Ascendis Pharma A/S Reports Second Quarter 2021 Financial Results

August 25, 2021

– Announced U.S. Food and Drug Administration Approval of SKYTROFA® (lonapegsomatropin-tcgd), the First Once-weekly Treatment for Pediatric Growth Hormone Deficiency –

– Exceeded target enrollment in Phase 3 PaTHway Trial for TransCon PTH (palopegteriparatide) in adults with hypoparathyroidism (HP); top-line results expected in Q1 2022 –

– Initiated combination therapy arm in transcendIT-101; TransCon TLR7/8 Agonist used in combination with a check point inhibitor (CPI) –

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

COPENHAGEN, Denmark, Aug. 25, 2021 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND), a biopharmaceutical company that utilizes its innovative TransCon™ technologies to potentially create new treatments that make a meaningful difference in patients' lives, today announced financial results for the second quarter ended June 30, 2021.

"We are actively preparing for the U.S. commercial launch of SKYTROFA for the treatment of children with GHD, which is now the first FDA-approved once-weekly treatment for pediatric GHD. SKTROFA is also the first FDA-approved product utilizing our innovative TransCon technology. Our pivotal heiGHt Trial demonstrated that once-weekly TransCon hGH increased annualized height velocity in treatment-naïve subjects at 52 weeks compared to a daily growth hormone with comparable safety and tolerability," said Jan Mikkelsen, Ascendis Pharma's President and Chief Executive Officer. "We see this approval as the first step in creating a market leading product and building a fully integrated global biopharmaceutical company guided by our values of patients, science, and passion."

Company Highlights & Progress

- TransCon hGH (lonapegsomatropin)
  - TransCon hGH is now FDA approved in the U.S. under the brand name SKYTROFA. Continued preparation for commercial launch for the treatment of pediatric patients with GHD in the U.S.
  - European Commission decision on the company's Marketing Authorisation Application (MAA) for the treatment of pediatric patients with GHD is anticipated in the fourth quarter of 2021.
  - Ongoing enrollment in the foresiGHt Trial, a global phase 3 trial in adults with GHD, and the riGHt Trial, a phase 3 trial in Japan in pediatric patients with GHD.
  - Patient follow-up continues in enliGHten, a multi-center phase 3, long-term open-label trial investigating safety and efficacy of SKYTROFA in pediatric patients with GHD.
  - Comprehensive results from the heiGHt Trial recently published on-line in the *Journal of Clinical Endocrinology & Metabolism*, an official journal of the Endocrine Society.
- TransCon PTH (palopegteriparatide)
  - Exceeded target enrollment in the PaTHway Trial, a phase 3 trial evaluating the safety, tolerability, and efficacy of palopegteriparatide in adult subjects with hypoparathyroidism with similar demographics as enrolled in the phase 2 trial including broad representation of different non-surgical disease etiologies and leading influential clinical sites balanced between North America and Europe.
  - On track to announce 84-week top line results from the open label extension (OLE) portion of the PaTH Forward Trial in the fourth quarter of 2021. Continued strong long-term subject retention with 58 out of the 59 randomized subjects continuing in the OLE portion of the trial as of August 23, 2021.
  - Clinical trial notification for the PaTHway Japan Trial was accepted by the Japanese Pharmaceuticals and Medical Device Agency. The single-arm, phase 3 study will enroll a minimum of 12 Japanese subjects with HP.
  - Received Orphan Drug Designation (ODD) from the Japanese Ministry of Health, Labor and Welfare.
  - VISEN Pharmaceuticals (VISEN) obtained investigational new drug (IND) approval to initiate the phase 3 PaTHway China Trial.
- 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 years with achondroplasia.
  - Clinical program update planned for the fourth quarter of 2021.
- TransCon TLR7/8 Agonist
  - Initiated combination therapy arm in transcendIT-101 with TLR7/8 Agonist and a CPI.
- TransCon IL-2 ß/y
  - IND filing on track for this quarter.

- Ended the second quarter of 2021 with cash, cash equivalents and marketable securities totaling €641.3 million.

Second Quarter 2021 Financial Results

For the second quarter, Ascendis Pharma reported a net loss of €134.4 million, or €2.50 per share (basic and diluted) compared to a net loss of €94.9 million, or €1.97 per share (basic and diluted) for the same period in 2020.

Revenue for the second quarter was €1.0 million compared to €1.4 million in the same quarter of 2020. The decrease was due to a lower amount of license revenue being recognized, partly offset by higher sale of clinical supplies and services to VISEN and recognition of revenue from services rendered to another collaboration partner.

Research and development (R&D) costs for the second quarter were €83.3 million compared to €63.6 million during the same period in 2020. Higher R&D costs in 2021 reflect an increase in external development costs of the company's product candidates and an increase in personnel-related costs.

Selling, general and administrative expenses for the second quarter were €35.3 million compared to €20.8 million during the same period in 2020. The increase is primarily due to higher personnel-related costs and an increase in IT costs.

Net loss of associate for the second quarter was €4.8 million compared to a net loss of €1.9 million in the same quarter of 2020. The net loss of associate represents our share of the net result from VISEN.

As of June 30, 2021, Ascendis Pharma had cash, cash equivalents and marketable securities of €641.3 million compared to €771.1 million as of March 31, 2021. As of June 30, 2021, Ascendis Pharma had 53,900,990 ordinary shares outstanding.

