Ascendis Pharma A/S Reports Third Quarter 2020 Financial Results

November 11, 2020

– European Medicines Agency (EMA) validated the Marketing Authorisation Application (MAA) for TransCon^{TM} hGH (lonapegsomatropin) in pediatric growth hormone deficiency (GHD) –

– Submitted regulatory filings to enable initiation of European and Canadian sites for phase 3 PaTHway Trial for TransCon PTH –

– In collaboration with VISEN Pharmaceuticals, filed an Investigational New Drug Application (IND) to initiate the phase 2 ACcomplisH China Trial of TransCon CNP –

– On track for remaining 2020 corporate milestone of filing an IND or similar for TransCon TLR7/8 Agonist by year-end –

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

COPENHAGEN, Denmark, Nov. 11, 2020 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND), a biopharmaceutical company that utilizes its innovative TransCon™ technologies to create product candidates that address unmet medical needs, today announced financial results for the quarter ended September 30, 2020.

"As Ascendis progresses toward closing out 2020, I am proud that we are on track or ahead of schedule for all 2020 corporate goals and to achieve Vision 3x3, moving us one step closer to becoming a fully integrated, global biopharmaceutical company," said Jan Mikkelsen, President and CEO. "As a company, we are driven by our core values to achieve our long-term strategic Vision 3x3, using the power of the TransCon technology. We push ourselves to continually deliver not only in endocrinology but also in oncology and to bring these products to patients as efficiently and safely as possible."

Corporate Highlights & Progress

- TransCon hGH (lonapegsomatropin)
  - Submitted MAA to the EMA, which has now validated the application.
  - Filed a Clinical Trial Notification with the Pharmaceuticals and Medical Devices Agency in Japan to initiate the company's phase 3 riGHt Trial of lonapegsomatropin for the treatment of pediatric GHD.
  - Announced the U.S. Food and Drug Administration (FDA) accepted the company's Biologics License Application and set a PDUFA date for June 25, 2021.
- TransCon PTH
  - Submitted regulatory filings to enable initiation of European and Canadian sites for phase 3 PaTHway Trial.
  - Received orphan designation by the European Commission (EC) for the treatment of hypoparathyroidism (HP).
  - Submitted an amendment to its IND with FDA for the PaTHway phase 3 clinical trial evaluating the safety, tolerability and efficacy of TransCon PTH in adults with HP.
  - Announced preliminary six-month results from the open-label extension portion of PaTH Forward, a global phase 2 trial evaluating the safety, tolerability and efficacy of TransCon PTH in adult subjects with HP. The preliminary results support potential use of TransCon PTH as a hormone replacement therapy for HP and demonstrated normalization of quality of life.
- TransCon CNP
  - In collaboration with VISEN Pharmaceuticals, filed an IND application to initiate the phase 2 ACcomplisH China Trial.
  - Received orphan designation by the EC.
- Oncology
  - On track for filing IND or similar for TransCon TLR7/8 Agonist by year-end. TransCon TLR7/8 Agonist is a long-acting prodrug of resiquimod. Administered as an intratumoral injection, TransCon TLR7/8 Agonist is designed to provide sustained release of unmodified resiquimod directly to the tumor.
- Announced Mark A. Bach, M.D., Ph.D., as Senior Vice President of Clinical Development and Medical Affairs for Endocrinology Rare Diseases. Dr. Bach has 30 years of experience in clinical research and development.
- Ended the third quarter 2020 with cash, cash equivalents and marketable securities totaling €957.5 million.

Third Quarter 2020 Financial Results

For the third quarter, Ascendis Pharma reported a net loss of €121.7 million, or €2.31 per share (basic and diluted) compared to a net loss of €25.1 million, or €0.53 per share (basic and diluted) for the same period in 2019.

Revenue for the third quarter was €2.8 million compared to €2.2 million in the same quarter of 2019. The increase was due to a higher sale of clinical supply and services to VISEN, partly offset by a lower amount of license revenue being recognized.

Research and development (R&D) costs for the third quarter were €64.1 million compared to €46.3 million during the same period in 2019. Higher R&D costs in 2020 reflect an increase in personnel-related costs, expansion of R&D facilities and the continued progress in development of the company's product candidates.

Selling, general and administrative (SG&A) expenses for the third quarter were €17.5 million compared to €10.0 million during the same period in 2019. The increase is primarily due to additional personnel-related costs, higher IT and other infrastructure costs and continued build of the company's commercial capabilities.

As of September 30, 2020, Ascendis Pharma had cash, cash equivalents and marketable securities totaling €957.5 million compared to €471.6 million as of June 30, 2020. As of September 30, 2020, Ascendis Pharma had 53,416,091 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 2020 financial results. Details include:

Date	November 11, 2020
Time	4:30 p.m. ET
Dial In (U.S.)	844-290-3904
Dial In (International)	574-990-1036
Access Code	8955314

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, is designed as a once-weekly treatment for GHD and is currently under regulatory review by the U.S. Food and Drug Administration and the European Medicines Agency.
- TransCon PTH, an investigational long-acting prodrug of parathyroid hormone (PTH) in phase 3 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 similar by year-end for TransCon TLR7/8 Agonist, the company's first oncology product candidate.

