



Ascendis Pharma A/S Provides Update on Vision 3x3 Strategic Roadmap at 40th Annual J.P. Morgan Healthcare Conference

January 10, 2022

COPENHAGEN, Denmark, Jan. 10, 2022 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND) today announced that the Company will today provide an update on its Vision 3x3 and planned 2022 key milestones at the 40th Annual J.P. Morgan Healthcare Conference. Details of the update are outlined below, and CEO Jan Mikkelsen will give a virtual presentation to attendees from 3:00-3:40 p.m. Eastern Time.

"We took major steps in 2021 to becoming a viable, sustainable, and profitable biopharmaceutical company," said Jan Mikkelsen, Ascendis Pharma's President and Chief Executive Officer. "A strong flow of clinical results and our first commercial TransCon[™] product showcased how we are leveraging our technology platform to build a leading global biopharmaceutical company able to successfully meet the needs of patients with differentiated product candidates."

"This year, I am happy to report that our U.S. launch of SKYTROFA[®] (lonapegsomatropin-tcgd), the first U.S. FDA-approved once-weekly treatment for pediatric growth hormone deficiency is off to a strong start," continued Mr. Mikkelsen. "In only two short months since launch, we have already seen 10% of our target prescribers' writing prescriptions for SKYTROFA instead of daily growth hormone."

"During 2022, we look forward to sharing multiple clinical data readouts across our high-value endocrinology rare disease and oncology portfolios," continued Mr. Mikkelsen. "We believe the clinical readouts will demonstrate our ability to deliver therapies that make a meaningful impact on patients' lives. In addition, we look forward to announcing a third independent therapeutic area with its own diversified pipeline in the fourth quarter of 2022."

Pipeline Updates

- **TransCon hGH:** TransCon hGH is an investigational once-weekly prodrug designed to deliver somatropin over a one-week period. TransCon hGH is approved by the FDA in the U.S. under the brand name SKYTROFA (lonapegsomatropin-tcgd) for the treatment of pediatric patients one year and older who weigh at least 11.5 kg and have growth failure due to inadequate secretion of endogenous growth hormone.
 - In mid-October, the Company commercially launched in the U.S. TransCon hGH under the brand name SKYTROFA. Since launch, physician enthusiasm for SKYTROFA is reflected by an increase in prescriptions, submission of formulary exceptions, and repeat prescribers. During the fourth quarter, 369 SKYTROFA prescriptions were written by 139 targeted prescribers, which includes 42% repeat prescribers.¹
 - In November 2021, the Company received a positive opinion from the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency for TransCon hGH for patients with pediatric growth hormone deficiency. The European Commission's approval of the Marketing Authorisation Application (MAA) is expected by the end of January 2022.
 - During the second quarter of 2022, the Company plans to submit a protocol to the FDA for TransCon hGH in Turner Syndrome subjects.
 - Ascendis is targeting completion of enrollment in foresiGHt, a global Phase 3 trial evaluating the safety and efficacy of TransCon hGH in adult patients with growth hormone deficiency during the second quarter of 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):
 - During the first quarter of 2022, top-line results are expected 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.
 - If the Phase 3 PaTHway Trial results are positive, Ascendis plans to submit a New Drug Application (NDA) to the FDA in the third quarter of 2022 followed by a MAA submission to the EMA in the fourth quarter of 2022.
 - Top-line results from PaTHway Japan, a single-arm Phase 3 trial of TransCon PTH in a minimum of 12-Japanese subjects with HP are expected in the third quarter of 2022.
 - Initiation of a pediatric HP program is planned for the fourth quarter of 2022.
- **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):

- Top-line data from the ACcomplish Trial, a Phase 2 randomized, double-blind, placebo-controlled clinical trial in North America, Europe, and Oceania in subjects with achondroplasia (age 2–10) are expected in the fourth quarter of 2022.
- During the second quarter of 2022, the Company plans to file an Investigational New Drug (IND) application or similar for the ACcomplish Infants Trial in subjects with achondroplasia (age 0–2).
- **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:
 - Enrollment continues in transcendIT-101. Top-line data from monotherapy and combo-therapy dose escalation expected in the third quarter of 2022.
- **TransCon IL-2 β/γ :** TransCon IL-2 β/γ is an investigational long-acting prodrug designed to improve cancer immunotherapy by sustained exposure to an IL-2 variant that selectively activates the IL-2R β/γ , with minimal binding to IL-2R α :
 - Top-line monotherapy data from the IL- β elieye Trial are expected in the fourth quarter of 2022.
 - The Company expects to dose the first patient in the combo-therapy and dose escalation arm of the IL- β elieye Trial in the first quarter of 2022.
- **TransCon TLR7/8 Agonist and TransCon IL-2 β/γ Combinations:**
 - During the fourth quarter of 2022, the Company plans to submit an IND or similar for Phase 2 cohort expansion for TransCon TLR7/8 Agonist and TransCon IL-2 β/γ .

Presentation at J.P. Morgan Healthcare Conference on Monday, January 10th

The live webcast of the J.P. Morgan presentation will be available on the Investors & News section of the Ascendis Pharma website at www.ascendispharma.com. A webcast replay will be available on this website shortly after conclusion of the event for 30 days. The Company's slides from the J.P. Morgan presentation also will also be available on the Investor Relations website.

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 uses its TransCon technologies to create new and potentially best-in-class therapies. Ascendis is headquartered in Copenhagen, Denmark, and has additional facilities in Heidelberg and Berlin, Germany; Palo Alto and Redwood City, California; and Princeton, New Jersey. Please visit www.ascendispharma.com to learn more.

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) the expected timing of Ascendis' announcement of a third independent therapeutic area, (ii) the expected timing of the European Commission's final decision on Ascendis' Marketing Authorization Application for TransCon hGH, (iii) Ascendis' plans to submit a protocol to the FDA for TransCon hGH in Turner Syndrome subjects, (iv) the expected timing for completion of enrollment in the foresiGHt Trial, (v) the expected timing of planned regulatory filings for TransCon PTH to the FDA and the EMA, (vi) the expected timing of top-line results from the Phase 3 PaTHway Trial and the PaTHway Japan Trial, (vii) the expected timing of initiation of a pediatric HP program for TransCon PTH, (viii) the expected timing of planned regulatory filings for TransCon CNP, (ix) the expected timing of top-line results from the ACcomplish Trial, (x) the expected timing of dose escalation data for transcendIT-101, (xi) the expected timing of the IL- β elieye Trial, (xii) the expected timing of top-line monotherapy data for the IL- β elieye Trial, (xiii) the expected timing of planned regulatory filings for TransCon TLR7/8 Agonist and TransCon IL-2 β/γ , (xiv) Ascendis' ability to apply its platform technology to build a leading, fully integrated biopharma company, and (xv) Ascendis' use of 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: dependence on third party manufacturers and distributors to supply TransCon hGH, and other study drug for commercial sales in the U.S. and clinical studies; unforeseen safety or efficacy results in its oncology programs, TransCon hGH, TransCon PTH and TransCon CNP or other development programs; unforeseen expenses related to commercialization of lonapegsomatropin-tcgd in the U.S., the co-pay program, and the further development of TransCon hGH, 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 from 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' Annual Report on Form 20-F filed with the U.S. Securities and Exchange Commission (SEC) on March 10, 2021 and Ascendis' other future reports filed with, or submitted to, the SEC. Forward-looking statements do not reflect the potential impact of any future 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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¹ *Fourth quarter SKYTROFA prescription figures have not been verified by any third party and represent the Company's estimates. The Company assumes no obligation to update them.*



Source: Ascendis Pharma