Ascendis Pharma A/S Reports First Quarter 2017 Financial Results

May 23, 2017

- Phase 3 Program Advances for TransCon Growth Hormone and Two More Rare Disease Product Candidates Approach Clinic -

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

COPENHAGEN, Denmark, May 23, 2017 /PRNewswire/ -- 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 quarter ended March 31, 2017.

"We are on track with all three of our development programs, according to our Vision 20/20 strategic plan to establish Ascendis as a leading, integrated rare disease company," said Jan Mikkelsen, Ascendis Pharma's President and Chief Executive Officer. "In addition to advancing our Phase 3 program for TransCon Growth Hormone, we delivered positive data at the recent ENDO conference supporting all three product candidates in our diversified potential 'best-in-class' product pipeline - TransCon Growth Hormone, TransCon Parathyroid Hormone (PTH) and TransCon C-Type Natriuretic Peptide (CNP)."

Recent Corporate Highlights

- Continued to enroll subjects in the Phase 3 heiGHt Trial for TransCon Growth Hormone in pediatric growth hormone deficiency; the company remains on track to complete enrollment in the fourth quarter of 2017
- Presented data on all three rare disease endocrinology pipeline programs at ENDO 2017, including four posters supporting the differentiated product profiles for TransCon PTH in hypoparathyroidism and TransCon CNP in achondroplasia, as well as four posters summarizing the phase 3 clinical program and auto-injector development for TransCon Growth Hormone
- Announced data with TransCon CNP demonstrating potential reversal of the achondroplasia phenotype in a mouse disease model, and repeat dosing studies demonstrating the potential of TransCon PTH to be delivered with an infusion-like profile which may lead to a highly differentiated treatment for hypoparathyroidism
- Announced receipt of a letter from the U.S. Food and Drug Administration indicating the Agency's concurrence with the company's proposed Pediatric Study Plan for the TransCon Growth Hormone development program
- Ended the quarter with cash and cash equivalents of €157.6 million

First Quarter 2017 Financial Results

For the first quarter, Ascendis Pharma reported a net loss of €25.1 million, or €0.78 per share (basic and diluted) compared to a net loss of €20.5 million, or €0.82 per share (basic and diluted) during the same period in 2016.

Research and development (R&D) costs for the quarter were €20.6 million compared to €16.2 million in the same quarter during 2016. Higher R&D costs in the 2017 quarter reflect an increase in development costs for TransCon PTH and TransCon CNP due to the continued development and progress with these two product candidates. This increase was partly offset by lower manufacturing costs for TransCon Growth Hormone in the first quarter of 2017 compared to the 2016 quarter.

General and administrative expenses for the first quarter of 2017 were €3.3 million compared to €2.9 million during the same quarter of 2016. The increase is primarily due to an increase in personnel costs, partly offset by a decrease in professional fees.

As of March 31, 2017, the company had cash and cash equivalents of €157.6 million compared to €180.3 million as of December 31, 2016. As of March 31, 2017, Ascendis had 32,502,555 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 first quarter financial results. The live conference call can be accessed at (844) 290-3904 (United States) and (574) 990-1036 (International). The access code for all callers is 20021109. The webcast can be accessed from the Investors & News section of the Ascendis Pharma website at www.ascendispharma.com, and will be available for replay for at least 30 days.

About Ascendis Pharma A/S

Ascendis Pharma is applying the TransCon technology platform to build a leading rare disease commercial company. The company utilizes its innovative TransCon technology to address significant unmet medical needs in rare diseases by improving clinically validated parent drugs and creating therapies with potential for best-in-class efficacy, safety and/or convenience.

Ascendis Pharma has a wholly-owned pipeline of rare disease endocrinology programs, including once-weekly TransCon Growth Hormone, which is currently being evaluated in the Phase 3 heiGHt Trial for children with growth hormone deficiency, TransCon PTH, a long-acting prodrug of parathyroid hormone for hypoparathyroidism, 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 potential to become a leading, integrated rare disease company; (ii) our product pipeline; (iii) our expectations about when we will complete enrollment in the Phase 3 heiGHt Trial for TransCon Growth Hormone; (iv) our expectations regarding the potential of TransCon CNP and TransCon PTH; (v) our ability to apply the TransCon technology platform to build a leading rare disease commercial company and (vi) our expectations regarding our ability to create therapies with potential for best-in-class efficacy, safety and/or convenience. 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, 2016, which we filed with the SEC on March 22, 2017. 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,	
	2017	**2016**
Revenue	372	1,258
Research and development costs	(20,608)	(16,242)
General and administrative expenses	(3,325)	(2,908)
Operating profit / (loss)	**(23,561)**	**(17,892)**
Finance income	130	20
Finance expenses	(1,722)	(2,764)
Profit / (loss) before tax	**(25,153)**	**(20,636)**
Tax on profit / (loss) for the period	14	118
Net profit / (loss) for the period	**(25,139)**	**(20,518)**
Other comprehensive income / (loss)		
Items that may be reclassified subsequently to profit or loss:		
Exchange differences on translating foreign operations	4	21
Other comprehensive income / (loss) for the period, net of tax	**4**	**21**
Total comprehensive income / (loss) for the period, net of tax	**(25,135)**	**(20,497)**
Profit / (loss) for the period attributable to owners of the Company	(25,139)	(20,518)
Total comprehensive income / (loss) for the period attributable to owners of the Company	(25,135)	(20,497)
	EUR	**EUR**
Basic earnings / (loss) per share	(0.78)	(0.82)
Diluted earnings / (loss) per share	(0.78)	(0.82)
Number of shares used for calculation (basic and diluted)	32,428,908	25,128,242

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

	March 31, 2017	December 31, 2016
Assets		
Non-current assets		
Intangible assets	3,495	3,495
Property, plant and equipment	2,559	2,350
Deposits	267	268
	6,321	**6,113**
Current assets		
Trade receivables	378	287
Other receivables	1,921	640
Prepayments	5,624	1,962
Income taxes receivable	872	740
Cash and cash equivalents	157,648	180,329
	166,443	**183,958**
Total assets	**172,764**	**190,071**
Equity and liabilities		
Equity		
Share capital	4,365	4,354
Other reserves	15,714	13,005
Retained earnings	134,748	159,254
Total equity	**154,827**	**176,613**
Current liabilities		
Trade payables and other payables	17,494	13,078
Deferred income	94	94
Income taxes payable	349	286
	17,937	**13,458**
Total liabilities	**17,937**	**13,458**
Total equity and liabilities	**172,764**	**190,071**

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