



## **Ascendis Pharma A/S Reports Second Quarter 2020 Financial Results and Announces New Data from the Four-Week, Fixed Dose, Double-Blind Portion of the PaTH Forward Trial**

August 27, 2020

**– U.S. Biologics License Application for TransCon™ hGH submitted to U.S. Food and Drug Administration for pediatric growth hormone deficiency –**

**– Approval of Paediatric Investigation Plan by European Medicines Agency clears path for filing Marketing Authorisation Application for TransCon hGH in Europe in third quarter –**

**– New data from the four-week, fixed dose, double-blind portion of PaTH Forward demonstrated statistically significant and clinically meaningful improvements in SF-36 functional health and well being outcomes –**

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

COPENHAGEN, Denmark, Aug. 27, 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 new data from PaTH Forward and financial results for the quarter ended June 30, 2020.

"We are executing across the globe on all elements of our Vision 3x3, including preparation for the expected U.S. launch for TransCon hGH following submission of our Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) in June, and preparing to submit the Marketing Authorisation Application (MAA) to the European Medicines Agency (EMA) for TransCon hGH in Europe planned for third quarter," said Jan Mikkelsen, Ascendis Pharma's President and Chief Executive Officer.

Mr. Mikkelsen continued, "Additionally, with today's data release, we have, for the first time in a randomized, double-blind, placebo-controlled trial, demonstrated that a therapy for hypoparathyroidism (HP) may have a significant impact on improving quality of life for people living with HP compared to the standard of care. Our new data showed that TransCon PTH demonstrated a statistically significant<sup>1</sup> and clinically meaningful impact on the SF-36<sup>®</sup> Health Survey (SF-36), a quality of life assessment tool validated to measure functional health and well being compared to placebo.<sup>2</sup> Analysis of these exploratory endpoints, combined with expected findings from our proprietary patient reported outcome instrument, called Hypoparathyroidism Patient Experience Scale (HPES), will support the emerging body of evidence that TransCon PTH may function as a hormone replacement therapy and make a meaningful difference in improving the lives of people with HP."

### **Corporate Highlights & Progress**

- Submitted a BLA for TransCon hGH for pediatric growth hormone deficiency to the FDA on June 25, 2020.
- In July, received approval of its proposed Paediatric Investigation Plan covering ages 6 months to less than 18 years of age from EMA for TransCon hGH and remain on track for a planned third quarter 2020 MAA submission to the EMA for pediatric growth hormone deficiency.
- Today announced new data on exploratory endpoints 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 on TransCon PTH from the validated SF-36 quality-of-life instrument showed a statistically significant improvement related to both the physical (LS mean difference=5.2; p=0.013) and mental (LS mean difference=9.8; p=0.0003) components of the measure compared to placebo.<sup>1</sup> Importantly, the results were consistent with a clinically meaningful improvement in functional health and well being for subjects receiving TransCon PTH as compared with placebo after four weeks.<sup>2</sup> Detailed results are included in the company's investor presentation which can be found on the company's website at [www.ascendispharma.com](http://www.ascendispharma.com). The company plans to present additional data and analyses from the PaTH Forward trial four-week results at upcoming medical conferences, anticipates announcing the six-month data from the open-label extension portion of the PaTH Forward Trial during the third quarter and is preparing to submit regulatory filings to initiate a phase 3 trial in the fourth quarter.
- In July, received orphan designation by the European Commission (EC) for TransCon C-Type Natriuretic Peptide (CNP), an investigational long-acting prodrug of CNP in development as a therapy for achondroplasia, the most common form of dwarfism.<sup>3</sup>
- Presented preclinical data for TransCon IL-2  $\beta/\gamma$ , an oncology product candidate designed to provide sustained systemic release of a receptor-biased IL-2 (IL-2  $\beta/\gamma$ ), at the American Association of Cancer Research (AACR) Virtual Annual Meeting II. Results showed that TransCon IL-2  $\beta/\gamma$  demonstrated a long in vivo half-life of approximately 32 hours, expected to support potential dosing of every three weeks in patients.<sup>4</sup>

- After the end of the second quarter, the company announced the completion of its underwritten public offering of 4,859,154 American Depositary Shares (“ADSs”). Net proceeds from this offering in July 2020 were approximately \$654.7 million, or approximately €580.7 million based on exchange rates on the date of the closing.
- Ended the second quarter 2020 with cash, cash equivalents and marketable securities totaling €471.6 million, excluding the net proceeds from the July 2020 equity offering.

### Second Quarter 2020 Financial Results

For the second quarter, Ascendis Pharma reported a net loss of €94.9 million, or €1.97 per share (basic and diluted) compared to a net loss of €58.9 million, or €1.25 per share (basic and diluted) for the same period in 2019.

Revenue for the second quarter was €1.4 million compared to €3.2 million in the same quarter of 2019. The decrease was due to a lower amount of license and service revenue being recognized from the company’s strategic investment in VISEN Pharmaceuticals.

Research and development (R&D) costs for the second quarter were €63.6 million compared to €43.8 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.

Selling, general and administrative (SG&A) expenses for the second quarter were €20.8 million compared to €11.0 million during the same period in 2019. The increase is primarily due to additional personnel-related costs, higher IT and other site costs, and continued build out of the company’s commercial capabilities.

