

Ascendis Pharma A/S Reports Full Year 2017 Financial Results

March 28, 2018

- Rare Disease Endocrinology Pipeline Advances, with Significant Milestones Anticipated Over the Next Twelve Months -

- Conference Call Today at 4:30 p.m. Eastern Time -

COPENHAGEN, Denmark, March 28, 2018 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq:ASND), a biopharmaceutical company that utilizes its innovative TransCon technology to address significant unmet medical needs in rare diseases, today announced financial results for the full year ended December 31, 2017.

"We are excited by the progress of our rare disease endocrinology pipeline and the potential to bring patients differentiated treatments for unmet medical needs. We anticipate data across all three of our clinical programs in the next twelve months, including full data from our ongoing phase 1 trial of TransCon PTH, top-line data from our planned TransCon CNP phase 1 trial, and top-line data from our phase 3 heiGHt Trial for TransCon Growth Hormone," said Jan Mikkelsen, Ascendis Pharma's President and Chief Executive Officer. "The value of our TransCon technology platform is increasing as we move into multiple clinical programs, and we continue to see the broad applicability of our technology."

Recent Corporate Highlights

• Presented two posters at ENDO 2018, the annual conference of the Endocrine Society, both of which were selected for inclusion in special moderated sessions:

A poster describing the baseline demographic characteristics of the phase 3 heiGHt Trial for TransCon Growth Hormone (GH), in development as a potential once-weekly therapy for pediatric growth hormone deficiency (GHD)

^o A poster on the phase 1 trial for TransCon PTH, in development as a treatment for hypoparathyroidism (HP). The trial is assessing single and multiple ascending doses in healthy adults. The poster summarized pharmacokinetic and pharmocodynamic findings to date, which reinforced the potential for TransCon PTH administered daily as a physiologic PTH replacement therapy

- Completed enrollment of 161 subjects in the global, phase 3 heiGHt Trial of TransCon GH for pediatric GHD
- Initiated enrollment in the fliGHt Trial evaluating TransCon GH in approximately 150 subjects who have previously been treated with daily growth hormone
- Initiated the enliGHten Trial, the long term extension trial of TransCon GH, which is enrolling subjects from both the heiGHt Trial and fliGHt Trial
- Announced initiation of the regulatory process in Australia to enable its first-in-human phase 1 trial for TransCon CNP, in development for achondroplasia
- Subsequent to year-end 2017, the company announced the completion of its underwritten public offering of 4,539,473 American Depositary Shares ("ADSs"), each of which represented one ordinary share of Ascendis Pharma at a price to the public of \$57.00 per ADS. Net proceeds from this offering in February 2018 were approximately \$242.5 million, or approximately €196.8 million based on exchange rates on the date of the offering
- Ended 2017 with cash and cash equivalents of €195.4 million on a reported basis

Full Year 2017 Financial Results

For the full year 2017, Ascendis Pharma reported a net loss of \leq 123.9 million, or \leq 3.68 per share (basic and diluted) compared to a net loss of \leq 68.5 million, or \leq 2.58 per share (basic and diluted) during the same period in 2016.

Research and development (R&D) costs for 2017 were €99.6 million compared to €66.0 million during 2016. Higher R&D costs in 2017 reflect an increase in manufacturing costs and clinical costs related to preparation for and execution of the company's phase 3 clinical program for TransCon GH, including the heiGHt, fliGHt and enliGHten Trials, ongoing development of our proprietary auto-injector for use with TransCon GH, preparation for and execution of the company's phase 1 clinical trial of TransCon PTH, as well as preclinical development of TransCon PTH and TransCon CNP.

General and administrative expenses for the 2017 year were €13.5 million compared to €11.5 million during the 2016 year. The increase is primarily due to an increase in general and administrative personnel.

