Ascendis Pharma A/S Reports Third Quarter 2018 Financial Results

November 28, 2018

- TransCon CNP preliminary phase 1 data support target product profile and further validates TransCon ™ technology -

- Diversified pipeline with three independent, clinically-validated rare disease endocrinology product candidates now in place -

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

COPENHAGEN, Denmark, Nov. 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, today announced financial results for the quarter ended September 30, 2018 and released preliminary phase 1 data for TransCon CNP.

"To support our vision for sustainable growth, we are creating a diversified pipeline of three independent product candidates in rare endocrine diseases. We have made a major step towards this goal with three clinically-validated, high value product candidates in one therapeutic area," said Jan Mikkelsen, Ascendis Pharma's President and Chief Executive Officer. "With today's positive TransCon CNP data, we are intrigued by the opportunity to establish a leadership position in treatment of numerous growth disorders by pursuing additional indications and potential treatment synergies within our endocrinology pipeline."

He continued, "we remain focused on delivering top-line results for our pivotal phase 3 trial of TransCon hGH, while we continue to make progress towards initiation of the phase 2 trial for TransCon PTH, both key milestones anticipated in the first quarter of 2019. Additionally, we announced the formation of VISEN Pharmaceuticals to develop and commercialize our rare disease endocrinology programs in Greater China. This progress advances our vision to build a global, fully integrated biopharma company that will deliver sustainable growth."

Recent Corporate Highlights & Progress

- Released preliminary data from the phase 1, double-blind, randomized, placebo-controlled trial of TransCon CNP, a long-acting prodrug of CNP in development as a therapeutic option for achondroplasia. Results of the trial in 45 healthy adult subjects showed that TransCon CNP was generally well tolerated and provided continuous exposure to CNP at target levels over seven days with a single subcutaneous administration. Details are available in a separate press release issued today and a related slide presentation may be viewed on the Ascendis Pharma website at: https://ascendispharma.gcs-web.com/events-and-presentations/upcoming-events.
- On track to report top-line results in the first quarter of 2019 for the phase 3 heiGHt Trial, a randomized, open-label, active-control trial comparing once-weekly TransCon hGH with a daily growth hormone therapy in treatment-naïve pediatric subjects with growth hormone deficiency (GHD). Completed third independent safety committee review; to date, the observed aggregate data from the heiGHt Trial continue to indicate a safety profile for TransCon hGH that is consistent with the published safety profile of the active comparator.
- Completed enrollment in the fliGHt Trial, an open-label trial evaluating TransCon hGH in pediatric subjects with GHD who switched from daily growth hormone therapy. As a result, more than 300 subjects have now enrolled in the phase 3 TransCon hGH program, which includes the heiGHt, fliGHt and enliGHten Trials.
- Continued planning for initiation of a phase 2 trial in the first quarter of 2019 to evaluate TransCon PTH, a long-acting prodrug of parathyroid hormone (PTH), in subjects with hypoparathyroidism. Final results of a phase 1 trial reinforced the potential of TransCon PTH to replace and restore PTH to physiologic levels for 24 hours per day.
- Formed VISEN Pharmaceuticals with an investor syndicate led by Vivo Capital with participation from Sofinnova Ventures, to develop, manufacture and commercialize TransCon endocrinology rare disease therapies in Greater China, which includes mainland China, Hong Kong, Macau and Taiwan. This 50/50 partnership expands the global reach of TransCon technology and establishes a presence in the region with collaborators who have significant experience and knowledge of the biopharmaceutical opportunity in China.
- Elected Lars Holtug, M.Sc., former partner and chairman of PricewaterhouseCoopers Statsautoriseret Revisionspartnerselskab, to the Ascendis Board of Directors.
- Ended the quarter with cash and cash equivalents of €310.3 million.

Third Quarter 2018 Financial Results

For the third quarter, Ascendis Pharma reported a net loss of €34.0 million, or €0.81 per share (basic and diluted) compared to a net loss of €33.9 million, or €1.04 per share (basic and diluted) for the same period in 2017.

