Ascendis Pharma A/S Virtual R&D Program Update Highlights Continued Development Across R&D Pipeline

December 14, 2021 at 8:00 AM EST

— TransCon™ PTH Phase 3 topline results expected in Q1 2022 and, if positive, planned NDA submission in Q3 2022 followed by expected MAA submission in Q4 2022

— Enrollment completed in Phase 2 ACcomplisH Trial of TransCon CNP; blinded data informed dose selection of 50 and 100 µg/kg/week in dose expansion cohorts for ACcomplisH China Trial

— Early signs of clinical activity in three out of three efficacy-evaluable cancer patients treated with TransCon TLR7/8 Agonist as monotherapy or in combination with pembrolizumab

COPENHAGEN, Denmark, Dec. 14, 2021 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND) today plans to provide an update on two of its investigational endocrinology rare disease development programs, TransCon PTH for hypoparathyroidism, and TransCon CNP for achondroplasia, and on one of its investigational oncology product candidates, TransCon TLR7/8 Agonist at the Company's virtual R&D Program Update.

"Today marks an important milestone for Vision 3x3, our strategic roadmap for achieving sustainable growth and building a leading global biopharma company. In addition to providing updates on our Endocrinology Rare Disease pipeline, we are also presenting the first early-stage clinical data from our second therapeutic area, Oncology," said Jan Mikkelsen, Ascendis Pharma's President and Chief Executive Officer. "I am very encouraged that TransCon TLR7/8 Agonist is so far performing as designed, demonstrating early signs of anti-tumor activity with a well-tolerated safety profile as monotherapy or in combination with pembrolizumab."

Ascendis' management outlined continued clinical advancement on each of the three rare disease development programs. Highlights include:

- Update from ongoing TransCon PTH development program:
  - Reviewed clinical data from ongoing Phase 2 PaTH Forward Trial
  - Topline results from the Phase 3 PaTHway Trial expected in Q1 2022
  - Planned NDA submission in Q3 2022 for the treatment of adults with hypoparathyroidism
  - Topline results from the PaTHway Japan Trial expected in Q3 2022
  - Planned MAA submission in Q4 2022 for the treatment of adults with hypoparathyroidism
  - Initiation of the pediatric hypoparathyroidism program planned for Q4 2022

- Update from ongoing TransCon CNP development program:

  - Interim Phase 2 ACcomplisH Trial Update
    - ACcomplisH enrollment complete (N=57)
    - Preliminary PK data demonstrated that TransCon CNP provided continuous and dose-dependent exposure with a half-life of ~110 hours mirroring Phase 1 data
    - Well tolerated in children with achondroplasia with up to 65 weeks follow-up, with mean orthostatic vital signs unchanged
  - Interim blinded data from the ACcomplisH Trial informed dose selection of 50 and 100 µg/kg/week for the ACcomplisH China Trial
  - ACcomplisH Infants Trial (0-2 years of age) IND or equivalent submission planned for Q2 2022
  - ACcomplisH Trial topline unblinded data anticipated in Q4 2022

- Interim update from ongoing TransCon TLR7/8 Agonist first-in-human trial (transcendIT-101):
  - Early signs of clinical activity in three out of three efficacy-evaluable cancer patients treated with TransCon TLR7/8 Agonist as monotherapy or in combination with pembrolizumab
  - PK data indicate sustained release of active drug with a half-life of ~7 days and low systemic exposure providing a wide safety margin
  - Consistent and robust immune activation in tumor tissue was observed for at least 7 days post dose
  - In the safety-evaluable population observed to date (n=8), TransCon TLR7/8 Agonist was well-tolerated with no dose-limiting toxicities or drug-related systemic side effects; the only related adverse events reported were transient, mild injection site-related reactions (Grade 1/2)
  - transcendIT-101 dose expansion expected to start enrollment in Q2 2022

Virtual R&D Program Update Conference Call & Webcast information

Date	Tuesday, December 14, 2021
Time	9:00 a.m. to 11:30 a.m. Eastern Time
Dial In (U.S.)	877-870-9135
Dial In (International)	646-741-3167
Access Code	2169055

A live webcast of the event will be available on the Investors & News section of the Ascendis Pharma website at https://ascendispharma.com. A webcast replay will be available on the site shortly after conclusion of the event and will stay available for 30 days.

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 planned regulatory filings for TransCon PTH with the U.S. FDA and European Commission, (ii) the expected timing of topline results from the Phase 3 PaTHway Trial and the PaTHway Japan Trial, (iii) the expected timing of initiation of a pediatric hypoparathyroidism program for TransCon PTH, (iv) the expected timing of planned regulatory filings for TransCon CNP, (v) the expected timing of topline results from the ACcomplisH Trial, (vi) the expected timing for enrollment for transcendIT-101 dose expansion, (vii) Ascendis' ability to apply its platform technology to build a leading, fully integrated biopharma company, and (viii) 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, the SKYTROFA® Auto-Injector 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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