



Ascendis Pharma A/S Reports Full Year 2018 Financial Results

April 3, 2019

– Rare disease endocrinology pipeline continues to advance, with significant 2019 milestones anticipated –

– Phase 3 heiGHt Trial results showcase potential of TransCon™ technologies and support company algorithm for product innovation –

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

COPENHAGEN, Denmark, April 03, 2019 (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, 2018.

"We have achieved clinical validation for all three of our rare disease endocrinology programs, showing we can translate product concepts into therapeutic candidates that make a meaningful difference for patients," said Jan Mikkelsen, Ascendis Pharma's President and Chief Executive Officer. "We have demonstrated that our unique algorithm and approach to product innovation are effective, and we are now applying it in our second therapeutic area of oncology. With our phase 3 results for TransCon hGH, we are now even closer to offering a highly differentiated therapy – reflecting our values and commitment to delivering on our mission."

Corporate Highlights & Progress

- Presented positive top-line data from the phase 3 heiGHt Trial for TransCon Growth Hormone (hGH), a once-weekly growth hormone therapy for the treatment of pediatric growth hormone deficiency (GHD). Results of the trial demonstrated that TransCon hGH has comparable safety and tolerability with superior efficacy to a daily hGH (Genotropin®). These data were highlighted as an oral presentation at ENDO 2019, the annual meeting of the Endocrine Society.
- Completed last patient visit in the fliGHt Trial, which is evaluating TransCon hGH in subjects who switch from daily hGH; on track to report top-line data in the second quarter of 2019. Including long-term safety data from the ongoing enliGHten Trial, the company plans a clinical database lock for the TransCon hGH phase 3 program in the third quarter of 2019, and intends to submit a Biologics License Application (BLA) with the U.S. Food and Drug Administration (FDA) for TransCon hGH to treat pediatric GHD in the first half of 2020.
- Initiated PaTH Forward, a global phase 2 trial designed to evaluate the safety, tolerability and efficacy of TransCon PTH in adult subjects with hypoparathyroidism (HP). The trial is also evaluating a titration regimen for the complete withdrawal of standard of care (i.e., active vitamin D and calcium supplements). TransCon PTH is a long-acting prodrug of parathyroid hormone (PTH) in development as a potential replacement therapy for HP designed to achieve and maintain a steady concentration of PTH within the normal range for 24 hours a day. The company expects to report top-line results from PaTH Forward in the fourth quarter of 2019.
- Reported preliminary phase 1 data for TransCon CNP, a long-acting prodrug of C-type natriuretic peptide (CNP). Ascendis expects to initiate a phase 2 trial in children with achondroplasia in the third quarter of this year. In addition, the company is conducting the ACHieve Study, a natural history study that aims to provide important observational insights into the experience of children living with achondroplasia.
- Introduced Vision 3x3, the company's strategic roadmap through 2025 to achieve sustainable growth through multiple approaches and established oncology as a second independent therapeutic area of focus.
- Appointed Sigurd Okkels, Ph.D., as Senior Vice President of Product Development. Dr. Okkels brings more than 20 years of experience in international leadership positions at Novo Nordisk, Profound Pharma/Maxygen, Nycomed, Takeda and, most recently, as an independent consultant. He has been responsible for numerous pharmaceutical projects at all stages of development, from preclinical and launch to life cycle management. Dr. Okkels holds a Ph.D. in Biochemistry and Molecular Biology.
- Subsequent to year-end 2018, Ascendis completed an underwritten public offering of 4,791,667 American Depositary Shares ("ADSs"), each of which represents one ordinary share of Ascendis, at a price to the public of \$120.00 per ADS. The company received net proceeds from the offering of approximately \$539.8 million, or €476.9 million at the date of closing, after deducting the underwriters' commissions and the company's estimated offering expenses.
- Ended 2018 with cash and cash equivalents of €277.9 million.

Full Year 2018 Financial Results

For the full year 2018, Ascendis Pharma reported a net loss of €130.1 million, or €3.17 per share (basic and diluted) compared to a net loss of €123.9

million, or €3.68 per share (basic and diluted) during the same period in 2017.

Revenue for 2018 was €10.6 million compared to €1.5 million during 2017. The increase in revenue reflects the sale of licenses recognized as part of forming the strategic investment in VISEN Pharmaceuticals.

Research and development (R&D) costs for 2018 were €140.3 million compared to €99.6 million during 2017. Higher R&D costs in 2018 reflect an increase in clinical trial costs for the phase 3 clinical program for TransCon hGH, costs for preparation of the manufacturing of validation batches required as part of the regulatory approval process, ongoing development of our proprietary auto-injector for use with TransCon hGH, costs associated with the company's phase 1 clinical trials of TransCon PTH and TransCon CNP, phase 2 enabling activities for both programs, and increased headcount in R&D functions.

