Ascendis Pharma A/S Provides Vision 3x3 Update at 39th Annual J.P. Morgan Healthcare Conference

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COPENHAGEN, Denmark, Jan. 10, 2021 (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, will provide an update on Vision 3x3 and the company's 2021 key milestones at the 39 th Annual J.P. Morgan Healthcare Conference.

"In 2019, we introduced Vision 3x3, the company's strategic roadmap through 2025, to build a leading biopharma company by achieving sustainable growth through multiple approaches. 2020 was a remarkable year of progress for Ascendis reflecting the dedication and commitment of our employees worldwide as we made meaningful strides to achieve our vision, and meet or accelerate completion of our key milestones," said Jan Mikkelsen, Ascendis Pharma's President and Chief Executive Officer.

"We expect 2021 will mark a number of key clinical and commercial milestones. By the end of the year, we expect to have five independent TransCon product candidates in clinical development leveraging TransCon technologies through our algorithm for product innovation. We expect to further advance our late stage endocrinology pipeline with the anticipated approval and launch in the United States and the approval in Europe of TransCon hGH for pediatric growth hormone deficiency, and to obtain phase 3 results for TransCon PTH in adult hypoparathyroidism. Development of TransCon CNP is progressing as planned with the recent initiation by VISEN Pharmaceuticals of a second phase 2 trial in patients with achondroplasia, the ACcomplisH China Trial, which provides for dose expansion at an effective dose determined from the ACcomplisH Trial. Finally, we expect to have the first two product candidates from our second therapeutic area of oncology in clinical development," Mr. Mikkelsen added.

Pipeline Updates

- ***TransCon hGH (lonapegsomatropin)***: Lonapegsomatropin is an investigational long-acting prodrug of somatropin (human growth hormone or hGH) currently under review for use in pediatric growth hormone deficiency (GHD) by the United States Food and Drug Administration (FDA) and the European Medicines Agency (EMA):
  - The company submitted its Biologic License Application to the FDA which has set a Prescription Drug User Fee Act (PDUFA) date for June 25, 2021. If approved on the PDUFA date, Ascendis anticipates commercial launch in the third quarter of 2021.
  - The company submitted its Marketing Authorisation Application (MAA) to the EMA in September 2020 and anticipates receiving MAA approval for lonapegsomatropin for use in pediatric GHD in the fourth quarter of 2021.
  - Ascendis anticipates completion of enrollment in foresiGHt, a global phase 3 trial evaluating the safety and efficacy of lonapegsomatropin in adult patients with GHD, by late 2021 or early 2022.

- ***TransCon PTH:*** TransCon PTH is an investigational long-acting prodrug of parathyroid hormone (PTH) in development as a potential once-daily replacement therapy for adult hypoparathyroidism (HP):
  - From the PaTH Forward phase 2 trial, 58 out of 59 randomized subjects continue receiving TransCon PTH in the open label extension (OLE) as of January 5, 2021.
  - After 26 weeks of follow-up in the PaTH Forward Trial, bone densitometry data from subjects treated with TransCon PTH demonstrated trends towards normalization of bone mineral density. In addition, quality of life as measured by the SF-36® Health Survey showed normalization of mean scores for all summary domains and all subdomains.
  - During the second quarter of 2021, Ascendis expects to provide a 12-month OLE update and plans to submit a Clinical Trial Notification for a clinical trial evaluating TransCon PTH for adult HP in Japan.
  - Top-line results from PaTHway, a phase 3 randomized, double-blind, placebo-controlled clinical trial in North America and Europe, investigating the safety, tolerability, and efficacy of TransCon PTH in adults with HP are expected in the fourth quarter of 2021.

- ***TransCon CNP:*** TransCon CNP, an investigational long-acting prodrug of C-type natriuretic peptide (CNP), as a potential therapeutic option for patients with achondroplasia (ACH):
  - Dosing of sequential ascending dose cohorts continues in the ACcomplisH Trial, a phase 2 randomized, double-blind, placebo-controlled clinical trial in North America, Europe, and Oceania.
  - VISEN Pharmaceuticals (VISEN), our strategic investment in China, received approval from China's Center for Drug Evaluation to conduct the ACcomplisH China Trial, a phase 2 randomized, double-blind, placebo-controlled clinical trial.
  - Ascendis expects that the ACcomplisH and ACcomplisH China Trials will enroll more than 120 subjects in total (ages 2-10), to be followed for 12 months.
  - The company plans to provide a TransCon CNP clinical program update in the fourth quarter of 2021.