Research and development (R&D) costs for the third quarter were €31.5 million compared to €29.1 million in the same quarter of 2017. Increased R&D costs in the 2018 quarter primarily reflect costs to prepare for the manufacture of validation batches for TransCon Growth Hormone; costs of the ongoing clinical trials for this product candidate; and the ongoing clinical and project costs for TransCon PTH and TransCon CNP.

General and administrative expenses for the third quarter of 2018 were €6.8 million compared to €2.8 million in the same quarter of 2017. The increase is primarily due to an increase in personnel costs and general costs arising from additional administrative personnel and initial costs of preparing for a commercial organization.

As of September 30, 2018, the company had cash and cash equivalents of €310.3 million compared to €352.6 million as of June 30, 2018. As of September 30, 2018, Ascendis had 42,032,522 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 third quarter 2018 financial results. Details include:

Date	Wednesday, November 28, 2018
Time	4:30 p.m. ET
Dial In (U.S.)	844-290-3904
Dial In (International)	574-990-1036
Access Code	4371358

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 ™ technology 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. 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 release top-line data for our phase 3 heiGHt trial in the first quarter of 2019, (ii) our plans to initiate our phase 2 TransCon PTH trial in patients with hypoparathyroidism in early 2019, (iii) our ability to apply our platform technology 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 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.

FINANCIAL TABLES TO 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,	
	2018	**2017**	**2018**	**2017**

Revenue	20	434	66	1,250
Research and development costs	(31,511)	(29,067)	(102,286)	(71,555)
General and administrative expenses	(6,796)	(2,840)	(16,684)	(9,396)
Operating profit / (loss)	**(38,287)**	**(31,473)**	**(118,904)**	**(79,701)**
Finance income	4,262	165	20,532	453
Finance expenses	(42)	(2,809)	(53)	(10,765)
Profit / (loss) before tax	**(34,067)**	**(34,117)**	**(98,425)**	**(90,013)**
Tax on profit / (loss) for the period	100	240	306	291
Net profit / (loss) for the period	**(33,967)**	**(33,877)**	**(98,119)**	**(89,722)**
Other comprehensive income / (loss)				
Items that may be reclassified subsequently to profit or loss:				
Exchange differences on translating foreign operations	(9)	(5)	(16)	41
Other comprehensive income / (loss) for the period, net of tax	**(9)**	**(5)**	**(16)**	**41**
Total comprehensive income / (loss) for the period, net of tax	**(33,976)**	**(33,882)**	**(98,135)**	**(89,681)**
Profit / (loss) for the period attributable to owners of the Company	(33,967)	(33,877)	(98,119)	(89,722)
Total comprehensive income / (loss) for the period attributable to owners of the Company	(33,976)	(33,882)	(98,135)	(89,681)
	EUR	**EUR**	**EUR**	**EUR**
Basic and diluted earnings / (loss) per share	(0.81)	(1.04)	(2.41)	(2.76)
Number of shares used for calculation (basic and diluted)	41,888,908	32,607,497	40,757,686	32,513,641

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

	September 30, 2018	**December 31, 2017**
Assets		
Non-current assets		
Intangible assets	3,495	3,495
Property, plant and equipment	3,513	2,557
Deposits	1,241	293
	8,249	**6,345**
Current assets		
Trade receivables	21	188
Other receivables	2,750	1,410
Prepayments	12,390	6,907
Income taxes receivable	1,491	778
Cash and cash equivalents	310,333	195,351
	326,985	**204,634**
Total assets	**335,234**	**210,979**
Equity and liabilities		
Equity		
Share capital	5,645	4,967
Distributable equity	298,560	182,244
Total equity	**304,205**	**187,211**
Current liabilities		

Trade payables and other payables	31,021	23,768
Income taxes payable	8	-
Total liabilities	**31,029**	**23,768**
Total equity and liabilities	**335,234**	**210,979**

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