

PRESS RELEASE

Significant and Sustained Improvements in Renal Function Reported for Adults with Chronic Hypoparathyroidism Treated with TransCon™ PTH (Palopegteriparatide): 2-year Results from Phase 3 PaTHway Trial

COPENHAGEN, Denmark, May 13, 2024 (GLOBE NEWSWIRE) – Ascendis Pharma A/S (Nasdaq: ASND) today announced 2-year results from a post-hoc analysis of the Company’s Phase 3 PaTHway Trial demonstrating significant and sustained improvements in renal function in adults with chronic hypoparathyroidism treated with TransCon PTH (palopegteriparatide). The data were shared in an oral presentation on May 12 by Peter Schwarz, M.D., Professor of Clinical Medicine at the University of Copenhagen, during the European Congress of Endocrinology 2024 (ECE 2024), the annual meeting of the European Society of Endocrinology.

“This unparalleled data showing sustained, clinically meaningful improvements in kidney function in these patients reinforce the potential for TransCon PTH to address concerns about soft-tissue calcifications and decreased kidney function associated with conventional therapy,” said Aimee Shu, M.D., Ascendis Pharma’s Senior Vice President of Clinical Development, Endocrine Medical Sciences.

The post-hoc analysis examined the impact of treatment with TransCon PTH on renal function using estimated glomerular filtration rate (eGFR) through Week 104 (n=76) of PaTHway, a Phase 3 double-blind, placebo-controlled trial of 82 dosed adults with chronic hypoparathyroidism randomized 3:1 (TransCon PTH:placebo; both arms initially co-administered with conventional therapy of active vitamin D and oral calcium), with a 26-week blinded period followed by an ongoing 156-week open-label extension period. Across both treatment arms, TransCon PTH treatment resulted in a mean eGFR increase of 8.9 mL/min/1.73m² (p<0.0001) from baseline at Week 52, sustained at Week 104 with a mean change from baseline of 9.0 mL/min/1.73m² (p<0.0001). Treatment was generally well-tolerated, with no new safety signals.

eGFR* Change from Baseline by Study Arm							
Study Arm	Baseline	Week 26		Week 52		Week 104	
	eGFR (mL/min/1.73m ²)	N	Mean (p value)	N	Mean (p value)	N	Mean (p value)
TransCon PTH / TransCon PTH	eGFR < 60	19	+11.4 (p=0.0002)	19	+11.5 (p=0.0003)	18	+13.4 (p<0.0001)
	eGFR ≥ 60	41	+6.3 (p=0.0002)	40	+8.2 (p<0.0001)	40	+6.9 (p<0.0001)
	All	60	+7.9 (p<0.0001)	59	+9.3 (p<0.0001)	58	+8.9 (p<0.0001)
Placebo (first 26 weeks) / TransCon PTH**	eGFR < 60	4	+0.05 (p=0.9877)	4	+11.7 (p=0.0018)	4	+15.6 (p=0.0067)
	eGFR ≥ 60	15	-2.4 (p=0.3280)	15	+6.5 (p=0.0199)	14	+7.6 (p=0.0121)
	All	19	-1.9 (p=0.3468)	19	+7.6 (p=0.0014)	18	+9.4 (p=0.0006)

*eGFR (an assessment of kidney filtering capacity) was calculated by the trial's central lab using the Modification of Diet in Renal Disease Study Group (MDRD) equation (Levey, Ann Intern Med 2006). An eGFR level <60 mL/min/1.73m² is considered the threshold for impaired kidney function.