- ***TransCon TLR7/8 Agonist:*** TransCon TLR7/8 Agonist is an investigational long-acting prodrug of resiquimod, a small molecule agonist of Toll-like receptors (TLR) 7 and 8 designed to provide sustained activation of intratumoral antigen-presenting cells driving tumor antigen presentation and induction of immune stimulatory cytokines for weeks or months with a single intratumoral injection:
  - Submitted IND to the FDA in December 2020 to initiate clinical program.
  - During the second quarter of 2021, following monotherapy evaluation, the company plans to initiate TransCon TLR7/8 Agonist dose escalation in combination with a checkpoint inhibitor.

- Initial monotherapy dose escalation results are expected in the fourth quarter of 2021.

- ***TransCon IL-2 β/γ:*** TransCon IL-2 β/γ is an investigational novel long-acting prodrug of IL-2 β/γ designed to selectively bind and activate the IL-2Rβ/γ:
  - Ascendis reported pre-clinical data for TransCon IL-2 β/γ demonstrating:
    - Independently optimized receptor bias and potency as well as pharmacokinetics to create a potentially best-in-class IL-2 product.
    - An effective half-life of approximately 32 hours in non-human primates (NHP).
    - Following a single dose of TransCon IL-2 β/γ in NHP, observed potential best-in-class expansion and activation of cytotoxic lymphocyte subsets with minimal effect on eosinophils, minimal IL-5 and IL-6 levels which suggests low risk of vascular leak syndrome.
  - The company expects to submit an IND or similar for TransCon IL-2 β/γ in the third quarter of 2021.

Global Endocrinology Rare Disease Commercial Strategy

In anticipation of regulatory approvals for lonapegsomatropin in the United States and Europe, Ascendis is establishing a global commercial presence through multiple approaches. This global commercial approach will be laying the groundwork for future potential endocrinology rare disease launches as TransCon PTH and TransCon CNP advance in clinical development.

The company's US commercial organization is in place and commercial manufacturing is ongoing for the potential launch of lonapegsomatropin in pediatric GHD planned for the third quarter of 2021 after anticipated regulatory approval.

In addition, Ascendis is preparing for potential commercialization in Europe, building an integrated organization in select European countries and evaluating established distribution channels in other European countries to be ready for the anticipated MAA approval of lonapegsomatropin in pediatric GHD in the fourth quarter of 2021.

Lastly, Ascendis plans to serve patients in other regions around the world through established sales and distribution networks and following applicable regulatory approvals. The company has invested in VISEN in Greater China and plans to partner in Japan and South Korea when appropriate.

Presentation at J.P. Morgan Healthcare Conference on Monday, January 11th

Live webcast of the J.P. Morgan presentation will be available on the Events & Presentations section of the investor relations webpage at https://investors.ascendispharma.com/events-and-presentations. The presentation will begin at 11:40 a.m. ET. A webcast replay will also be available for 30 days.

The company's slides from the J.P. Morgan presentation will be available on the investor relations website.

About TransCon™ Technology Platform

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. The TransCon technology platform 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, Palo Alto and Redwood City, California, and Princeton, New Jersey.

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' expectation that 2021 will mark a number of key milestones (ii) Ascendis' expectation that by the end of 2021 it will have five independent TransCon product candidates in clinical development, (iii) Ascendis' expectations regarding the timing of potential approval and launch of TransCon hGH (lonapegsomatropin), (iv) Ascendis' expectations regarding when it will obtain phase 3 results for TransCon PTH in adult hypoparathyroidism, (v) Ascendis' expectation that it will have the first two product candidates from its second therapeutic area of oncology in clinical development in 2021, (vi) Ascendis' ability to apply its platform technology to build a leading, fully integrated biopharmaceutical company, (vii) Ascendis' product pipeline and expansion into additional therapeutic areas and (viii) 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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