Conference Call Details

Date	Wednesday, August 25, 2021
Time	4:30 p.m. ET/1:30 p.m. Pacific Time
Dial In (U.S.)	844-290-3904
Dial In (International)	574-990-1036
Access Code	8553236

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-tcgd), 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 approved for pediatric GHD by the U.S. Food and Drug Administration and under review by the European Medicines Agency.
- TransCon PTH (palopegteriparatide), 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 ß/y is an investigational long-acting prodrug of IL-2 ß/y designed for optimized IL-2R ß/y 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 multiple independent endocrinology rare disease and oncology product candidates in 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 U.S. commercial launch of SKYTROFA, (ii) Ascendis' planned IND submission for TransCon IL-2 ß/y in the third quarter of 2021, (iii) Ascendis' expectations regarding the European Commission's decision on its Marketing Authorisation Application in the fourth quarter of 2021, (iv) Ascendis' expectations regarding the announcement of top line results from the OLE portion of the PaTH Forward Trial in the fourth quarter of 2021, (v) Ascendis' expectations regarding the announcement of top line results from the PaTHway Trial in the first quarter of 2022, (vi) Ascendis' ability to apply its platform technology to build a leading, fully integrated biopharma company, (vii) Ascendis' product pipeline and expansion into additional therapeutic areas and (viii) 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: dependence on third party manufacturers to supply SKYTROFA, the SKYTROFA® Auto-Injector and other study drug for commercial sales and clinical studies; unforeseen safety or efficacy results in its oncology programs, SKYTROFA, TransCon PTH and TransCon CNP or other development programs; unforeseen expenses related to commercialization of SKYTROFA and the further development of SKYTROFA, expenses related to the development and potential commercialization of its oncology programs, 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, SKYTROFA, 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 from 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' other 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 Comprehensive Income / (loss)
(In EUR'000s, except share and per share data)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2021	**2020**	**2021**	**2020**
Revenue	1,022	1,436	1,767	3,661
Research and development costs	(83,306)	(63,578)	(171,455)	(121,093)
Selling, general and administrative expenses	(35,345)	(20,805)	(72,591)	(38,720)
Operating profit / (loss)	**(117,629)**	**(82,947)**	**(242,279)**	**(156,152)**
Share of profit / (loss) of associate	(4,817)	(1,885)	23,289	(3,400)
Finance income	145	86	23,268	1,996
Finance expenses	(12,141)	(10,292)	(1,703)	(876)
Profit / (loss) before tax	**(134,442)**	**(95,038)**	**(197,425)**	**(158,432)**
Tax on profit / (loss) for the period	68	106	259	183
Net profit / (loss) for the period	**(134,374)**	**(94,932)**	**(197,166)**	**(158,249)**
Attributable to owners of the Company	(134,374)	(94,932)	(197,166)	(158,249)
Basic and diluted earnings / (loss) per share	€ (2.50)	€ (1.97)	€ (3.66)	€ (3.29)
Number of shares used for calculation (basic and diluted)	53,848,166	48,207,661	53,804,300	48,096,749
Net profit / (loss) for the period	**(134,374)**	**(94,932)**	**(197,166)**	**(158,249)**

Other comprehensive income / (loss)				
Items that may be reclassified subsequently to profit or loss:				
Exchange differences on translating foreign operations	77	(147)	1,765	(61)
Other comprehensive income / (loss) for the period, net of tax	**77**	**(147)**	**1,765**	**(61)**
Total comprehensive income / (loss) for the period, net of tax	**(134,297)**	**(95,079)**	**(195,401)**	**(158,310)**
Attributable to owners of the Company	(134,297)	(95,079)	(195,401)	(158,310)

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

	June 30, 2021	December 31, 2020
Assets		
Non-current assets		
Intangible assets	5,495	5,717
Property, plant and equipment	123,924	108,112
Investment in associate	45,783	9,176
Deposits	1,702	1,375
Marketable securities	90,693	115,280
	267,597	**239,660**
Current assets		
Trade receivables	394	387
Other receivables	11,398	6,957
Prepayments	21,826	13,994
Marketable securities	166,094	134,278
Cash and cash equivalents	384,539	584,517
	584,251	**740,133**
Total assets	**851,848**	**979,793**
Equity and liabilities		
Equity		
Share capital	7,237	7,217
Distributable equity	680,250	831,494
Total equity	**687,487**	**838,711**
Non-current liabilities		
Lease liabilities	94,059	85,116
Other liabilities	-	3,162
	94,059	**88,278**
Current liabilities		
Lease liabilities	6,950	6,859
Contract liabilities	145	363
Trade payables and accrued expenses	44,207	21,897
Other payables	18,623	23,384
Income taxes payable	377	301
	70,302	**52,804**
Total liabilities	**164,361**	**141,082**
Total equity and liabilities	**851,848**	**979,793**

Investor contacts:
Tim Lee
Ascendis Pharma
(650) 374-6343
tle@ascendispharma.com

Patti Bank
Westwicke Partners
(415) 513-1284
patti.bank@westwicke.com
ir@ascendispharma.com

Media contact:
Melinda Baker
Ascendis Pharma
(650) 709-8875
media@ascendispharma.com

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