Ascendis Pharma A/S Announces Presentations on Achondroplasia and TransCon™ CNP at International Skeletal Dysplasia Society Meeting

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– TransCon CNP program gains momentum with initiation of phase 2 ACcomplisH Trial –

– Presentations provide new estimate of global birth prevalence and highlight quality-of-life impact of achondroplasia on children and families –

– Program demonstrates company's commitment to developing a therapy that considers all aspects of achondroplasia –

COPENHAGEN, Denmark, Sept. 12, 2019 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND), a biopharmaceutical company that utilizes its innovative TransCon technologies to address unmet medical needs, today announced four presentations on achondroplasia and TransCon CNP, the company's investigational therapy for the condition, at the 14 thInternational Skeletal Dysplasia Society (ISDS) meeting in Oslo, Norway. ISDS is a leading international conference on skeletal dysplasias, bringing together global leaders every two years to promote scientific progress in the field of skeletal dysplasia.

The company's presentations include an oral presentation reporting results from a phase 1 trial of TransCon CNP, a long-acting prodrug of C-natriuretic peptide (CNP) in development as a potential therapy for children with achondroplasia. Additional poster presentations report on the global birth prevalence and quality-of-life impact of achondroplasia, including the social and emotional aspects of the condition from the perspectives of both children and their parents.

"This research reflects our holistic view of achondroplasia as we develop TransCon CNP as a safe and effective potential therapy designed to provide continuous exposure to CNP which could make a meaningful impact on patient's lives," said Jan Mikkelsen, Ascendis Pharma's President and Chief Executive Officer. "In addition to the many medical complications associated with the condition, families experience broad social and emotional impacts from achondroplasia. We are committed to fully understanding the patient experience so as to inform our TransCon CNP program – including aspects of achondroplasia beyond skeletal growth."

TransCon CNP has recently advanced into the global phase 2 ACcomplisH Trial, a randomized, double-blind, placebo-controlled trial to evaluate the safety and efficacy of TransCon CNP in approximately 60 subjects with achondroplasia. By delivering continuous levels of CNP, TransCon CNP is expected to help restore balance to the fibroblast growth factor receptor 3 pathway, thereby addressing not only height but the many skeletal complications and comorbidities that can have life-altering implications for children with achondroplasia.

The four presentations at ISDS include:

- An oral presentation titled, "TransCon CNP: A Once-weekly Novel C-type Natriuretic Peptide Therapy in Children with Achondroplasia," summarized data from the phase 1 clinical trial which demonstrates that TransCon CNP resulted in continuous exposure of CNP when administered once-weekly, and was generally well tolerated at doses up to 150 µg/kg, with no serious adverse events or anti-CNP antibodies reported.
- A poster titled, "An Estimate of the Global Birth Prevalence of Achondroplasia," which reported a new meta-analysis of the global birth rate of achondroplasia, concluding that approximately 5,000 new cases occur worldwide each year.
- A poster titled, "Achondroplasia: Impacts on Children's Functioning and Well-Being," describing what Ascendis believes to be the first study to evaluate the broad impacts of achondroplasia on a child's functioning and well-being. The research showed that children with achondroplasia experience a range of social and emotional difficulties, including challenges to school participation.
- A poster titled, "Experiences of Parents of Children with Achondroplasia: Impacts on Quality of Life," describing what Ascendis believes to be the first study to evaluate the effect of achondroplasia on a parent's quality-of-life and well being. The research showed that parents experience a range of caretaking challenges, including missed work time due to medical appointments and increased impact on emotional well-being, such as feelings of stress and worry.

A copy of the poster presentations is available on the Selected Publications page of the company's website at www.ascendispharma.com.

TransCon CNP is a long-acting prodrug of C-type natriuretic peptide (CNP) in development as a potential therapy for children with achondroplasia. TransCon CNP received Orphan Drug Designation (ODD) from the U.S. FDA in February 2019. Ascendis recently initiated the ACcomplisH Trial, a global phase 2 trial designed to evaluate the safety and efficacy of TransCon CNP in subjects with achondroplasia. All subjects who complete the ACcomplisH Trial will have the opportunity to receive TransCon CNP in a long-term safety trial. Ascendis expects to enroll the first subjects in the ACcomplisH Trial later this year.

Ascendis Pharma is also conducting the ACHieve Study, a natural history study that aims to provide important observational insights into the experience of children living with achondroplasia.

About TransCon™ Technology

TransCon is short for “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 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 a small molecule in multiple therapeutic areas, and can 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 development of the vertebra 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.

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 ™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 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 ongoing Phase 2 trial of TransCon CNP in children with achondroplasia, (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 and potential commercialization 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, 2018, which we filed with the SEC on April 3, 2019. 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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