Ascendis Pharma A/S Receives Orphan Designation for TransCon™ hGH for the Treatment of Pediatric Growth Hormone Deficiency in Europe

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COPENHAGEN, Denmark, Oct. 21, 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 the European Commission (EC) has granted Orphan Designation to TransCon Growth Hormone (hGH), a long-acting growth hormone therapy in development as a once-weekly treatment for pediatric growth hormone deficiency. TransCon hGH is designed to provide sustained release of unmodified growth hormone, the same growth hormone used in daily therapies, at a predictable rate over one week. Currently, there is no approved long-acting growth hormone treatment in Europe.

"This designation from the EC for TransCon Growth Hormone acknowledges the need in Europe for a long-acting therapy that can address the overall endocrine health of children with growth hormone deficiency," said Jan Mikkelsen, Ascendis Pharma's President and Chief Executive Officer. "We are on track to file our U.S. Biologics License Application (BLA) in the first half of 2020 and a marketing application (MAA) in Europe in the second half of 2020 for TransCon Growth Hormone. This is an important step towards providing a new treatment option for children with GHD, reflecting our commitment to developing therapies globally that address unmet patient needs and make a meaningful difference in patients' lives."

Orphan Designation 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 in the European Union (EU) and for which no satisfactory therapy is available. The medicine must also 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 and the designation supports future reimbursement and access to new therapies. Under certain conditions, market exclusivity for pediatric indications may be extended for an additional two years.

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 Pediatric Growth Hormone Deficiency (GHD)

Pediatric GHD is a serious orphan disease caused when the pituitary gland does not produce enough growth hormone. Children with GHD are not only characterized by short stature, but they also experience metabolic abnormalities, psychosocial challenges, cognitive deficiencies and poor quality of life.

For decades, the standard of care for GHD has been a daily subcutaneous injection of hGH, which improves growth and metabolic effects. For caregivers and patients, the treatment burden with daily injections is high, which leads to poor adherence and reduced overall treatment outcomes.

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) the expected timing of our BLA and MAA filings for TransCon hGH, (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.

Internal contact:	**Media contact:**	**Investor contact:**
Scott T. Smith	Ami Knoefler	Patti Bank
Chief Financial Officer	Head of Global Communications	Westwicke Partners
(650) 352-8389	(650) 739-9952	(415) 513-1284
ir@ascendispharma.com	ack@ascendispharma.com	patti.bank@westwicke.com

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