Ascendis Pharma A/S Reports First Quarter 2019 Financial Results

May 30, 2019

– Continued execution of global endocrinology rare disease programs, following validation of TransCon ™ platform in phase 3 heiGHt Trial –

– R&D Day on June 26 to feature endocrinology and introduction of oncology –

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

COPENHAGEN, Denmark , May 30, 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 March 31, 2019 .

"In my view, 2019 has been the most transformative period in Ascendis history as we validated our TransCon platform with clinical evidence from the phase 3 heiGHt and fliGHt Trials for TransCon Growth Hormone, while continuing to execute on all three of our global endocrinology rare disease programs," said Jan Mikkelsen, Ascendis Pharma's President and Chief Executive Officer. "Our long-term commitment to science and addressing unmet medical needs where we can make a difference for patients has never been stronger. I am pleased with our progress in endocrinology rare diseases and look forward to providing detailed data, as well as introducing our oncology programs, at the upcoming R&D Day on June 26 th in New York City."

Corporate Highlights & Progress

- Reported preliminary results from the phase 3, open label, single arm, fliGHt Trial of TransCon Growth Hormone (hGH), a long-acting growth hormone therapy in development as a once-weekly treatment for pediatric growth hormone deficiency (GHD). Data from this 26-week trial showed TransCon hGH was safe and well-tolerated in pediatric subjects previously treated with commercially-available daily growth hormone therapy. These data also include new information demonstrating safety, efficacy and tolerability in treatment-naïve subjects under three years of age. Detailed results from the fliGHt Trial will be presented at the June 26 R&D Day. Data from the heiGHt and fliGHt Trials, and long-term safety data from the ongoing enliGHten (long-term extension) Trial, will form the safety database that supports submission of 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.
- Presented additional top-line analyses from the pivotal, phase 3 heiGHt Trial for TransCon hGH, which were consistent with the previously reported top-line results and demonstrated that TransCon hGH had comparable safety and tolerability to a daily hGH (Genotropin®), with a significantly greater annualized height velocity over the one-year study period. The additional data were presented at the Pediatric Endocrinology Nursing Society (PENS) and Pediatric Endocrine Society (PES) annual meetings.
- Continued initiating sites to expand reach in the global PaTH Forward phase 2 clinical trial of TransCon PTH, a long-acting parathyroid hormone therapy in development for the treatment of adult hypoparathyroidism, providing physiologic levels of PTH for 24 hours a day. In addition, presented three posters relating to TransCon PTH and hypoparathyroidism: one poster highlighted the short-term symptoms and burden of hypoparathyroidism at ENDO 2019; a second poster presented design of the PaTH Forward trial at the European Calcified Tissue Society (ECTS) meeting; and a third poster presented new data on the economic burden of symptoms of hypoparathyroidism at the International Society for Pharmacoeconomics and Outcomes Research (ISPOR) meeting.
- Completed analysis of data from the phase 1 trial of TransCon CNP, a long-acting prodrug of C-type natriuretic peptide (CNP) in development as a once-weekly therapeutic option for achondroplasia, in healthy subjects following preliminary data reported in November 2018 . The results showed that TransCon CNP provided continuous exposure to CNP with a pharmacokinetic profile designed to provide therapeutic levels of CNP with once-weekly dosing. TransCon CNP was generally well tolerated at doses up to 150 µg/kg, with no serious adverse events reported, and the resting blood pressure and heart rate were unchanged from predose at all time points, in all cohorts.
- Received Orphan Drug Designation (ODD) from the U.S. FDA for TransCon CNP in achondroplasia. The company is preparing to initiate a phase 2 trial of TransCon CNP in children with the condition in the third quarter of this year.
- Continued ongoing discussions with regulatory agencies to establish global reach for all three of the company's endocrinology rare disease clinical programs and advance the global clinical development of TransCon hGH, TransCon PTH and TransCon CNP.
- Ended the first quarter of 2019 with cash and cash equivalents of €696.7 million.

First Quarter 2019 Financial Results

For the first quarter, Ascendis Pharma reported a net loss of €53.6 million, or €1.24 per share (basic and diluted) compared to a net loss of €41.4 million, or €1.07 per share (basic and diluted) for the same period in 2018.

Revenue for the first quarter was €5.4 million compared to €28 thousand in the same quarter of 2018. The increase reflects recognition of revenue from the sale of licenses in connection with the formation of the strategic investment in VISEN Pharmaceuticals .

