PRESS RELEASE

New Post Hoc Analysis Showed Substantial Improvement in Observed Estimated Glomerular Filtration Rate (eGFR), in TransCon PTH-Treated Adults with Hypoparathyroidism

– *Approximately 50% of Phase 3 PaTHway Trial patients with eGFR < 60 mL/min/1.73m², the threshold for kidney dysfunction, receiving TransCon PTH experienced eGFR improvement to above 60 mL/min/1.73m².*

COPENHAGEN, Denmark, September 5, 2023 (GLOBE NEWSWIRE) – Ascendis Pharma A/S (Nasdaq: ASND) today announced new post hoc analysis showing adults with hypoparathyroidism treated with TransCon PTH demonstrated substantial improvement in estimated glomerular filtration rate (eGFR), suggesting improved kidney function. TransCon PTH (palopegteriparatide) is an investigational prodrug of parathyroid hormone (PTH 1-34) administered once daily designed to provide sustained release of active PTH within the physiological range for 24 hours per day in adult patients with hypoparathyroidism.

"Chronic kidney disease is one of the most consequential complications of chronic hypoparathyroidism and its treatment with conventional therapy. Diminished kidney function is associated with its own set of complications, including hypertension, edema, and weakness," said Dr. Aliya Khan, M.D., Clinical Professor of Medicine at McMaster University and Director of the Calcium Disorders Clinic at McMaster University Medical Center. "The observations in the post hoc analysis of the PaTHway Trial, suggesting that treatment with TransCon PTH reverses impaired kidney function in patients with hypoparathyroidism, further substantiating the promise for a major improvement in treatment opportunities and outcomes for people living with hypoparathyroidism."

In the Phase 3 PaTHway Trial, mean baseline eGFR was 67.3 and 72.7 mL/min/1.73m^2 for subjects randomized to TransCon PTH and placebo, respectively. At Week 26, patients treated with TransCon PTH experienced a mean increase in eGFR of 7.9 mL/min/1.73m^2 compared to baseline ($p<0.0001$) while those on placebo experienced a mean decrease in eGFR of -1.9 mL/min/1.73m^2 compared to baseline ($p=0.3468$). By Week 52, patients treated with TransCon PTH, including those crossing over from placebo, experienced a mean increase in eGFR of 8.9 mL/min/1.73m^2 compared to baseline ($p<0.0001$). The improvement at Week 52 was even greater, with patients with eGFR <60 at baseline, the threshold for kidney dysfunction, experiencing a mean increase in eGFR of 11.5 mL/min/1.73m^2.

PaTHway: eGFR Change from Baseline by eGFR Group

Study Arm	Baseline eGFR (mL/min/1.73m^2)	Week 26		Week 52	
		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)
	eGFR ≥ 60	41	+6.3 (p=0.0002)	40	+8.2 (p <0.0001)
	All	60	+7.9 (p< 0.0001)	59	+9.3 (p<0.0001)
Placebo (first 26 weeks) / TransCon PTH*	eGFR < 60	4	+0.05 (p=0.9877)	4	+11.7 (p=0.0018)
	eGFR ≥ 60	15	-2.4 (p=0.3280)	15	+6.5 (p=0.0199)
	All	19	-1.9 (p=0.3468)	19	+7.6 (p=0.0014)

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

Among subjects with baseline eGFR < 60 mL/min/m^2 (considered the threshold for impaired kidney function), approximately 50% were able to improve their eGFR to > 60 mL/min with TransCon PTH therapy.

	eGFR < 60 at Baseline (n)	Number of Responders* (n, %) Week 26	Number of Responders* (n, %) Week 52
TransCon PTH / TransCon PTH	n=19	n=12 63%	n=10 53%
Placebo (first 26 weeks) / TransCon PTH**	n=4	n=0 0%	n=3 75%
Total PaTHway Trial	n=23	n=12 52%	n=13 57%

eGFR based on central lab data using the MDRD Study Group formula.
** Responders defined as moving from eGFR < 60 to eGFR ≥ 60. Units in (mL/min/1.73m2).*
*** Patients in the placebo arm switched to TransCon PTH following the Week 26 visit.*

"We will continue to study this important data and topic, which suggests the potential of TransCon PTH to address physician and patient concerns about soft-tissue calcifications and decreased kidney function associated with conventional therapy," said Aimee Shu, M.D., Vice President, Clinical Development, Endocrine & Rare Diseases at Ascendis Pharma. "In addition to reducing risk for patients, this could also help significantly reduce the healthcare burden associated with hypoparathyroidism. We look forward to presenting detailed results at an upcoming medical conference."

About Ascendis Pharma A/S

Ascendis Pharma is applying its innovative platform technology to build a leading, fully integrated, global 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 Germany (Heidelberg, Berlin and Munich) and the United States (Palo Alto and Redwood City, California, and Princeton, New Jersey). 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) TransCon PTH's ability to sustained release of active PTH within the physiological range for 24 hours per day in adult patients with hypoparathyroidism; (ii) the potential of TransCon PTH's ability to reverse impaired kidney function in patients with hypoparathyroidism; (iii) the potential of TransCon PTH to address physician and patient concerns about soft-tissue calcifications and decreased kidney function associated with conventional therapy, (iv) TransCon PTH's ability to reduce risk for the patients and help reduce the healthcare burden associated with hypoparathyroidism, (v) Ascendis' ability to apply its TransCon technology platform to build a leading, fully integrated, global 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 its 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, including inflation, the effects on its business from the worldwide COVID-19 pandemic and ongoing conflicts such as that in the region surrounding Ukraine and Russia. 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 16, 2023 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.

Investor Contacts:
Tim Lee
Ascendis Pharma
+1 (650) 374-6343
tle@ascendispharma.com
ir@ascendispharma.com

Patti Bank
ICR Westwicke
+1 (415) 513-1284
patti.bank@westwicke.com

Media Contact:
Melinda Baker
Ascendis Pharma
+1 (650) 709-8875
media@ascendispharma.com