General and administrative expenses for the 2018 year were €25.1 million compared to €13.5 million during 2017. The increase is primarily due to higher personnel costs and site costs, increased professional fees, recruitment costs and initial costs of building out a commercial organization.

As of December 31, 2018, the company had cash and cash equivalents of €277.9 million compared to €195.4 million in the prior year period. As of December 31, 2018, Ascendis Pharma had 42,135,448 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 2018 financial results. Details include:

| | |
|--------------------------------|--------------------------|
| Date | Wednesday, April 3, 2019 |
| Time | 4:30 p.m. ET |
| Dial In (U.S.) | 844-290-3904 |
| Dial In (International) | 574-990-1036 |
| Access Code | 6090229 |

A live audio webcast of the event will be available in 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 rare disease endocrinology product candidates in clinical development and has established oncology as its second therapeutic area of focus. Additionally, Ascendis Pharma has multi-product collaborations with Sanofi in diabetes and Genentech in the field of ophthalmology and continues to expand into additional therapeutic areas for both internal and external development.

Ascendis is headquartered in Copenhagen, Denmark, with 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 for TransCon hGH to treat pediatric GHD in the first half of 2020, (ii) our plans to report top-line results from PaTH Forward in the fourth quarter of 2019, (iii) our ability to apply our TransCon technology platform to build a leading, fully integrated biopharma company, (iv) our expectations regarding our ability to create potentially best-in-class therapies and (v) 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: 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 Annual Report on Form 20-F for the year ended December 31, 2017, which we filed with the SEC on March 28, 2018. 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

Consolidated Statements of Profit or Loss and Other Comprehensive Income / (loss)
(In EUR'000s, except share and per share data)

| | Year ended December 31, | |
|--|-------------------------|------------------|
| | 2018 | 2017 |
| Revenue | 10,581 | 1,530 |
| Research and development costs | (140,281) | (99,589) |
| General and administrative expenses | (25,057) | (13,482) |
| Operating profit / (loss) | (154,757) | (111,541) |
| Share of profit / (loss) of associate | (321) | - |
| Finance income | 24,714 | 923 |
| Finance expenses | (127) | (13,756) |
| Profit / (loss) before tax | (130,491) | (124,374) |
| Tax on profit / (loss) for the year | 394 | 477 |
| Net profit / (loss) for the year | (130,097) | (123,897) |
| Other comprehensive income / (loss) | | |
| <i>Items that may be reclassified subsequently to profit or loss:</i> | | |
| Exchange differences on translating foreign operations | 17 | 65 |
| Other comprehensive income / (loss) for the year, net of tax | 17 | 65 |
| Total comprehensive income / (loss) for the year, net of tax | (130,080) | (123,832) |
| Profit / (loss) for the year attributable to owners of the Company | (130,097) | (123,897) |
| Total comprehensive income / (loss) for the year attributable to owners of the Company | (130,080) | (123,832) |
| | EUR | EUR |
| Basic and diluted earnings / (loss) per share | (3.17) | (3.68) |
| Number of shares used for calculation (basic and diluted) | 41,085,237 | 33,626,305 |

Ascendis Pharma A/S
Consolidated Statements of Financial Position
(In EUR'000s)

| | December 31, 2018 | December 31, 2017 |
|-------------------------------|----------------------|----------------------|
| Assets | | |
| Non-current assets | | |
| Intangible assets | 3,495 | 3,495 |
| Property, plant and equipment | 4,325 | 2,557 |
| Investment in associate | 17,083 | - |
| Deposits | 1,158 | 293 |
| | 26,061 | 6,345 |
| Current assets | | |
| Trade receivables | 6 | 188 |
| Other receivables | 1,775 | 1,410 |
| Prepayments | 12,415 | 6,907 |
| Income taxes receivable | 849 | 778 |
| Cash and cash equivalents | 277,862 | 195,351 |
| | 292,907 | 204,634 |
| Total assets | 318,968 | 210,979 |

Equity and liabilities**Equity**

| | | |
|----------------------|----------------|----------------|
| Share capital | 5,659 | 4,967 |
| Distributable equity | 274,391 | 182,244 |
| Total equity | 280,050 | 187,211 |

Current liabilities

| | | |
|--------------------------|---------------|---------------|
| Contract liabilities | 6,902 | - |
| Trade payables | 19,740 | 17,434 |
| Other payables | 12,267 | 6,334 |
| Income taxes payable | 9 | - |
| | 38,918 | 23,768 |
| Total liabilities | 38,918 | 23,768 |

Total equity and liabilities

| | | |
|--|----------------|----------------|
| | 318,968 | 210,979 |
|--|----------------|----------------|

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Source: Ascendis Pharma A/S