As of December 31, 2017, the company had cash and cash equivalents of \in 195.4 million compared to \in 180.3 million as ofDecember 31, 2016. The company's cash position was positively impacted by net proceeds of \in 123.1 million from an underwritten public offering in September 2017 and \in 1.6 million from warrant exercises. As of December 31, 2017, Ascendis Pharma had 36,984,292 ordinary shares outstanding. As of March 28, 2018, including the 2018 offering, Ascendis Pharma had 41,523,765 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 2017 financial results. Details include:

Date Time Dial In (U.S.) Dial In (International) Access Code Wednesday, March 28, 2018 4:30 p.m. ET 844-290-3904 574-990-1036 6799954

A live audio webcast of the event will be available in the Investors and News section of the Ascendis Pharma website at <u>www.ascendispharma.com</u>. 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 prodrug technology to build a leading, fully integrated rare disease company focused on making a meaningful difference in patients' lives. The company utilizes its TransCon technology with clinically validated parent drugs to create new therapies with potential for best-in-class efficacy, safety and/or convenience.

Ascendis Pharma has a wholly-owned pipeline of three rare disease endocrinology programs, including once-weekly TransCon Growth Hormone, which is currently being evaluated in a phase 3 program for children with growth hormone deficiency (GHD), TransCon PTH, a long-acting prodrug of parathyroid hormone for hypoparathyroidism currently in a phase 1 trial, and TransCon CNP, a long-acting prodrug of C-type natriuretic peptide for achondroplasia. Additionally, Ascendis Pharma has multi-product collaborations with Sanofi in diabetes and Genentech in the field of ophthalmology.

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 ability to apply our prodrug technology to build a leading, fully integrated rare disease company, (ii) our expectations regarding when we will announce full data from our ongoing phase 1 trial of TransCon PTH (iii)

our expectations regarding when we will announce top-line data from our planned TransCon CNP phase 1 trial, (iv) our expectations regarding when we will announce top-line data from our phase 3 heiGHt Trial for TransCon Growth Hormone, (v) our expectations regarding our ability to create therapies with potential for best-in-class efficacy, safety and/or convenience 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 Growth Hormone, TransCon PTH and TransCon CNP or other development programs; unforeseen expenses related to the development of TransCon Growth Hormone, 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 Growth Hormone, 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.

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)

	2017		2016	
Revenue	1,530		4,606	
Research and development costs	(99,589)	(66,022)
General and administrative expenses	(13,482)	(11,504)
Operating profit / (loss)	(111,541)	(72,920)
Finance income	923		7,300	
Finance expenses	(13,756)	(3,112)
Profit / (loss) before tax	(124,374)	(68,732)
Tax on profit / (loss) for the year	477		227	
Net profit / (loss) for the year	(123,897)	(68,505)
Other comprehensive income / (loss)				
Items that may be reclassified subsequently to profit or loss:				
Exchange differences on translating foreign operations	65		6	
Other comprehensive income / (loss) for the year, net of tax	65		6	
Total comprehensive income / (loss) for the year, net of tax	(123,832)	(68,499)

Year ended December 31,

Profit / (loss) for the year attributable to owners of the Company Total comprehensive income / (loss) for the year attributable to owners of the Company	(123,897 (123,832) (68,505) (68,499	,
Basic earnings / (loss) per share Diluted earnings / (loss) per share	EUR (3.68 (3.68	EUR) (2.58) (2.58))
Number of shares used for calculation (basic and diluted)	33,626,305	26,564,	414

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

	December 31,	December 31,
	2017	2016
Assets		
Non-current assets		
Intangible assets	3,495	3,495
Property, plant and equipment	2,557	2,350
Deposits	293	268
	6,345	6,113
Current assets		
Trade receivables	188	287
Other receivables	1,410	640
Prepayments	6,907	1,962
Income taxes receivable	778	740
Cash and cash equivalents	195,351	180,329
	204,634	183,958
Total assets	210,979	190,071
Equity and liabilities		
Equity		
Share capital	4,967	4,354
Distributable equity	182,244	172,259
Total equity	187,211	176,613
Current liabilities		
Trade payables and other payables	23,768	13,078
Deferred income	-	94
Income taxes payable	-	286
	23,768	13,458
Total liabilities	23,768	13,458
Total equity and liabilities	210,979	190,071

Internal contact:

Scott T. Smith Chief Financial Officer (650) 352-8389 ir@ascendispharma.com

Investor contact:

Patti Bank Westwicke Partners (415) 513-1284 patti.bank@westwicke.com

Media contact:

Ami Knoefler SparkBioComm (650) 739-9952 ami@sparkbiocomm.com



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