Ascendis Pharma A/S Announces Filing of Investigational New Drug Application to Initiate TransCon™ TLR7/8 Agonist Clinical Program

December 30, 2020

- TransCon TLR7/8 Agonist is designed for intratumoral, sustained release of resiquimod with minimal systemic exposure, while inducing a potent anti-tumor response -

COPENHAGEN, Denmark, Dec. 30, 2020 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND), a biopharmaceutical company that utilizes its innovative TransCon™ technologies to create product candidates that address unmet medical needs, today announced the filing of an investigational new drug (IND) application with the U.S. Food and Drug Administration to initiate the clinical program of TransCon TLR7/8 Agonist.

TransCon TLR7/8 Agonist is a long-acting prodrug of resiquimod, a small molecule agonist of Toll-like receptors (TLR) 7 and 8. Administered as an intratumoral injection, TransCon TLR7/8 Agonist is designed to provide sustained activation of intratumoral antigen presenting cells driving tumor antigen presentation and induction of immune stimulatory cytokines in the tumor.

"The filing of our first oncology IND for TransCon TLR7/8 Agonist – which is designed to provide intratumoral, sustained release of resiquimod over several weeks from a single administration with minimal systemic exposure and potent immune response against cancer cells – is a major milestone for Ascendis," said Juha Punnonen, M.D., Ph.D., Senior Vice President and Head of Oncology at Ascendis Pharma. "We believe TransCon TLR7/8 Agonist represents a potential paradigm shift in the treatment of cancer through sustained release of an immunostimulatory molecule over several weeks inside the tumor, thereby employing the patients' own immune systems to destroy cancer cells with reduced risk of systemic adverse events."

About TransCon™ Technology

TransCon refers to "transient conjugation." The proprietary TransCon platform is an innovative technology to create new therapies that are designed to optimize therapeutic effect, including efficacy and safety and through 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 is expected to be maintained. TransCon technology can be applied broadly to proteins, peptides or small molecules in multiple therapeutic areas, and can be designed for systemic or localized release.

About Ascendis Pharma A/S

Ascendis Pharma is applying its innovative TransCon technologies to build a leading, fully integrated biopharmaceutical 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 biopharmaceutical company, (ii) Ascendis' product pipeline and expansion into additional therapeutic areas and (iii) 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: 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, selling, 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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