Ascendis Pharma A/S Submits Investigational New Drug Application to Initiate TransCon™ IL-2 β/γ Clinical Program

September 7, 2021

– TransCon IL-2 β/γ is an investigational long-acting prodrug designed to improve cancer immunotherapy through the sustained systemic release of an IL-2 variant with potential for prolonged activation of IL-2Rβ/γ with low C_{max} –

COPENHAGEN, Denmark, Sept. 07, 2021 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND), a biopharmaceutical company using its innovative TransCon technologies to potentially create new treatments to make a meaningful difference in patients' lives, today announced the submission of an investigational new drug (IND) application with the U.S. Food & Drug Administration (FDA) to initiate IL βelieγe (I'll Believe) Trial, a phase 1/2 clinical trial to evaluate TransCon IL-2 β/γ in patients with advanced cancer. TransCon IL-2 β/γ is an investigational long-acting prodrug designed to improve cancer immunotherapy by sustained exposure to an IL-2 variant that selectively activates the IL-2Rβ/γ, with minimal binding to IL-2Rα.

"This IND submission for our second clinical stage oncology product candidate represents another major milestone for Ascendis. Our understanding of the biology has guided us in designing a novel parent drug, which we have combined with our clinically validated TransCon platform. We believe this combination has the potential to overcome the known shortcomings of current IL-2 compounds, such as low potency, short half-life and high C_{max}. By solving the different elements related to efficacy and safety independently, we believe it will be possible to realize the full potential of the IL-2 pathway and create a potentially best-in-class cancer immunotherapy product," said Kennett Sprogøe, Ph.D., Ascendis Pharma's Head of Innovation and Research.

About TransCon ™Technologies

TransCon refers to "transient conjugation." Ascendis Pharma's proprietary TransCon platform is an innovative technology used to create new therapies that are designed to potentially optimize therapeutic effect, including efficacy, safety, and dosing frequency. TransCon molecules have three components: a 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, maintaining its original mode of action. 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 uses its TransCon technologies to create new and potentially best-in-class therapies.

The company's first commercial product based on the company's proprietary TransCon Technology is SKYTROFA ® (lonapegsomatropin-tcgd), approved by the U.S. FDA in August 2021 for the treatment of pediatric patients one year and older who weigh at least 11.5 kg (25.4 lb) and have growth failure due to inadequate secretion of endogenous growth hormone. Ascendis Pharma also has a pipeline of independent endocrinology rare disease and oncology product candidates in development and continues to expand into additional therapeutic areas to address unmet patient needs.

Ascendis is headquartered in Copenhagen, Denmark, with additional facilities in Heidelberg and Berlin, Germany, Palo Alto and Redwood City, California, and Princeton, New Jersey.

Please visit www.ascendispharma.com for global information or www.ascendispharma.us for U.S. information.

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 realize the full potential of the IL-2 pathway to create a potentially best-in-class cancer immunotherapy product; (ii) Ascendis' ability to apply its platform technology to build a leading, fully integrated biopharma company, (iii) Ascendis' product pipeline and expansion into additional therapeutic areas and (iv) Ascendis' expectations regarding its ability to utilize its TransCon technologies to create new and potentially best-in-class therapies. 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: dependence on third party manufacturers to supply lonapegsomatropin-tcgd, the SKYTROFA® Auto-Injector and other study drug for commercial sales and clinical studies; unforeseen safety or efficacy results in its oncology programs, lonapegsomatropin-tcgd, palopegteriparatide and TransCon CNP or other development programs; unforeseen expenses related to commercialization of lonapegsomatropin-tcgd and the further development of lonapegsomatropin-tcgd, expenses related to the development and potential commercialization of its oncology programs, palopegteriparatide and TransCon CNP or other development programs, selling, general and administrative expenses, other research and development expenses and Ascendis' business generally; delays in the development of its oncology programs, lonapegsomatropin-tcgd, palopegteriparatide 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 from 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' Annual Report on Form 20-F filed with the U.S. Securities and Exchange Commission (SEC) on March 10, 2021 and Ascendis' other future reports filed with, or submitted to, the SEC. 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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Source: Ascendis Pharma