potential for best-in-class efficacy, safety and/or convenience 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 GH, TransCon PTH and TransCon CNP or other development programs; unforeseen expenses related to the development of TransCon GH, 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 GH, 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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Source: Ascendis Pharma A/S