Ascendis Pharma A/S Reports Full Year 2020 Financial Results

March 10, 2021 at 4:01 PM EST

– Preparations for potential launch of first Endocrinology Rare Disease product candidate, TransCon hGH (lonapegsomatropin), continues on track –

– Oncology pipeline advancing into clinical trials beginning with TransCon TLR7/8 Agonist –

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

COPENHAGEN, Denmark, March 10, 2021 (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 full year ended December 31, 2020.

“2020 was a year to remember for Ascendis as our global workforce delivered on time or ahead of schedule on all of our corporate milestones, advancing Vision 3x3, the company’s strategic roadmap through 2025 to build a leading biopharma company by achieving sustainable growth through multiple approaches,” said Jan Mikkelsen, Ascendis’ President and CEO. “As we look to 2021, we expect to report important clinical updates across our entire investigational product portfolio, including Endocrinology Rare Disease and Oncology, while also achieving key commercial milestones including the anticipated regulatory approval and commercial launch in the United States, and regulatory approval in Europe, of TransCon hGH for pediatric growth hormone deficiency.”

Company Highlights & Progress

- TransCon hGH (lonapegsomatropin)
  - ○ Continued to build the global commercial infrastructure in preparation for the PDUFA date of June 25, 2021, and expected subsequent commercial launch of lonapegsomatropin in the United States in the third quarter of 2021. European Commission decision is anticipated in the fourth quarter of 2021 for pediatric patients with growth hormone deficiency (GHD).
  - ○ Reported two year data from the enliGHten long-term extension trial demonstrating children with GHD initially treated with lonapegsomatropin maintained an advantage in height SDS (standard deviation score) improvement.
  - ○ Continued execution in the ongoing foresiGHt Trial, a global phase 3 study in adults with GHD, complete enrollment expected by late 2021 or early 2022.
  - ○ Submitted Clinical Trial Notification (CTN) to initiate pediatric GHD phase 3 riGHt Trial in Japan.
- TransCon PTH
  - ○ Submitted regulatory filings in North America and Europe to initiate PaTHway, a phase 3 clinical trial evaluating the safety, tolerability and efficacy of TransCon PTH in adults with hypoparathyroidism (HP). Topline results are expected from PaTHway in the fourth quarter of 2021.
  - ○ Announced six-month data from the PaTH Forward Trial open-label extension (OLE), which demonstrated TransCon PTH replaced standard of care, normalized quality of life and urinary calcium. These results will be presented in an oral presentation on March 23, 2021 at ENDO 2021.
  - ○ As of March 10, 2021, 58 out of the 59 randomized subjects continue in the phase 2 PaTH Forward clinical trial.
  - ○ On track to file CTN to initiate adult HP phase 3 trial in Japan in the second quarter of 2021.
- TransCon CNP
  - ○ Continued dose escalation in the phase 2 ACcomplisH Trial to evaluate the safety and efficacy of TransCon CNP in children ages two to ten with achondroplasia.
  - ○ IND approved for the ACcomplisH China Trial, a phase 2 trial to enable dose expansion cohorts, in children ages two to ten, conducted in Greater China by Visen Pharmaceuticals (VISEN).
  - ○ Clinical program update on both randomized, double-blind, placebo-controlled trials expected in the fourth quarter of 2021.
- TransCon TLR7/8 Agonist
  - ○ Filed IND application with the FDA to initiate the transcendIT-101 clinical trial.
  - ○ Initial results from the first part of transcendIT-101, the monotherapy dose escalation, are expected in the fourth quarter of 2021.
  - ○ Expect to initiate the second part of transcendIT-101, dose escalation of TransCon TLR7/8 Agonist in combination with a checkpoint inhibitor, in the second quarter of 2021.
- TransCon IL-2 β/γ
  - ○ New preclinical data demonstrated a single dose of TransCon IL-2 β/γ resulted in durable and robust increases in the ratios of CD8+ T cells and NK cells over Treg cells in non-human primates.

    - IND filing or similar planned in the third quarter of 2021.

- Ended 2020 with cash, cash equivalents and marketable securities totaling €834.1 million.

Full Year 2020 Financial Results

For the full year 2020, Ascendis Pharma reported a net loss of €419.0 million, or €8.28 per share (basic and diluted) compared to a net loss of €218.0 million, or €4.69 per share (basic and diluted) for the same period in 2019.

Revenue for 2020 was €7.0 million compared to €13.4 million in 2019. The decrease was due to lower license and service revenue, partly offset by sale of clinical supply, to VISEN.

Research and development (R&D) costs for 2020 were €260.9 million compared to €191.6 million 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 expenses for 2020 were €76.7 million compared to €48.5 million in 2019. The increase is primarily due to higher personnel-related costs, higher IT costs and continued build out of the company's commercial capabilities.

