Ascendis Pharma A/S Receives Orphan Drug Designation for TransCon™ hGH as Treatment for Growth Hormone Deficiency in the United States

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COPENHAGEN, Denmark, April 15, 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 that the United States (U.S.) Food and Drug Administration (FDA) has granted Orphan Drug Designation (ODD) to TransCon hGH (*lonapegsomatropin*), as a treatment for growth hormone deficiency (GHD).

TransCon hGH is an investigational long-acting prodrug of somatropin (human growth hormone or hGH) that is being developed as a once-weekly treatment for GHD. TransCon hGH is designed to maintain the same mode of action as daily hGH therapies by releasing the same growth hormone molecule, somatropin. Currently, there is no approved long-acting growth hormone treatment on the market in the U.S. or Europe. TransCon hGH received Orphan Designation (OD) for the treatment of GHD in Europe from the European Commission (EC) in October 2019.

"TransCon hGH has now been granted orphan designation in both the U.S. and Europe, which we believe is an important acknowledgement of the global need for a long-acting therapy to address GHD and overall endocrine health," said Dana Pizzuti, M.D., Ascendis Pharma's Senior Vice President of Development Operations. "As the only long-acting growth hormone product in development that delivers somatropin, we believe TransCon hGH has significant potential to improve patients' lives. We remain on track to file our marketing applications for TransCon hGH in the U.S. and Europe, as planned, in the second and fourth quarters of this year, respectively."

The FDA grants orphan designation 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, and potentially may be safer or more effective than already approved products. Orphan designation 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 orphan 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.

About TransCon™ Technology

TransCon refers to "transient conjugation." The proprietary TransCon platform is an innovative technology to create new therapies designed to 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 (i.e., 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 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 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, 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 submit a BLA with the FDA in the second quarter of 2020 and a MAA in Europe in the fourth quarter of 2020 for TransCon hGH for the treatment of pediatric GHD, (ii) our ability to apply our TransCon platform to build a leading, fully integrated biopharma company, (iii) our expectations regarding our ability to create new and potentially best-in-class therapies and (iv) our product pipeline and expansion into additional therapeutic areas. 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; our ability to obtain additional funding, if needed, to support our business activities and the effects on our 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 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 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 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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