Ascendis Pharma A/S Reports First Quarter 2021 Financial Results

May 27, 2021 at 4:01 PM EDT

– Pre-launch activities continue in preparation for a potential FDA approval of TransCon™ hGH (lonapegsomatropin) for pediatric growth hormone deficiency; PDUFA date of June 25, 2021 –

– 58-week data from PaTH Forward demonstrated durable benefit of TransCon PTH signifying potential as a hormone replacement therapy for patients with hypoparathyroidism –

– First-in-human dosing for Ascendis Oncology portfolio with the transcendIT-101 clinical trial moving forward –

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

COPENHAGEN, Denmark, May 27, 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 first quarter ended March 31, 2021.

"With potential U.S. FDA approval for TransCon hGH now less than a month away, we believe we are one step closer to fulfilling our Vision 3x3 to build a leading biopharma company. Guided by our values of patients, science and passion, we have built a pipeline of three differentiated endocrinology rare disease product candidates by applying our TransCon technology to clinically validated parent drugs and/or targets. Each candidate targeting substantial unmet medical needs in large markets where we have the potential to become the market leader. We plan to replicate that success in oncology where we have dosed our first patients this past quarter in the transcendIT-101 Trial of TransCon TLR7/8 Agonist and expect to file an IND or similar for our second oncology product candidate TransCon IL-2 β/γ in Q3 2021. Improving patient lives is the driving force behind our dedicated employees," said Jan Mikkelsen, Ascendis Pharma's President and Chief Executive Officer.

Company Highlights & Progress

- TransCon hGH (lonapegsomatropin)
  - Continued pre-launch commercial activities in preparation of the PDUFA date on June 25, 2021 and an expected subsequent commercial launch of lonapegsomatropin in the United States in the third quarter of 2021 for the treatment of pediatric patients with growth hormone deficiency (GHD). All FDA information requests relating to our BLA for TransCon hGH for the treatment of pediatric GHD have been responded to with no questions currently outstanding.
  - European Commission decision on the company's Marketing Authorisation Application (MAA) is anticipated in the fourth quarter of 2021 for the treatment of pediatric patients with GHD.
  - A recent study published online in the Journal of Managed Care & Specialty Pharmacy[1] and presented at ENDO 2021 highlighted the economic burden of GHD in the U.S. pediatric population. The study demonstrated that pediatric GHD is a significant healthcare burden, and many patients remain untreated or undertreated with high rates of non-adherence (70-80%).
  - Continued execution in the ongoing foresiGHt Trial, a global phase 3 trial in adults with GHD, complete enrollment expected by late 2021 or early 2022.
  - Continued execution in the ongoing riGHt Trial, a Japanese phase 3 trial for pediatric GHD.
  - In Greater China, VISEN Pharmaceuticals completed the patient enrollment of 154 treatment-naïve, prepubertal children for the ongoing phase 3 pivotal trial of lonapegsomatropin in patients with pediatric GHD.
- TransCon PTH
  - Announced preliminary 58-week results from the continuing open-label extension (OLE) portion of the PaTH Forward Trial, a global phase 2 trial evaluating the safety, tolerability, and efficacy of its investigational product candidate TransCon PTH in adult subjects with hypoparathyroidism (HP). The results demonstrated that TransCon PTH was well tolerated at all doses administered and provided durable benefit in adults with HP. As of May 25, 2021, 58 out of the 59 randomized subjects continue in the OLE portion of the phase 2 PaTH Forward Trial.
  - Filed a Clinical Trial Notification with the Pharmaceuticals and Medical Devices Agency in Japan to initiate the company's phase 3 clinical trial of TransCon PTH in adult subjects with HP, the PaTHway Japan Trial.
  - Continued execution in the ongoing North American and European phase 3 PaTHway Trial in adults with HP with topline results expected in the fourth quarter of 2021.
- TransCon CNP
  - Continued execution in the ongoing phase 2 ACcomplisH Trial and ACcomplisH China Trial to evaluate the safety and efficacy of TransCon CNP in children ages two to ten with achondroplasia.
  - On track for clinical program update on both randomized, double-blind, placebo-controlled trials expected in the

fourth quarter of 2021.

- TransCon TLR7/8 Agonist
  - Continued execution in the ongoing phase 1/2 trial, transcendIT-101.
- TransCon IL-2 β/γ
  - IND filing or similar planned in the third quarter of 2021.
- Ended the first quarter of 2021 with cash, cash equivalents and marketable securities totaling €771.1 million.

First Quarter 2021 Financial Results

For the first quarter, Ascendis Pharma reported a net loss of €62.8 million, or €1.17 per share (basic and diluted) compared to a net loss of €63.3 million, or €1.32 per share (basic and diluted) for the same period in 2020.

