Ascendis Pharma A/S Provides Pipeline Update and Reviews Progress Towards Vision 3x3 at 38th Annual J.P. Morgan Healthcare Conference

January 13, 2020

COPENHAGEN, Denmark, Jan. 12, 2020 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND), a biopharmaceutical company that utilizes its innovative TransCon ™ technologies to address significant unmet medical needs, will provide an outlook for 2020 and review progress towards Vision 3x3, the company's strategic roadmap through 2025 to achieve sustainable growth at the 38 th Annual J.P. Morgan Healthcare Conference in San Francisco.

"2019 was a transformative year for Ascendis Pharma. For our endocrinology rare disease portfolio, we were successful in our first phase 3 trial for a TransCon product candidate, TransCon hGH, and we advanced two other programs globally into phase 2. In addition, we established oncology as our second therapeutic area with a highly-differentiated pipeline leveraging the TransCon technology," said Jan Mikkelsen, Ascendis Pharma's President and Chief Executive Officer. "As groundbreaking as 2019 was for Ascendis, it was only the beginning of our path to build a leading fully-integrated global biopharma company. We are on track with our Vision 3x3 goals to deliver multiple sources of sustainable growth as we seek to make a meaningful difference in patients' lives."

Pipeline Updates and 2020 Outlook

- ***TransCon hGH***: TransCon hGH is an investigational long-acting prodrug of human growth hormone (hGH) in phase 3 development as a once-weekly treatment for growth hormone deficiency (GHD). TransCon hGH releases unmodified somatropin and has demonstrated a statistically significant increase in height velocity compared to a daily hGH in the phase 3 heiGHt Trial:
    - Ascendis Pharma recently held two pre-BLA meetings with the U.S. Food and Drug Adminstration (FDA) to review its Chemistry, Manufacturing and Controls (CMC), and clinical/non-clinical packages for TransCon hGH as a potential treatment for pediatric GHD. The company is on track to file a Biologics License Application with the FDA in the second quarter. A Marketing Authorisation Application to the European Medicines Agency is planned to follow in the fourth quarter.
    - Long-term data presented from the ongoing enliGHten Trial (long-term extension) continued to demonstrate statistically superior growth of subjects treated with once-weekly TransCon hGH in the heiGHt Trial who continued into enliGHten, compared to those who started treatment with daily Genotropin® and switched to TransCon hGH after one year. The adverse event profile of TransCon hGH, which was comparable to Genotropin in the phase 3 heiGHt Trial, was consistent across the phase 3 clinical trials.
    - The company plans to submit regulatory filings to initiate a global, phase 3 clinical trial in adult GHD during the first quarter, and to initiate a trial in pediatric GHD in Japan during the fourth quarter.

- ***TransCon PTH***: TransCon PTH is an investigational long-acting prodrug of parathyroid hormone (PTH) in development as a once-daily replacement therapy for hypoparathyroidism (HP) designed to replace PTH at physiologic levels for 24 hours each day and fully address all aspects of the disease:
    - Following completion of screening of subjects in the recently expanded phase 2 PaTH Forward Trial, Ascendis intends to enroll approximately 55 subjects in the trial. The company expects to report top-line results from the trial around the end of March 2020, with six-month data from the open-label extension phase expected in the third quarter.
    - Preliminary data presented from the first eight subjects who completed four weeks of follow-up in the open-label extension portion of the phase 2 PaTH Forward Trial reinforce the company's target product profile for TransCon PTH as a promising new potential therapy for HP in the absence of standard of care.
    - The company plans to submit regulatory filings to initiate a global, phase 3 clinical trial in adults with HP during the fourth quarter.

- ***TransCon CNP***: TransCon CNP is an investigational long-acting prodrug of CNP in development as a therapy for children with achondroplasia, the most common form of dwarfism, for which there is no FDA-approved treatment. TransCon CNP is designed to provide continuous exposure of CNP at safe, therapeutic levels via a single, weekly subcutaneous dose:
    - Ascendis is conducting the phase 2 ACcomplisH Trial of TransCon CNP in children (ages 2-10 years) with

achondroplasia and plans to escalate sequential dose cohorts throughout 2020.
    - The company is expanding the TransCon CNP program in China through its strategic investment in VISEN Pharmaceuticals, with initiation of a second phase 2 trial in children with achondroplasia during the fourth quarter.

- ***Oncology***: Ascendis continues to advance a pipeline of multiple pre-clinical programs in oncology by applying both systemic and sustained localized TransCon technologies for clinically validated pathways:

    - Additional data from non-human primate studies demonstrated that a single dose of TransCon IL-2 b/g provided biased receptor binding and prolonged enhancement of lymphocyte counts, suggesting feasibility of every three week dosing and reduced risk of toxicity.
    - Ascendis Pharma's innovative TransCon technology for sustained localized release intratumorally (IT) was accepted to participate in the FDA's Emerging Technology Program. The program provides for enhanced interactions and dialogue with the FDA to discuss, identify and resolve potential technical and CMC regulatory questions related to the TransCon sustained IT programs prior to filing regulatory submissions.
    - The company plans to file an IND or equivalent for its first oncology program in 2020, furthering the goal to create best-in-class oncology therapeutics.

Presentation at J.P. Morgan Healthcare Conference on Monday, January 13

Live webcasts of the J.P. Morgan presentation and associated Question & Answer session will be available in the Investors and News section of the Ascendis Pharma website at:

https://investors.ascendispharma.com/events/event-details/38th-annual-jp-morgan-healthcare-conference

The presentation will begin at 8:00 a.m. Pacific Time, followed by the Question & Answer session at 8:30 a.m. A webcast replay will also be available for 30 days.

The company's corporate investor presentation and slides from the J.P. Morgan presentation are also available in the Investors and News section.

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 expectation to file a Biologics License Application with the U.S. FDA in the second quarter of 2020 and a Marketing Authorisation Application to the European Medicines Agency in the fourth quarter of 2020 for TransCon hGH, (ii) our plans to submit regulatory filings to initiate a global, phase 3 clinical trial in adult GHD during the first quarter of 2020, and to initiate a trial in pediatric GHD in Japan during the fourth quarter of 2020 for TransCon hGH, (iii) our intentions to enroll additional subjects in the expanded phase 2 PaTH Forward Trial and to report top-line results from the trial around the end of March 2020, and additional six-month data from the open label extension phase of the trial in the third quarter of 2020, (iv) our plans to submit regulatory filings to initiate a global, phase 3 clinical trial in adults with HP during the fourth quarter of 2020 for TransCon PTH, (v) our expectation to escalate sequential dose cohorts in the phase 2 ACcomplisH Trial of TransCon CNP throughout 2020, (vi) our expectation that VISEN Pharmaceuticals will initiate a second phase 2 trial in children with achondroplasia during the fourth quarter of 2020 for TransCon CNP, (vii) our plans to file an IND or equivalent for our first oncology program in 2020, (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, 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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