Ascendis Pharma A/S Reports Third Quarter 2019 Financial Results

November 18, 2019 at 4:01 PM EST

– Continued execution of global endocrinology rare disease programs as planned regulatory filings for TransCon™ hGH in 2020 advance on track –

– Expanded PaTH Forward Trial expedites enrollment of subjects previously treated with parathyroid hormone (PTH) –

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

COPENHAGEN, Denmark, Nov. 18, 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 quarter ended September 30, 2019.

"Our achievements this year reflect our ability to bring a product from idea stage all the way through clinic, as we near the finish line for TransCon hGH and approach our planned regulatory filings in the United States (U.S.) and Europe," said Jan Mikkelsen, Ascendis Pharma's President and Chief Executive Officer. "Our recent progress in the TransCon hGH program, including both in Europe and China, also support our strategy to establish global reach. The power of the TransCon technology is enabling us to build a leading fully integrated patient-focused biopharma company, and advance towards our 'Vision 3x3' goals."

He continued, "One of our driving values at Ascendis is patient focus. In response to the recent recall of NATPARA ® in the U.S., we recently expanded our TransCon PTH Phase 2 PaTH Forward Trial with a protocol addendum to help expedite participation of some of those patients affected by the recall. We believe the expanded trial will provide meaningful clinical data to help patients, both naïve to PTH and previously treated with PTH, and further demonstrate the value of our unique approach to product development."

Corporate Highlights & Progress

- Completed the last subject visit for the long-term clinical database for TransCon hGH, paving the way for the company's planned Biologics License Application to the U.S. Food and Drug Administration in the first half of 2020 and the Marketing Authorisation Application to the European Medicines Agency in the second half of 2020. TransCon hGH is a long-acting prodrug of human growth hormone (hGH) in phase 3 development as a once-weekly therapy for pediatric growth hormone deficiency (GHD).
- Completed the manufacturing of drug product Process Performance Qualification (PPQ) batches required to support the planned regulatory filings in 2020 for TransCon hGH. The company is now finalizing the associated analytics and preparing qualification reports.
- Advanced the TransCon hGH program in Greater China with VISEN Pharmaceuticals, who initiated a phase 3 trial for TransCon hGH in pediatric GHD.
- Received Orphan Designation from the European Commission for TransCon hGH, which is provided to therapies aimed at treating, preventing or diagnosing a disease that is life-threatening or chronically debilitating, affects no more than five in 10,000 persons in the European Union and which may provide significant additional benefit over existing therapies.
- Announced a protocol addendum in the U.S. designed to expedite enrollment of subjects previously treated with NATPARA in the TransCon PTH PaTH Forward Trial, a global phase 2 trial evaluating the safety, tolerability and efficacy of TransCon PTH in adult subjects with hypoparathyroidism (HP). TransCon PTH is an investigational long-acting prodrug of PTH in development as a potential once-daily replacement therapy for HP, designed to provide physiologic levels of PTH for 24 hours a day, seven days a week. Under the addendum, patients previously treated with NATPARA in the U.S. will now have an expedited pathway to enroll in PaTH Forward. As a result, the company expects to exceed targeted enrollment of 40 subjects, and plans to report top-line data from the expanded trial in first quarter of 2020. The goal of PaTH Forward is to evaluate TransCon PTH control of serum and urinary calcium, and identify a titration regimen for complete withdrawal of standard of care (i.e., active vitamin D and calcium supplements).
- Initiated the ACcomplisH Trial, a global, phase 2, randomized, double-blind, placebo-controlled trial designed to evaluate the safety and efficacy of TransCon CNP, a long-acting prodrug of C-type natriuretic peptide (CNP), at escalating doses in children with achondroplasia (ACH). TransCon CNP is designed to provide continuous exposure to CNP at therapeutic levels with once-weekly dosing.
- Presented preclinical data for TransCon TLR7/8 Agonist, a product candidate in development for oncology, at the Society of Immunotherapy of Cancer annual meeting. TransCon TLR7/8 Agonist is a prodrug of resiquimod, a small molecule with immune-activating and anti-tumor properties that is transiently conjugated to a hydrogel carrier via a TransCon linker. Administered as an intratumoral injection, TransCon TLR7/8 Agonist delivered sustained local release of resiquimod over weeks directly to the tumor site and demonstrated potent anti-tumor activity as a monotherapy, as well as in combination

with interleukin-2 (IL-2).
- Ended the third quarter of 2019 with cash and cash equivalents of €658.7 million.

Third Quarter 2019 Financial Results

For the third quarter, Ascendis Pharma reported a net loss of €25.1 million, or €0.53 per share (basic and diluted) compared to a net loss of €34.0 million, or €0.81 per share (basic and diluted) for the same period in 2018.