As of June 30, 2020, Ascendis Pharma had cash, cash equivalents and marketable securities totaling €471.6 million compared to €534.4 million as of March 31, 2020. As of June 30, 2020, Ascendis Pharma had 48,345,782 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 second quarter 2020 financial results. Details include:

<b>Date</b>	August 27, 2020
<b>Time</b>	4:30 p.m. ET
<b>Dial In (U.S.)</b>	844-290-3904
<b>Dial In (International)</b>	574-990-1036
<b>Access Code</b>	9987362

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](http://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<sup>3</sup>, for which there is no FDA-approved treatment<sup>5</sup>. 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 in the fourth quarter of 2020 for TransCon TLR7/8 Agonist, the company’s 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.

### 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' plans to submit its marketing application for TransCon hGH in Europe in the third quarter of 2020, (ii) Ascendis' plans to report six-month data from the open-label extension portion of the PaTH Forward Trial during the third quarter of 2020, (iii) Ascendis' 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, (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, TransCon hGH, TransCon PTH and TransCon CNP or other development programs; unforeseen expenses related to the development and potential commercialization of its oncology programs, TransCon hGH, 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, 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; 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.

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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 June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Revenue	1,436	3,211	3,661	8,625
Research and development costs	(63,578)	(43,826)	(121,093)	(95,085)
Selling, general and administrative expenses	(20,805)	(10,960)	(38,720)	(21,396)
<b>Operating profit / (loss)</b>	<b>(82,947)</b>	<b>(51,575)</b>	<b>(156,152)</b>	<b>(107,856)</b>
Share of profit / (loss) of associate	(1,885)	(2,262)	(3,400)	(4,114)
Finance income	86	3,362	1,996	4,917
Finance expenses	(10,292)	(8,494)	(876)	(5,623)
<b>Profit / (loss) before tax</b>	<b>(95,038)</b>	<b>(58,969)</b>	<b>(158,432)</b>	<b>(112,676)</b>
Tax on profit / (loss) for the year	106	65	183	135
<b>Net profit / (loss) for the year</b>	<b>(94,932)</b>	<b>(58,904)</b>	<b>(158,249)</b>	<b>(112,541)</b>
Attributable to owners of the Company	(94,932)	(58,904)	(158,249)	(112,541)
Basic and diluted earnings / (loss) per share	€ (1.97)	€ (1.25)	€ (3.29)	€ (2.48)
Number of shares used for calculation (basic and diluted)	48,207,661	47,190,717	48,096,749	45,291,688
<b>Net profit / (loss) for the year</b>	<b>(94,932)</b>	<b>(58,904)</b>	<b>(158,249)</b>	<b>(112,541)</b>
<b>Other comprehensive income / (loss)</b>				
<i>Items that may be reclassified subsequently to profit or loss:</i>				
Exchange differences on translating foreign operations	(147)	(594)	(61)	(35)
<b>Other comprehensive income / (loss) for the year, net of tax</b>	<b>(147)</b>	<b>(594)</b>	<b>(61)</b>	<b>(35)</b>

Total comprehensive income / (loss) for the year, net of tax	(95,079	)	(59,498	)	(158,310	)	(112,576	)
Attributable to owners of the Company	(95,079	)	(59,498	)	(158,310	)	(112,576	)

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

	June 30, 2020	December 31, 2019
<b>Assets</b>		
<b>Non-current assets</b>		
Intangible assets	3,806	3,495
Property, plant and equipment	49,743	45,069
Investment in associate	14,167	15,538
Deposits	1,268	1,463
	<b>68,984</b>	<b>65,565</b>
<b>Current assets</b>		
Receivable from associate	588	804
Other receivables	5,332	3,136
Prepayments	13,033	7,648
Income taxes receivable	1,107	1,473
Marketable securities	230,958	-
Cash and cash equivalents	240,605	598,106
	<b>491,623</b>	<b>611,167</b>
<b>Total assets</b>	<b>560,607</b>	<b>676,732</b>
<b>Equity and liabilities</b>		
<b>Equity</b>		
Share capital	6,491	6,443
Distributable equity	470,653	590,671
<b>Total equity</b>	<b>477,144</b>	<b>597,114</b>
<b>Non-current liabilities</b>		
Lease liabilities	29,092	30,720
Other payables	-	908
	<b>29,092</b>	<b>31,628</b>
<b>Current liabilities</b>		
Lease liabilities	6,389	5,899
Contract liabilities	-	858
Trade payables	31,575	27,765
Other payables	16,221	13,349
Income taxes payable	186	119
	<b>54,371</b>	<b>47,990</b>
<b>Total liabilities</b>	<b>83,463</b>	<b>79,618</b>
<b>Total equity and liabilities</b>	<b>560,607</b>	<b>676,732</b>

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<sup>1</sup> Data on file.

<sup>2</sup> Maruish, M. E. (Ed.). *User's manual for the SF-36v2 Health Survey* (3rd ed.). Lincoln, RI: QualityMetric Incorporated.

<sup>3</sup> Horton WA, et al. *Lancet*. 2007;370(9582):162–172.

<sup>4</sup> D.B. Rosen, et al. Poster presented at the American Association of Cancer Research (AACR) 2020 Virtual Annual Meeting II, June 22–24 (virtual).

<sup>5</sup> Pauli RM. *J Rare Dis*. 2019; 14:1



Source: Ascendis Pharma