



Ascendis Pharma A/S Reports Full-Year 2019 Financial Results

April 1, 2020

– On track with 2020 corporate milestones –

– Top-line data from PaTH Forward phase 2 trial on track for mid-April –

– Conference call today at 4:30 p.m. Eastern Time –

COPENHAGEN, Denmark, April 01, 2020 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND), a biopharmaceutical company that utilizes its innovative TransCon™ technologies to address unmet medical needs, today announced financial results for the full year ended December 31, 2019.

"Following a transformative 2019, Ascendis remains on track with our corporate milestones for an even stronger 2020," said Jan Mikkelsen, Ascendis Pharma's President and Chief Executive Officer. "Our flexible, global workforce, corporate structure and supply chain, supported by our information technology infrastructure, have allowed our teams to continue work unabated, guided by our corporate values and vision, and adapt to the global pandemic. We look forward to reporting our top-line results for the TransCon PTH PaTH Forward Trial mid-April, and submitting our marketing applications for TransCon hGH in the United States (U.S.) and Europe, as planned, in the second and fourth quarters, respectively. I would like to acknowledge the extraordinary commitment of our employees, the patients in our clinical trials, and the teams at our investigator sites to move forward during this time."

Corporate Highlights & Progress

- **TransCon hGH:** TransCon hGH is an investigational long-acting prodrug of somatotropin (human growth hormone or hGH) that releases somatotropin in phase 3 development as a once-weekly treatment for growth hormone deficiency (GHD):
 - Following discussions with the U.S. Food and Drug Administration (FDA), submitted an Investigational New Drug amendment to initiate the global, phase 3 foresiGHt Trial in adult GHD. The foresiGHt Trial is expected to begin enrollment later this year.
 - Held two pre-BLA meetings with FDA to review the 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 for planned submission of a Biologics License Application (BLA) to the FDA in the second quarter of 2020 and a Marketing Authorisation Application (MAA) to the European Medicines Agency in the fourth quarter of 2020.
 - Received Orphan Designation from the European Commission for TransCon hGH in pediatric GHD.
 - Advanced TransCon hGH in Greater China following initiation of a phase 3 trial for TransCon hGH in pediatric GHD by VISEN Pharmaceuticals, the company's strategic investment to establish global reach in Greater China.
- **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 address both short-term symptoms and long-term complications of the disease:
 - Completed enrollment of 59 subjects in the PaTH Forward Trial, a global phase 2 trial evaluating the safety, tolerability and efficacy of TransCon PTH in adult HP subjects.
 - The company expanded the trial in November 2019 to expedite the enrollment of subjects affected by the NATPARA® recall. Final enrollment of PaTH Forward included 17 subjects previously treated with NATPARA.
 - The goal of PaTH Forward is to identify a starting dose (15, 18, or 21 µg per day) for a pivotal phase 3 trial, establish a titration regimen for complete withdrawal of standard of care (i.e., active vitamin D and calcium supplements), and evaluate TransCon PTH control of serum and urinary calcium.
 - Following the one-month blinded portion of PaTH Forward, subjects entered an open-label extension where they will receive a customized maintenance dose of TransCon PTH (6 to 30 µg per day) titrated to optimize their calcium control and evaluated on the primary composite endpoint, both as planned for phase 3. Fifty-nine subjects completed the blinded portion, and 58 subjects continued in the open-label extension, with one subject withdrawing for reasons unrelated to safety or efficacy of the study drug.
 - The company expects to report top-line results from the one-month blinded portion of PaTH Forward in mid-April, with six-month data from the open-label extension expected during the third quarter of 2020.
- **TransCon CNP:** TransCon CNP is an investigational long-acting prodrug of C-type natriuretic peptide (CNP) in development as a therapy for children with achondroplasia (ACH), 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:
 - The ACcomplish Trial is a global, phase 2, randomized, double-blind, placebo-controlled trial designed to evaluate the

safety and efficacy of TransCon CNP at escalating doses in children (ages 2 to 10 years) with ACH. The company continues to work towards escalating sequential dose cohorts throughout the year, while ensuring the safety of subjects during the current pandemic and access to investigator site staff for future monitoring visits.

-- VISEN Pharmaceuticals remains on track to initiate a phase 2 trial in children with ACH during the fourth quarter of 2020.

- **Oncology:** The company advanced its pipeline of multiple programs in oncology for clinically validated pathways, including TransCon IL-2 b/g, TransCon TLR7/8 Agonist and TransCon VEGF-TKI, with the goal to file an IND or equivalent for the company's first oncology candidate in the fourth quarter of 2020.
- Corporate milestones remain on track for 2020 despite the current global pandemic. The company continues to monitor and adapt to the impact of COVID-19 and expects to provide further updates to the investment community if the update is warranted.
- Ended 2019 with cash and cash equivalents of €598.1 million.

Full Year 2019 Financial Results

For the full year 2019, Ascendis Pharma reported a net loss of €218.0 million, or €4.69 per share (basic and diluted) compared to a net loss of €130.1 million, or €3.17 per share (basic and diluted) for the same period in 2018.

Revenue for 2019 was €13.4 million compared to €10.6 million during 2018. The increase reflects recognition of revenue related to our strategic investment in VISEN Pharmaceuticals.