Revenue for the third quarter was €2.2 million compared to €20 thousand in the same quarter of 2018. The increase relates to our November 2018 strategic investment in VISEN Pharmaceuticals.

Research and development (R&D) costs for the third quarter were €46.3 million compared to €31.5 million during the same period in 2018. Higher R&D costs in 2019 reflect an increase in personnel-related costs to support development and manufacturing of TransCon hGH, TransCon PTH and TransCon CNP, increasing clinical trial costs for the PaTH Forward Trial and the ACcomplish Trial, as well as increased costs for other research programs, including oncology.

General and administrative expenses for the third quarter were €10.0 million compared to €6.8 million during the same period in 2018. The increase is primarily due to higher personnel-related costs and other increasing costs of expanding the company and preparing to become a commercial organization.

As of September 30, 2019, Ascendis Pharma had cash and cash equivalents of €658.7 million compared to €690.4 million as of June 30, 2019. As of September 30, 2019, Ascendis Pharma had 47,739,647 ordinary shares outstanding.

Conference Call and Webcast information

Ascendis Pharma will host a conference call and webcast today at 4:30 p.m. ET to discuss its third quarter 2019 financial results. Details include:

Date	Monday, November 18, 2019
Time	4:30 p.m. ET
Dial In (U.S.)	844-290-3904
Dial In (International)	574-990-1036
Access Code	5897398

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 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 in the first half of 2020 and a MAA with the EMA in the second half of 2020 for TransCon hGH, (ii) the plans to initiate a phase 3 trial for TransCon hGH in pediatric GHD in Greater China in collaboration with Visen Pharmaceuticals, (iv) our ability to apply our TransCon platform to build a leading, fully integrated biopharma company, (v) our expectations regarding our ability to create new and potentially best-in-class therapies and (vi) 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, 2018, which we filed with the SEC on April 3, 2019. 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
Unaudited Condensed Consolidated Interim Statements of Profit or Loss and Other Comprehensive Income / (loss)
(In EUR'000s, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	**2018**	**2019**	**2018**
Revenue	2,243	20	10,868	66
Research and development costs	(46,258)	(31,511)	(141,343)	(102,286)
General and administrative expenses	(10,000)	(6,796)	(31,396)	(16,684)
Operating profit / (loss)	**(54,015)**	**(38,287)**	**(161,871)**	**(118,904)**
Share of profit / (loss) of associate	(1,338)	-	(5,452)	-
Finance income	30,547	4,262	30,285	20,532
Finance expenses	(368)	(42)	(812)	(53)
Profit / (loss) before tax	**(25,174)**	**(34,067)**	**(137,850)**	**(98,425)**
Tax on profit / (loss) for the period	61	100	196	306
Net profit / (loss) for the period	**(25,113)**	**(33,967)**	**(137,654)**	**(98,119)**
Other comprehensive income / (loss)				
Items that may be reclassified subsequently to profit or loss:				
Exchange differences on translating foreign operations	37	(9)	2	(16)
Other comprehensive income / (loss) for the period, net of tax	**37**	**(9)**	**2**	**(16)**
Total comprehensive income / (loss) for the period, net of tax	**(25,076)**	**(33,976)**	**(137,652)**	**(98,135)**
Profit / (loss) for the period attributable to owners of the Company	(25,113)	(33,967)	(137,654)	(98,119)
Total comprehensive income / (loss) for the period attributable to owners of the Company	(25,076)	(33,976)	(137,652)	(98,135)
	EUR	**EUR**	**EUR**	**EUR**
Basic and diluted earnings / (loss) per share	(0.53)	(0.81)	(2.99)	(2.41)
Number of shares used for calculation (basic and diluted)	47,590,837	41,888,908	46,066,493	40,757,686

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

	September 30, 2019	**December 31, 2018**
Assets		
Non-current assets		
Intangible assets	3,495	3,495
Property, plant and equipment	43,272	4,325
Investment in associate	17,073	17,083
Deposits	1,469	1,158
	65,309	**26,061**
Current assets		
Trade receivables	-	6
Other receivables	1,755	1,775
Prepayments	7,937	12,415

Income taxes receivable	1,298	849
Cash and cash equivalents	658,660	277,862
	669,650	**292,907**
Total assets	**734,959**	**318,968**
Equity and liabilities		
Equity		
Share capital	6,410	5,659
Distributable equity	654,515	274,391
Total equity	**660,925**	**280,050**
Non-current liabilities		
Lease liabilities	31,503	-
	31,503	-
Current liabilities		
Lease liabilities	5,424	-
Contract liabilities	1,373	6,902
Trade payables	24,346	19,740
Other payables	11,364	12,267
Income taxes payable	24	9
	42,531	**38,918**
Total liabilities	**74,034**	**38,918**
Total equity and liabilities	**734,959**	**318,968**

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