Revenue for the first quarter was €0.7 million compared to €2.2 million in the same quarter of 2020. The decrease was due primarily to a lower amount of license revenue being recognized, as well as lower sale of clinical supply and services to VISEN compared to the same period the prior year.

Research and development (R&D) costs for the first quarter were €88.1 million compared to €57.5 million during the same period in 2020. Higher R&D costs in 2021 reflect an increase in personnel-related costs and continued progress in development of the company's product candidates.

Selling, general and administrative expenses for the first quarter were €37.2 million compared to €17.9 million during the same period in 2020. The increase is primarily due to higher personnel-related costs, continued build out of the company's commercial capabilities, and a change in expense reallocation of shared services.

Net profit of associate for the first quarter was €28.1 million compared to a net loss of €1.5 million in the same quarter of 2020. Net profit of associate for the first quarter included a non-cash gain of €42.3 million as a result of the Series B financing in VISEN on January 8, 2021, partly offset by our share of VISEN's net loss for the first quarter of €14.2 million.

As of March 31, 2021, Ascendis Pharma had cash, cash equivalents and marketable securities of €771.1 million compared to €834.1 million as of December 31, 2020. As of March 31, 2021, Ascendis Pharma had 53,829,379 ordinary shares outstanding.

Conference Call and Webcast Information

Date	Thursday, May 27, 2021
Time	4:30 p.m. Eastern Time/1:30 p.m. Pacific Time
Dial In (U.S.)	844-290-3904
Dial In (International)	574-990-1036
Access Code	4986247

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 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 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-2R β/γ bias and potency, combined with low Cmax and long exposure.

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 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 facilities in Heidelberg and Berlin, Germany, in Palo Alto and Redwood City, California, and in 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 the potential commercial launch of lonapegsomatropin, (ii) Ascendis' corporate goals and Vision 3x3, (iii) Ascendis' PDUFA date for its Biologics License Application for lonapegsomatropin, (iv) Ascendis' planned IND or similar submission for TransCon IL-2 β/γ in Q3 2021, (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' Annual Report on Form 20-F filed with the U.S. Securities and Exchange Commission (SEC) on March 10, 2021 and Ascendis' current and future reports filed with, or submitted to, the SEC. 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 March 31,	
	2021	**2020**
Revenue	746	2,225
Research and development costs	(88,149)	(57,515)
Selling, general and administrative expenses	(37,247)	(17,915)
Operating profit / (loss)	**(124,650)**	**(73,205)**
Share of profit / (loss) of associate	28,106	(1,515)
Finance income	34,430	11,773
Finance expenses	(869)	(447)
Profit / (loss) before tax	**(62,983)**	**(63,394)**
Tax on profit / (loss) for the period	191	77
Net profit / (loss) for the period	**(62,792)**	**(63,317)**
Attributable to owners of the Company	(62,792)	(63,317)
Basic and diluted earnings / (loss) per share	€ (1.17)	€ (1.32)
Number of shares used for calculation (basic and diluted)	53,759,952	47,985,837

Net profit / (loss) for the period	**(62,792)**	**(63,317)**
Other comprehensive income / (loss)		
Items that may be reclassified subsequently to profit or loss:		
Exchange differences on translating foreign operations	1,842	86
Other comprehensive income / (loss) for the period, net of tax	**1,842**	**86**
Total comprehensive income / (loss) for the period, net of tax	**(60,950)**	**(63,231)**
Attributable to owners of the Company	(60,950)	(63,231)

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

	March 31 2021	December 31, 2020
Assets		
Non-current assets		
Intangible assets	5,606	5,717
Property, plant and equipment	114,196	108,112
Investment in associate	50,035	9,176
Other receivables	1,779	1,375
Marketable securities	106,426	115,280
	278,042	**239,660**
Current assets		
Trade receivables	60	387
Other receivables	7,183	6,957
Prepayments	15,322	13,994
Marketable securities	169,659	134,278
Cash and cash equivalents	495,047	584,517
	687,271	**740,133**
Total assets	**965,313**	**979,793**
Equity and liabilities		
Equity		
Share capital	7,228	7,217
Distributable equity	795,591	831,494
Total equity	**802,819**	**838,711**
Non-current liabilities		
Lease liabilities	89,568	85,116
Other liabilities	-	3,162
	89,568	**88,278**
Current liabilities		
Lease liabilities	6,913	6,859
Contract liabilities	254	363
Trade payables and accrued expenses	54,133	21,897
Other payables	11,253	23,384
Income taxes payable	373	301
	72,926	**52,804**
Total liabilities	**162,494**	**141,082**
Total equity and liabilities	**965,313**	**979,793**

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[1] Kaplowitz P, et al. Economic burden of growth hormone deficiency in a US pediatric population. *J Manag Care Spec Pharm*. 2021 Apr 24:1-11. doi: 10.18553/jmcp.2021.21030.

Source: Ascendis Pharma