Ascendis Pharma A/S Reports First Quarter 2020 Financial Results

May 19, 2020

– On track for filing U.S. Biologics License Application for TransCon hGH in second quarter, and advancing Marketing Authorisation Application in Europe to third quarter –

– Expanded commercial organization to support planned global launch of TransCon hGH –

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

COPENHAGEN, Denmark, May 19, 2020 (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, 2020.

"We continue to execute on our vision to build a leading biopharma company. In addition to remaining on track to achieve our corporate milestones for 2020, we continue to expand our organization to support the planned global launch of our first commercial product, TransCon Growth Hormone," said Jan Mikkelsen, Ascendis Pharma's President and Chief Executive Officer. "We are preparing to submit our marketing application in the United States in the second quarter and have advanced the European submission now to be in the third quarter."

He continued, "Additionally, we recently reported positive top-line data from the fixed dose, blinded portion of PaTH Forward evaluating TransCon PTH. This portion of the phase 2 trial met its key objectives, marking a major potential advance in helping hypoparathyroidism patients who are in urgent need of an effective PTH replacement therapy. And, with the appointment of Jesper Høiland as Global Chief Commercial Officer, we have added significant commercial expertise related to our growth hormone and PTH product candidates."

Corporate Highlights & Progress

- For TransCon hGH, an investigational long-acting prodrug of somatropin (human growth hormone or hGH), remain on track for planned submission of a Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) in the second quarter, and a Marketing Authorisation Application (MAA) to the European Medicines Agency now in the third quarter, both for pediatric growth hormone deficiency (GHD).
- Submitted an Investigational New Drug (IND) amendment to initiate the global, phase 3 foresiGHt Trial evaluating TransCon hGH in adults with GHD. Enrollment is expected to begin later this year.
- Remain on track to initiate a phase 3 trial with TransCon hGH in pediatric GHD in Japan in the fourth quarter.
- Received Orphan Drug Designation from the FDA for TransCon hGH as a treatment for GHD.
- Announced positive top-line results from the four-week fixed dose, blinded portion of PaTH Forward, a global phase 2 trial evaluating the safety, tolerability and efficacy of TransCon PTH, an investigational long-acting prodrug of parathyroid hormone (PTH), in adult subjects with hypoparathyroidism. Data showed that within only four weeks TransCon PTH replaced the current standard of care for 82 percent of patients with hypoparathyroidism.
- Remain on track to report six-month data from the open-label extension portion of the PaTH Forward Trial during the third quarter, and to initiate a global phase 3 trial of TransCon PTH in North America, Europe and Asia in the fourth quarter.
- Remain on track to submit the first IND or equivalent in oncology in the fourth quarter for TransCon TLR7/8 Agonist.
- Announced the appointment of Jesper Høiland as Global Chief Commercial Officer. Mr. Høiland has over 25 years of global senior leadership experience in biopharma, including over 20 years of global commercial experience in growth hormone.
- Opened the company's new facility in Redwood City, California to advance its pipeline of oncology programs.
- Ended the first quarter 2020 with cash and cash equivalents of €534.4 million.

First Quarter 2020 Financial Results

For the first quarter, Ascendis Pharma reported a net loss of €63.3 million, or €1.32 per share (basic and diluted) compared to a net loss of €53.6 million, or €1.24 per share (basic and diluted) for the same period in 2019.

Revenue for the first quarter was €2.2 million compared to €5.4 million in the same quarter of 2019. The decrease was due to a lower amount of license and service revenue being recognized, partly offset by sale of clinical supply to Visen.

Research and development (R&D) costs for the first quarter were €57.5 million compared to €51.3 million during the same period in 2019. Higher R&D costs in 2020 reflect an increase in personnel-related costs, overhead costs allocated to R&D, and the continued progress in development of the company's product candidates.

General and administrative expenses for the first quarter were €17.9 million compared to €10.4 million during the same period in 2019. The increase is primarily due to additional personnel-related costs, higher IT and facility costs, and continued build out of the company's commercial capabilities.

As of March 31, 2020, Ascendis Pharma had cash and cash equivalents of €534.4 million compared to €598.1 million as of December 31, 2019. As of

March 31, 2020, Ascendis Pharma had 47,985,837 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 2020 financial results. Details include:

Date	May 19, 2020
Time	4:30 p.m. ET
Dial In (U.S.)	844-290-3904
Dial In (International)	574-990-1036
Access Code	8295749

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's Pipeline

Ascendis Pharma currently has three product candidates in clinical development in rare endocrine diseases:

- TransCon hGH (lonapegsomatropin), an investigational long-acting prodrug of somatropin (human growth hormone or hGH) that releases somatropin with the identical amino acid sequence and size as daily growth hormone, in phase 3 development as a once-weekly treatment for growth hormone deficiency (GHD).
- TransCon PTH, an investigational long-acting prodrug of parathyroid hormone (PTH) in phase 2 development as a once-daily replacement therapy for hypoparathyroidism (HP) designed to replace PTH at physiologic levels for 24 hours every day, and address both short-term symptoms and long-term complications of the disease.
- TransCon CNP, an investigational long-acting prodrug of C-type natriuretic peptide (CNP) in phase 2 development as a therapy for children with achondroplasia (ACH), the most common form of dwarfism, for which there is no FDA-approved treatment. TransCon CNP is designed to provide continuous exposure of CNP at safe, therapeutic levels via a single, weekly subcutaneous dose.

