Ascendis Pharma A/S Introduces Vision 3x3: A Strategic Roadmap Through 2025 to Achieve Sustainable Growth Using Multiple Approaches

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COPENHAGEN, Denmark, Jan. 07, 2019 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND), a biopharmaceutical company that utilizes its innovative TransCon ™ technologies to address significant unmet medical needs, today introduced Vision 3x3, the company's strategic roadmap through 2025 to achieve sustainable growth, at the 37th Annual J.P. Morgan Healthcare Conference in San Francisco. As part of that vision, the company also announced oncology as its second independent therapeutic area of focus.

"With the clinical validation of our three endocrinology rare disease product candidates, we have built a strong foundation to establish Ascendis as a leading rare disease company," said Jan Mikkelsen, Ascendis Pharma's President and Chief Executive Officer. "Having accomplished the fundamentals of our Vision 20/20, we are now implementing a new strategic roadmap through 2025, Vision 3x3. The goal is to position Ascendis as a leading biopharma company delivering sustainable growth through multiple approaches."

He continued, "Today, we are announcing that oncology is our next therapeutic focus, an area with major unmet medical needs. The cornerstone of our oncology pipeline strategy is to apply the same algorithm for product innovation that resulted in successful clinical validation of 3 out of 3 product candidates in endocrinology rare disease."

Vision 3x3: Sustainable Growth Through Multiple Approaches

The goal of Ascendis Pharma's Vision 3x3 strategic roadmap through 2025 is to achieve sustainable growth through multiple approaches:

- Obtain regulatory approval for three endocrinology rare disease products: TransCon hGH for pediatric growth hormone deficiency (GHD), TransCon PTH for adult hypoparathyroidism (HP), and TransCon CNP for achondroplasia.
- Create further growth of the company's endocrinology rare disease pipeline through:
  - Label expansion programs with the goal of obtaining 9 indications in total; and
  - Global clinical reach either directly or through partnerships.
- Build an integrated commercial business for the endocrinology rare disease franchise in North America and select European countries, and establish a global commercial presence with partners in other geographic areas.
- Create three independent therapeutic areas, each with a diversified pipeline built on TransCon technologies and the company's unique algorithm for product innovation. The company has established oncology as the next independent therapeutic area.

Endocrinology Rare Disease Pipeline: Selected Key Milestones

Ascendis Pharma has three endocrinology rare disease product candidates in clinical development based on its TransCon technology platform. The company expects each of these three independent programs to make significant clinical progress during 2019:

- ***TransCon hGH***: Top-line results from the phase 3 heiGHt Trial for TransCon hGH are expected in the first quarter and top-line results from the fliGHt Trial (switch) are expected in the second quarter. TransCon hGH is a once-weekly therapy in phase 3 development for children with GHD designed to deliver unmodified somatropin with the same benefits as daily human growth hormone (hGH). Ascendis believes this will address a limitation of the current standard of care – treatment compliance with daily injections of hGH – and improve treatment outcomes.
- ***TransCon PTH***: Top-line results from a phase 2 trial for TransCon PTH are expected in the fourth quarter. TransCon PTH is a long-acting prodrug of parathyroid hormone (PTH) in development as a once-daily replacement therapy for HP, a disease characterized by deficient or absent PTH. Unlike current therapies for HP, TransCon PTH is designed to achieve and maintain a steady concentration of PTH within the normal range for 24 hours a day.
- ***TransCon CNP***: A phase 2 trial of TransCon CNP in children with achondroplasia is expected to be initiated in the third quarter. TransCon CNP is a long-acting prodrug of CNP in development as a once-weekly therapy for children with achondroplasia, the most common form of dwarfism, for which there is no FDA-approved treatment. Achondroplasia is caused by a mutation in the fibroblast growth factor receptor 3 (FGFR3), which leads to an imbalance of the FGFR3 and C-natriuretic peptide (CNP) signaling pathways, causing decreased bone growth and other clinical complications. TransCon CNP is designed to restore this balance by providing sustained, long-term exposure to CNP.

Oncology: A New Therapeutic Area with Experienced Leadership

Ascendis also announced that it has established oncology as its second independent therapeutic area. To lead the oncology research and development programs, Ascendis recently appointed Juha Punnonen, MD, PhD, as Senior Vice President and Head of Oncology.

Dr. Punnonen is an experienced scientist, clinician, and biotechnology/pharma executive with a track record in building and managing R&D teams from early-stage research to development. He has more than 25 years of experience in the discovery, characterization and development of monoclonal antibodies, nanobodies, small molecules, cytokines, and immuno-modulatory proteins. Dr. Punnonen was most recently an Executive Director, Oncology Discovery Research, at Merck & Co., Inc. where he coordinated preclinical research and early development programs for Merck Oncology, including external clinical collaborations for Merck's anti-PD-1 antibody Keytruda ® (pembrolizumab). He has authored 89 scientific publications and 26 issued U.S. patents.

Ascendis Pharma's oncology division is based in the San Francisco Bay Area.

Presentation at J.P. Morgan Healthcare Conference

A live webcast of the J.P. Morgan presentation and associated Question and Answer session will be available today at 8:00 a.m. Pacific Time in the Investors and News section of the Ascendis Pharma website at: https://ascendispharma.gcs-web.com/events-and-presentations/upcoming-events. A webcast replay will also be available for 30 days.

The company's corporate presentation, including the slides from J.P. Morgan, will be available at the same link following commencement of the presentation.

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. 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 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 intention to obtain regulatory approval for our three endocrinology rare disease candidates, TransCon hGH, TransCon PTH and TransCon CNP, (ii) our intention to grow our endocrinology rare disease pipeline by pursuing label expansion programs, (iii) our intention to pursue oncology as our second of three independent therapeutic area of focus, (iv) our plans to release top-line data for our phase 3 heiGHt trial, (v) our plans to initiate our phase 2 TransCon PTH trial in patients with HP and our expectations regarding the timing of release of the results from such trial, (vi) our plans to initiate our phase 2 TransCon CNP trial for the treatment of children with achondroplasia, (vii) our intention to build an integrated commercial business for the endocrinology rare disease franchise in North America and select European countries, (viii) our ability to apply our platform technologies to build a leading, fully integrated biopharma company, (ix) our expectations regarding our ability to create potentially best-in-class therapies and (x) 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: our ability to apply our TransCon technology to the therapeutic area of oncology; 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, 2017, which we filed with the SEC on March 28, 2018. 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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