**Patients in the placebo arm switched to TransCon PTH following the Week 26 visit.

TransCon PTH treatment was associated with clinically meaningful increases (≥ 5 mL/min/1.73 m²) in eGFR within 26 weeks that were sustained through Week 104 of PaTHway:

Proportion of Participants (%) with ≥ 5 and ≥ 10 mL/min/1.73 m ² Increases in eGFR from Baseline through Week 104*						
eGFR Change from Baseline	All Participants					
	TransCon PTH / TransCon PTH (n=61)			Placebo (first 26 weeks) / TransCon PTH** (n=21)		
	Week 26	Week 52	Week 104	Week 26	Week 52	Week 104
	PTH	PTH	PTH	Placebo	Switch to PTH	Switch to PTH
≥ 5 mL/min/1.73 m ²	57%	64%	61%	24%	52%	62%
≥ 10 mL/min/1.73 m ²	43%	43%	46%	10%	39%	38%

eGFR Change from Baseline	Participants with Baseline eGFR < 60 mL/min/1.73 m ²					
	TransCon PTH / TransCon PTH (n=19)			Placebo (first 26 weeks) / TransCon PTH** (n=4)		
	Week 26	Week 52	Week 104	Week 26	Week 52	Week 104
	PTH	PTH	PTH	Placebo	Switch to PTH	Switch to PTH
≥ 5 mL/min/1.73 m ²	74%	68%	74%	25%	100%	100%
≥ 10 mL/min/1.73 m ²	47%	42%	53%	0%	75%	75%

*Percentages were calculated based on all participants. Patients who did not have an eGFR assessment at the visit were still included in the denominator.

**Patients in the placebo arm switched to TransCon PTH following the Week 26 visit.

Highlights from this ECE oral presentation will be made available on the Investor & News section of the Ascendis Pharma website at <https://investors.ascendispharma.com>.

About Hypoparathyroidism

Hypoparathyroidism is an endocrine disease caused by insufficient levels of PTH, the primary regulator of calcium and phosphate balance in the body, acting directly on bone and kidneys and indirectly on the intestines. Individuals with hypoparathyroidism may experience a range of severe and potentially life-threatening short-term and long-term complications, including neuromuscular irritability, renal complications, extra-skeletal calcifications, and cognitive impairment. Post-surgical hypoparathyroidism accounts for the majority of cases (70-80%), while other etiologies include autoimmune and idiopathic causes.

About TransCon PTH

TransCon PTH (paleopegteriparatide) is a prodrug of parathyroid hormone (PTH 1-34) administered once daily, designed to provide parathyroid hormone levels within the normal physiological range across the 24-hour dosing period. TransCon PTH was granted marketing authorization under the brand name YORVIPATH[®] by the European Commission (EC) and the European Economic Area (EEA)

in November 2023 and the United Kingdom's Medicines & Healthcare Products Regulatory Agency (MHRA) in Great Britain as a PTH replacement therapy indicated for the treatment of adults with chronic hypoparathyroidism. In the United States, the Food & Drug Administration (FDA) has set a PDUFA target action date of May 14, 2024, to complete their review of Ascendis Pharma's New Drug Application for TransCon PTH for adults with chronic hypoparathyroidism. TransCon PTH is also in development in Japan through Teijin Ltd. and in China through VISEN Pharmaceuticals.

About Ascendis Pharma A/S

Ascendis Pharma is applying its innovative TransCon technology platform 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, Ascendis uses its TransCon technologies to create new and potentially best-in-class therapies. Ascendis is headquartered in Copenhagen, Denmark and has additional facilities in Europe and the United States. Please visit ascendispharma.com to learn more.

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) the potential for TransCon PTH to address concerns about soft-tissue calcifications and decreased kidney function associated with conventional therapy, (ii) TransCon PTH's PDUFA date of May 14, 2024, (iii) Ascendis' ability to apply its TransCon technology platform to build a leading, fully integrated biopharma company and (iv) Ascendis' use of 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, distributors and service providers for Ascendis' products and product candidates; unforeseen safety or efficacy results in Ascendis' development programs or on-market products; unforeseen expenses related to commercialization of any approved Ascendis products; unforeseen expenses related to Ascendis' development programs; unforeseen selling, general and administrative expenses, other research and development expenses and Ascendis' business generally; delays in the development of its programs related to manufacturing, regulatory requirements, speed of patient recruitment or other unforeseen delays; Ascendis' ability to obtain additional funding, if needed, to support its business activities; the impact of international economic, political, legal, compliance, social and business factors. 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 February 7, 2024 and Ascendis' other future reports filed with, or submitted to, the SEC. Forward-looking statements do not reflect the potential impact of any future 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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