Additionally, the company has established oncology as its second therapeutic area of focus and plans to submit an IND or equivalent in the fourth quarter of 2020 for its first oncology product candidate.

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 is advancing oncology as its second therapeutic area of focus. The company continues to expand into additional therapeutic areas to address unmet patient needs.

Ascendis is headquartered in Copenhagen, Denmark, with additional offices in Heidelberg and Berlin, Germany, and in Palo Alto and Redwood City, 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 progress to achieve our corporate milestones for 2020, (ii) our planned global launch of TransCon Growth Hormone, (iii) our plans to submit our marketing applications for TransCon hGH in the United States in the second quarter of 2020 and in Europe in the third quarter of 2020, (iv) our plans to begin enrollment for the phase 3 foresiGHt Trial evaluating TransCon hGH in adults with GHD later this year; (v) our plans to initiate a phase 3 trial with TransCon hGH in pediatric GHD in Japan in the fourth quarter of 2020, (vi) our plans to submit an IND or equivalent in oncology, including for TransCon TLR7/8 Agonist, in the fourth quarter of 2020, (vii) our plans to report six-month data from the open-label extension portion of the PaTH Forward Trial during the third quarter of 2020, (viii) our plans to engage with global regulatory authorities on next steps for development of TransCon PTH, (ix) our plans to submit regulatory filings to initiate a global phase 3 trial evaluating TransCon PTH in North America, Europe and Asia in the fourth quarter of 2020, (x) our ability to apply our TransCon platform to build a leading, fully integrated biopharma company, (xi) our expectations regarding our ability to create new and potentially best-in-class therapies and (xii) our product pipeline and expansion into additional therapeutic areas. 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; our ability to obtain additional funding, if needed, to support our business activities and the effects on our business of the worldwide COVID-19 pandemic. 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 filed with the SEC on April 3, 2020. 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)

	Three Months Ended March 31,	
	2020	**2019**
Revenue	2,225	5,414
Research and development costs	(57,515)	(51,259)
General and administrative expenses	(17,915)	(10,436)
Operating profit / (loss)	**(73,205)**	**(56,281)**
Share of profit / (loss) of associate	(1,515)	(1,852)
Finance income	11,773	4,620
Finance expenses	(447)	(194)
Profit / (loss) before tax	**(63,394)**	**(53,707)**
Tax on profit / (loss) for the year	77	70
Net profit / (loss) for the year	**(63,317)**	**(53,637)**
Other comprehensive income / (loss)		
Items that may be reclassified subsequently to profit or loss:		
Exchange differences on translating foreign operations	86	559
Other comprehensive income / (loss) for the year, net of tax	**86**	**559**
Total comprehensive income / (loss) for the year, net of tax	**(63,231)**	**(53,078)**
Profit / (loss) for the year attributable to owners of the Company	(63,317)	(53,637)
Total comprehensive income / (loss) for the year attributable to owners of the Company	(63,231)	(53,078)
	EUR	**EUR**
Basic and diluted earnings / (loss) per share	(1.32)	(1.24)
Number of shares used for calculation (basic and diluted)	47,985,837	43,371,559

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

	March 31, 2020	**December 31, 2019**
Assets		
Non-current assets		
Intangible assets	3,495	3,495
Property, plant and equipment	49,761	45,069
Investment in associate	15,307	15,538
Deposits	1,270	1,463
	69,833	**65,565**

Current assets		
Receivable from associate	1,312	804
Other receivables	4,775	3,136
Prepayments	10,185	7,648
Income taxes receivable	921	1,473
Cash and cash equivalents	534,381	598,106
	551,574	**611,167**
Total assets	**621,407**	**676,732**
Equity and liabilities		
Equity		
Share capital	6,443	6,443
Distributable equity	542,389	590,671
Total equity	**548,832**	**597,114**
Non-current liabilities		
Lease liabilities	30,760	30,720
Other payables	-	908
	30,760	**31,628**
Current liabilities		
Lease liabilities	6,307	5,899
Contract liabilities	343	858
Trade payables	27,277	27,765
Other payables	7,679	13,349
Income taxes payable	209	119
	41,815	**47,990**
Total liabilities	**72,575**	**79,618**
Total equity and liabilities	**621,407**	**676,732**

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