Research and development (R&D) costs for 2019 were €191.6 million compared to €140.3 million during 2018. Higher R&D costs in 2019 reflect an increase in personnel and external costs for development and manufacturing of TransCon hGH, TransCon PTH and TransCon CNP, and other research programs, including oncology.

General and administrative expenses for 2019 were €48.5 million compared to €25.1 million during 2018. The increase is primarily due to higher personnel-related costs and other increasing costs of preparing to become a commercial organization.

As of December 31, 2019, Ascendis had cash and cash equivalents of €598.1 million compared to €277.9 million as of December 31, 2018. As of December 31, 2019, Ascendis Pharma had 47,985,837 ordinary shares outstanding.

Conference Call and Webcast information

Ascendis Pharma will host a conference call and webcast today at 4:30 p.m. Eastern Time (ET) to discuss its full year 2019 financial results. Details include:

| | |
|--------------------------------|---------------|
| Date | April 1, 2020 |
| Time | 4:30 p.m. ET |
| Dial In (U.S.) | 844-290-3904 |
| Dial In (International) | 574-990-1036 |
| Access Code | 7387576 |

A live webcast of the conference call will be available on the Investors and News section of the Ascendis Pharma website at www.ascendispharma.com. A webcast replay will also be available on this website shortly after conclusion of the event 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 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 plans to submit a BLA with the FDA in the second quarter of 2020 and a MAA in Europe in the fourth quarter of 2020 for TransCon hGH, (ii) our phase 2 ACcomplish Trial of TransCon CNP in children with achondroplasia, (iii) our ability to apply our TransCon platform to build a leading, fully integrated biopharma company, (iv) our expectations regarding our ability to create new and potentially best-in-class therapies and (v) our product pipeline and expansion into additional therapeutic areas. 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: unforeseen safety or efficacy results in our TransCon hGH, TransCon PTH and TransCon CNP or other development programs; unforeseen expenses related to the development and potential commercialization

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 most recent Annual Report on Form 20-F, which we have filed with the SEC. 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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FINANCIAL TABLES FOLLOW

Ascendis Pharma A/S

Consolidated Statements of Profit or Loss and Other Comprehensive Income / (loss)

(In EUR'000s, except share and per share data)

| | Year ended December 31, | |
|--|-------------------------|-------------------|
| | 2019 | 2018 |
| Revenue | 13,375 | 10,581 |
| Research and development costs | (191,621) | (140,281) |
| General and administrative expenses | (48,473) | (25,057) |
| Operating profit / (loss) | (226,719) | (154,757) |
| Share of profit / (loss) of associate | (8,113) | (321) |
| Finance income | 17,803 | 24,714 |
| Finance expenses | (1,221) | (127) |
| Profit / (loss) before tax | (218,250) | (130,491) |
| Tax on profit / (loss) for the year | 234 | 394 |
| Net profit / (loss) for the year | (218,016) | (130,097) |
| Other comprehensive income / (loss) | | |
| <i>Items that may be reclassified subsequently to profit or loss:</i> | | |
| Exchange differences on translating foreign operations | (37) | 17 |
| Other comprehensive income / (loss) for the year, net of tax | (37) | 17 |
| Total comprehensive income / (loss) for the year, net of tax | (218,053) | (130,080) |
| Profit / (loss) for the year attributable to owners of the Company | (218,016) | (130,097) |
| Total comprehensive income / (loss) for the year attributable to owners of the Company | (218,053) | (130,080) |
| | EUR | EUR |
| Basic and diluted earnings / (loss) per share | (4.69) | (3.17) |
| Number of shares used for calculation (basic and diluted) | 46,506,862 | 41,085,237 |

Ascendis Pharma A/S

Consolidated Statements of Financial Position

(In EUR'000s)

| | December 31, 2019 | December 31, 2018 |
|-------------------------------|-------------------|-------------------|
| Assets | | |
| Non-current assets | | |
| Intangible assets | 3,495 | 3,495 |
| Property, plant and equipment | 45,069 | 4,325 |
| Investment in associate | 15,538 | 17,083 |

| | | |
|-------------------------------------|----------------|----------------|
| Deposits | 1,463 | 1,158 |
| | 65,565 | 26,061 |
| Current assets | | |
| Receivable from associate | 804 | - |
| Trade receivables | - | 6 |
| Other receivables | 3,136 | 1,775 |
| Prepayments | 7,648 | 12,415 |
| Income taxes receivable | 1,473 | 849 |
| Cash and cash equivalents | 598,106 | 277,862 |
| | 611,167 | 292,907 |
| Total assets | 676,732 | 318,968 |
| Equity and liabilities | | |
| Equity | | |
| Share capital | 6,443 | 5,659 |
| Distributable equity | 590,671 | 274,391 |
| Total equity | 597,114 | 280,050 |
| Non-current liabilities | | |
| Lease liabilities | 30,720 | - |
| Other payables | 908 | - |
| | 31,628 | - |
| Current liabilities | | |
| Lease liabilities | 5,899 | - |
| Contract liabilities | 858 | 6,902 |
| Trade payables | 27,765 | 19,740 |
| Other payables | 13,349 | 12,267 |
| Income taxes payable | 119 | 9 |
| | 47,990 | 38,918 |
| Total liabilities | 79,618 | 38,918 |
| Total equity and liabilities | 676,732 | 318,968 |

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Source: Ascendis Pharma