



Ascendis Pharma A/S Announces First Presentation of Preclinical Data Utilizing TransCon™ Technology in Oncology at SITC 2019

November 6, 2019

- *A single dose of TransCon TLR7/8 Agonist demonstrated sustained localized release of active drug over weeks and potent anti-tumor activity when administered directly into the tumor –*
- *TransCon-enabled product candidate provided approximately 25-fold longer effective half-life of resiquimod than unconjugated parent drug –*
- *First presentation of Ascendis oncology pipeline data at a scientific meeting –*

COPENHAGEN, Denmark, Nov. 06, 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 presentation of preclinical data for TransCon Toll-like Receptor (TLR) 7/8 Agonist, an oncology product candidate designed to provide sustained local release of resiquimod, a small molecule with immune-activating and anti-tumor properties, at the Society for Immunotherapy of Cancer (SITC) annual meeting in National Harbor, Maryland. The poster will be presented on Saturday, November 9, 2019.

"Our preclinical data showed that TransCon TLR7/8 Agonist, administered as a single intratumoral injection in animal studies, delivered sustained local release of resiquimod over weeks directly to the tumor site and demonstrated potent anti-tumor activity as a monotherapy, as well as in combination with interleukin-2 (IL-2). This highlights the potential for TransCon technologies to improve the safety and efficacy of validated therapeutic candidates in oncology," said Juha Punnonen, M.D., Ph.D., Ascendis Pharma's Senior Vice President and Head of Oncology. "Presenting these results for the first time at a premier scientific meeting is significant for us, reinforcing the potential applicability of TransCon technologies in the field of oncology."

TransCon TLR7/8 Agonist is a long-acting prodrug of resiquimod that is transiently conjugated to a hydrogel carrier via a TransCon linker. Administered as an intratumoral injection, TransCon TLR7/8 Agonist is designed to provide sustained release of unmodified resiquimod directly to the tumor. In preclinical studies, the effective half-life of resiquimod released from TransCon TLR7/8 Agonist has been found to be approximately 25-fold longer than the parent drug, resiquimod.

Presentation Details

Society for Immunotherapy of Cancer (SITC) 2019

Title

P676: Intratumoral delivery of TransCon TLR7/8 Agonist provides potent anti-tumor activity as a monotherapy and in combination with IL-2 while minimizing systemic cytokine induction

Date/Time

Saturday, November 9, 2019

Following the session, the poster will also be available on the company's website under [Selected Publications](#) in the Pipeline section.

About TransCon™ Oncology Programs

Ascendis Pharma is working to create potentially best-in-class oncology therapies by applying systemic and intratumoral TransCon technologies for clinically validated pathways to improve outcomes currently limited by suboptimal efficacy and systemic toxicity. Three oncology programs are currently in preclinical studies: TransCon TLR7/8 Agonist, TransCon IL-2 b/g and TransCon Vascular Endothelial Growth Factor-Tyrosine Kinase Inhibitor (VEGF-TKI).

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 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 rare disease endocrinology 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 ability to apply our platform technology to build a leading, fully integrated biopharma company, (ii) our expectations regarding our ability to create new and potentially best-in-class therapies and (iii) 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 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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Source: Ascendis Pharma A/S