Research and development (R&D) costs for the first quarter were €51.3 million compared to €30.5 million during the same period in 2018. Higher R&D costs in 2019 reflect an increase in costs for the manufacturing of validation batches of TransCon hGH required as part of the regulatory approval process, partly offset by decreasing costs for the phase 3 clinical program; for TransCon PTH, an increase in costs associated with continued development and progress, including manufacturing of clinical material and pen device, and initiation of the phase 2 PaTH Forward clinical trial; for TransCon CNP, lower manufacturing and preclinical costs, partly offset by phase 2 enabling activities; and increased headcount in R&D functions.

General and administrative expenses for the first quarter were €10.4 million compared to €4.7 million during the same period in 2018. The increase is primarily due to higher personnel costs and other increasing costs of preparing to become a commercial organization.

As of March 31, 2019 , the company had cash and cash equivalents of €696.7 million compared to €277.9 million as of December 31, 2018 . This includes net proceeds to the company of $539.4 million , or €480.3 million, after deducting the underwriters' commissions and the company's estimated offering expenses, from an underwritten public offering of 4,791,667 American Depositary Shares ("ADSs"), completed in March 2019 . As of March 31, 2019 , Ascendis Pharma had 46,927,115 ordinary shares outstanding.

Realizing Vision 3x3: R&D Day on June 26, 2019

Ascendis Pharma is hosting an R&D Day for investors in New York City on June 26, 2019 from 9:00 a.m. to 1:00 p.m. Eastern Time (ET). The company will provide an update on its endocrinology rare disease and oncology research and development activities.

Conference Call and Webcast information

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

Date	Thursday, May 30, 2019
Time	4:30 p.m. ET
Dial In (U.S.)	844-290-3904
Dial In (International)	574-990-1036
Access Code	9879947

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 for TransCon hGH to treat pediatric GHD in the first half of 2020, (ii) our plans to initiate a phase 2 trial of TransCon CNP in children with achondroplasia in the third quarter of 2019, (iii) our ability to apply our TransCon 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, 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.

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 March 31,	
	2019	**2018**
Revenue	5,414	28
Research and development costs	(51,259)	(30,540)
General and administrative expenses	(10,436)	(4,662)
Operating profit / (loss)	**(56,281)**	**(35,174)**
Share of profit / (loss) of associate	(1,852)	-
Finance income	4,620	702
Finance expenses	(194)	(7,010)
Profit / (loss) before tax	**(53,707)**	**(41,482)**
Tax on profit / (loss) for the period	70	107
Net profit / (loss) for the period	**(53,637)**	**(41,375)**
Other comprehensive income / (loss)		
Items that may be reclassified subsequently to profit or loss:		
Exchange differences on translating foreign operations	559	(9)
Other comprehensive income / (loss) for the period, net of tax	**559**	**(9)**
Total comprehensive income / (loss) for the period, net of tax	**(53,078)**	**(41,384)**
Profit / (loss) for the period attributable to owners of the Company	(53,637)	(41,375)
Total comprehensive income / (loss) for the period attributable to owners of the Company	(53,078)	(41,384)
	EUR	**EUR**
Basic and diluted earnings / (loss) per share	(1.24)	(1.07)
Number of shares used for calculation (basic and diluted)	43,371,559	38,699,204

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

	March 31, 2019	**December 31, 2018**
Assets		
Non-current assets		
Intangible assets	3,495	3,495
Property, plant and equipment	24,032	4,325
Investment in associate	17,476	17,083
Deposits	1,161	1,158
	46,164	**26,061**
Current assets		
Trade receivables	4	6
Other receivables	6,863	1,775
Prepayments	11,282	12,415

Income taxes receivable	962	849
Cash and cash equivalents	696,664	277,862
	715,775	**292,907**
Total assets	**761,939**	**318,968**
Equity and liabilities		
Equity		
Share capital	6,301	5,659
Distributable equity	710,360	274,391
Total equity	**716,661**	**280,050**
Non-current liabilities		
Lease liabilities	13,213	-
	13,213	-
Current liabilities		
Lease liabilities	4,271	-
Contract liabilities	3,073	6,902
Trade payables	19,237	19,740
Other payables	5,445	12,267
Income taxes payable	39	9
	32,065	**38,918**
Total liabilities	**45,278**	**38,918**
Total equity and liabilities	**761,939**	**318,968**

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