Net loss in associate was €9.5 million for 2020 compared to €8.1 million in 2019, representing the company's share of net result from VISEN.

Net finance expenses were €79.0 million for 2020 compared to net finance income of €16.6 million in 2019, primarily reflecting negative exchange rate fluctuations compared to 2019 when the company recognized a gain.

As of December 31, 2020, Ascendis Pharma had cash, cash equivalents and marketable securities totaling €834.1 million compared to €598.1 million at the beginning of the year. As of December 31, 2020, Ascendis Pharma had 53,750,386 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 2020 financial results. Details include:

Date	March 10, 2021
Time	4:30 p.m. ET / 1:30 p.m. PT
Dial In (U.S.)	844-290-3904
Dial In (International)	574-990-1036
Access Code	1996657

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 and one oncology product candidate in clinical development:

- 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 for pediatric GHD 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 adults with hypoparathyroidism (HP) designed to replace PTH at physiologic levels for 24 hours, 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.
- TransCon TLR7/8 Agonist is an investigational long-acting prodrug of resiquimod, a small molecule agonist of Toll-like receptors (TLR) 7 and 8. Administered as an intratumoral injection, TransCon TLR7/8 Agonist is designed to provide sustained activation of intratumoral antigen presenting cells driving tumor antigen presentation and induction of immune stimulatory cytokines in the tumor.
- TransCon IL-2 β/γ is an investigational long-acting prodrug of IL-2 β/γ designed for optimized IL-2Rβ/γ bias and potency, combined with low Cmax and long exposure.

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 and one oncology product candidate in clinical development. 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, Palo Alto and Redwood City, California, and Princeton, New Jersey.

Please visit www.ascendispharma.com (for global information) or www.ascendispharma.us (for U.S. information.)

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' expectations regarding Ascendis' strategic roadmap through 2025 to build a leading biopharma company by achieving sustainable growth, (ii) Ascendis' expectations regarding potential commercial launch of lonapegsomatropin, (iii) Ascendis' 2020 corporate goals and Vision 3x3, (iv) Ascendis' PDUFA date for its Biologics License Application for lonapegsomatropin, (v) Ascendis' ability to apply its platform technology to build a leading, fully integrated biopharma company, (vi) Ascendis' product pipeline and expansion into additional therapeutic areas and (vii) 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)

	Year ended December 31,	
	2020	**2019**
Revenue	6,953	13,375
Research and development costs	(260,904)	(191,621)
Selling, general and administrative expenses	(76,669)	(48,473)
Operating profit / (loss)	**(330,620)**	**(226,719)**
Share of profit / (loss) of associate	(9,524)	(8,113)
Finance income	1,812	17,803
Finance expenses	(80,842)	(1,221)
Profit / (loss) before tax	**(419,174)**	**(218,250)**
Tax on profit / (loss) for the year	219	234
Net profit / (loss) for the year	**(418,955)**	**(218,016)**
Attributable to owners of the Company	(418,955)	(218,016)
Basic and diluted earnings / (loss) per share	€ (8.28)	€ (4.69)
Number of shares used for calculation (basic and diluted)	50,616,528	46,506,862

Net profit / (loss) for the year	**(418,955)**	**(218,016)**
Other comprehensive income / (loss)		
Items that may be reclassified subsequently to profit or loss:		
Exchange differences on translating foreign operations	(42)	(37)
Other comprehensive income / (loss) for the year, net of tax	**(42)**	**(37)**
Total comprehensive income / (loss) for the year, net of tax	**(418,997)**	**(218,053)**
Attributable to owners of the Company	(418,997)	(218,053)

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

	December 31, 2020	December 31, 2019
Assets		
Non-current assets		
Intangible assets	5,717	3,495
Property, plant and equipment	108,112	45,069
Investment in associate	9,176	15,538
Other receivables	1,375	1,463
Marketable securities	115,280	-
	239,660	**65,565**
Current assets		
Trade receivables	387	804
Other receivables	6,957	4,609
Prepayments	13,994	7,648
Marketable securities	134,278	-
Cash and cash equivalents	584,517	598,106
	740,133	**611,167**
Total assets	**979,793**	**676,732**
Equity and liabilities		
Equity		
Share capital	7,217	6,443
Distributable equity	831,494	590,671
Total equity	**838,711**	**597,114**
Non-current liabilities		
Lease liabilities	85,116	30,720
Other liabilities	3,162	908
	88,278	**31,628**
Current liabilities		
Lease liabilities	6,859	5,899
Contract liabilities	363	858
Trade payables and accrued expenses	21,897	27,765
Other liabilities	23,384	13,349
Income taxes payable	301	119
	52,804	**47,990**
Total liabilities	**141,082**	**79,618**
Total equity and liabilities	**979,793**	**676,732**

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Source: Ascendis Pharma