About Ascendis Pharma A/S

Ascendis Pharma is applying its innovative TransCon technologies to build a leading, fully integrated biopharmaceutical 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 Ascendis' 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) Ascendis' 2020 corporate goals and Vision 3x3, (ii) Ascendis' PDUFA date for its Biologics License Application for lonapegsomatropin, (iii) Ascendis' planned IND filing for TransCon TLR7/8 Agonist by year-end, (iv) Ascendis' ability to apply its platform technology to build a leading, fully integrated biopharma company, (v) Ascendis' product pipeline and expansion into additional therapeutic areas and (vi) Ascendis' expectations regarding its ability to utilize its TransCon technologies to create new and potentially best-in-class therapies. Ascendis 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 Ascendis makes, including the following: unforeseen safety or efficacy results in its oncology programs, lonapegsomatropin, TransCon PTH and TransCon CNP or other development programs; unforeseen expenses related to the development and potential commercialization of its oncology programs, lonapegsomatropin, TransCon PTH and TransCon CNP or other development programs, selling, general and administrative expenses, other research and development expenses and

Ascendis' business generally; delays in the development of its oncology programs, lonapegsomatropin, 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; Ascendis' ability to obtain additional funding, if needed, to support its business activities and the effects on its 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 Ascendis' business in general, see Ascendis' prospectus supplement filed on July 9, 2020 and Ascendis' current and future reports filed with, or submitted to, the U.S. Securities and Exchange Commission (SEC), including its 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 that Ascendis may enter into or make. Ascendis does 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 September 30,		Nine Months Ended September 30,	
	2020	**2019**	**2020**	**2019**
Revenue	2,757	2,243	6,418	10,868
Research and development costs	(64,059)	(46,258)	(185,152)	(141,343)
Selling, general and administrative expenses	(17,523)	(10,000)	(56,243)	(31,396)
Operating profit / (loss)	**(78,825)**	**(54,015)**	**(234,977)**	**(161,871)**
Share of profit / (loss) of associate	(3,101)	(1,338)	(6,501)	(5,452)
Finance income	136	30,547	1,677	30,285
Finance expenses	(39,970)	(368)	(40,391)	(812)
Profit / (loss) before tax	**(121,760)**	**(25,174)**	**(280,192)**	**(137,850)**
Tax on profit / (loss) for the period	19	61	202	196
Net profit / (loss) for the period	**(121,741)**	**(25,113)**	**(279,990)**	**(137,654)**
Attributable to owners of the Company	(121,741)	(25,113)	(279,990)	(137,654)
Basic and diluted earnings / (loss) per share	€ (2.31)	€ (0.53)	€ (5.64)	€ (2.99)
Number of shares used for calculation (basic and diluted)	52,715,204	47,590,837	49,647,471	46,066,493
Net profit / (loss) for the period	**(121,741)**	**(25,113)**	**(279,990)**	**(137,654)**
Other comprehensive income / (loss)				
Items that may be reclassified subsequently to profit or loss:				
Exchange differences on translating foreign operations	(75)	37	(136)	2
Other comprehensive income / (loss) for the period, net of tax	**(75)**	**37**	**(136)**	**2**
Total comprehensive income / (loss) for the period, net of tax	**(121,816)**	**(25,076)**	**(280,126)**	**(137,652)**
Attributable to owners of the Company	(121,816)	(25,076)	(280,126)	(137,652)

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

	September 30,	**December 31,**

	2020	2019
Assets		
Non-current assets		
Intangible assets	4,653	3,495
Property, plant and equipment	47,325	45,069
Investment in associate	11,574	15,538
Deposits	1,243	1,463
Marketable securities	27,728	-
	92,523	**65,565**
Current assets		
Trade receivables	2,722	804
Other receivables	4,511	3,136
Prepayments	15,266	7,648
Income taxes receivable	1,305	1,473
Marketable securities	166,343	-
Cash and cash equivalents	763,454	598,106
	953,601	**611,167**
Total assets	**1,046,124**	**676,732**
Equity and liabilities		
Equity		
Share capital	7,172	6,443
Distributable equity	944,413	590,671
Total equity	**951,585**	**597,114**
Non-current liabilities		
Lease liabilities	26,952	30,720
Other payables	3,160	908
	30,112	**31,628**
Current liabilities		
Lease liabilities	6,328	5,899
Contract liabilities	223	858
Trade payables and accrued expenses	38,108	27,765
Other payables	19,549	13,349
Income taxes payable	219	119
	64,427	**47,990**
Total liabilities	**94,539**	**79,618**
Total equity and liabilities	**1,046,124**	**676,732**

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