Ascendis Pharma A/S Receives Orphan Designation for TransCon™ CNP for the Treatment of Achondroplasia in Europe

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COPENHAGEN, Denmark, Aug. 12, 2020 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND), a biopharmaceutical company that utilizes its innovative TransCon technologies to address unmet medical needs, today announced the European Commission (EC) has granted Orphan Designation to TransCon C-Type Natriuretic Peptide (CNP) for the treatment of achondroplasia, the most common form of dwarfism.[1] TransCon CNP is a investigational long-acting prodrug of CNP, designed to provide continuous exposure of CNP at safe, therapeutic levels via a single, weekly subcutaneous dose. TransCon CNP also received orphan designation for the treatment of achondroplasia in the United States (U.S.) in February 2019. Currently, there is no medical therapy approved for the treatment of achondroplasia.[2]

"We now have orphan designations in Europe and the U.S. for TransCon CNP in achondroplasia, which we believe signifies the importance of developing a safe and effective therapy that can address the many medical complications these children face," Dana Pizzuti, M.D., Senior Vice President of Development Operations at Ascendis Pharma. "With our global phase 2 ACcomplisH Trial of TransCon CNP underway in achondroplasia, we are on track with the development of TransCon CNP and our receipt of the orphan designation in Europe is a key step towards developing a new treatment option for children with achondroplasia."

Orphan Designation in the European Union (EU) is granted to therapies aimed at the treatment, prevention or diagnosis of a disease that is life-threatening or chronically debilitating, affects no more than five in 10,000 persons and for which no satisfactory therapy is available. The medicine also must be expected to provide significant benefit to those affected by the condition. Orphan medicines have 10 years of market exclusivity after they receive marketing authorization in the EU. Under certain conditions, market exclusivity for pediatric indications may be extended for an additional two years.

About TransCon™ Technology

TransCon refers to "transient conjugation." The proprietary TransCon platform is an innovative technology designed 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 conditions (e.g., pH and temperature) initiate the release of the active, unmodified parent drug in a predictable manner. Because the parent drug is unmodified, its original mode of action may be maintained. TransCon technology is designed to be applied broadly to a protein, peptide or small molecule in multiple therapeutic areas, and to be used systemically or locally.

About Achondroplasia

Achondroplasia is the most common form of dwarfism, affecting approximately 250,000 people worldwide. Individuals living with achondroplasia may experience severe skeletal complications and comorbidities. For example, abnormal skeletal development can lead to sleep apnea, chronic back and leg pain from lower spine impingement and sudden infant death from cervical compression. Chronic ear infections due to abnormal eustachian tubes can lead to hearing loss and speech delay.[3]

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.[1]

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 endocrinology rare disease product candidates in clinical development and is advancing oncology as its second therapeutic area of focus. The company continues to expand into additional therapeutic areas to address unmet patient needs.

Ascendis is headquartered in Copenhagen, Denmark, with additional offices in Heidelberg and Berlin, Germany, and in Palo Alto and Redwood City, 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 Ascendis' 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) Ascendis' ability to apply its platform technology to build a leading, fully integrated biopharma company, (ii) Ascendis' product pipeline and expansion into additional therapeutic areas, (iii) Ascendis' expectations regarding its ability to utilize its TransCon technologies to create new and potentially best-in-class therapies and (iv) Ascendis' ability to

develop a treatment option for children with achondroplasia. Ascendis 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 Ascendis makes, including the following: unforeseen safety or efficacy results in its oncology programs, TransCon hGH, TransCon PTH and TransCon CNP or other development programs; unforeseen expenses related to the development and potential commercialization of its oncology programs, TransCon hGH, TransCon PTH and TransCon CNP or other development programs, general and administrative expenses, other research and development expenses and Ascendis' business generally; delays in the development of its oncology programs, 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; Ascendis' ability to obtain additional funding, if needed, to support its business activities and the effects on its business of the worldwide COVID-19 pandemic. 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 Ascendis' business in general, see Ascendis' prospectus supplement filed on July 9, 2020 and Ascendis' current and future reports filed with, or submitted to, the U.S. Securities and Exchange Commission (SEC), including its Annual Report on Form 20-F filed with the SEC on April 3, 2020. Forward-looking statements do not reflect the potential impact of any future in-licensing, collaborations, acquisitions, mergers, dispositions, joint ventures, or investments that Ascendis may enter into or make. Ascendis does not assume any obligation to update any forward-looking statements, except as required by law.

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