



Ascendis Pharma A/S Announces Mean Bone Mineral Density (BMD) Data from Phase 2 PaTH Forward Trial Demonstrating Continued Normalization and Stabilization of BMD Z-scores Between 26 and 58 Weeks

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– Data from subjects with available dual energy x-ray absorptiometry (DXA) scans demonstrated stabilization of BMD, in alignment with observed bone turnover markers previously reported –

COPENHAGEN, Denmark, Sept. 22, 2021 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND), a biopharmaceutical company using its innovative TransCon™ technologies to potentially create new treatments to make a meaningful difference in patients' lives, today announced 58-week BMD data from central lab reading in the PaTH Forward Trial, a global phase 2 trial of its investigational product candidate TransCon PTH in adult subjects with hypoparathyroidism (HP).

"HP patients, compared to people with normal parathyroid function, have insufficient parathyroid hormone (PTH) levels and low rates of PTH-driven skeletal remodeling, resulting in above-average bone mineral density and potentially an increased risk of fractures and other negative health effects," said Aimee Shu, M.D., Ascendis Pharma's Vice President of Clinical Development, Endocrine Medical Sciences. "We designed TransCon PTH as a once daily injection to restore physiologic levels of PTH, 24-hours a day. In PaTH Forward, mean BMD Z-scores, which parallel serum markers of bone turnover, trended towards stabilization and continued normalization at 58 weeks. We believe this is an indicator that TransCon PTH has the potential to be able to normalize calcium metabolism in the body over time."

About the Week 58 Analysis

BMD was measured with non-invasive DXA, a low-radiation exposure technology widely used to identify individuals with bone fracture risk. Results were read and reported by a central lab.

Mean Bone Mineral Density Z-scores by DXA*				
Anatomic region	n	Baseline	Week 26	Week 58
Lumbar spine (L1-L4)	42	1.6	1.0	0.9
Femoral neck	43	1.0	0.5	0.5
Total hip	43	1.0	0.6	0.5
Forearm/ 1/3 radius	41	0.3	0.3	0.3

*From central lab reading

About TransCon™ PTH¹

TransCon PTH is an investigational once-daily long-acting prodrug of parathyroid hormone (PTH[1-34]) in development as a treatment for adult hypoparathyroidism (HP), designed to restore PTH at physiologic levels for 24 hours each day to address both the short-term symptoms and long-term complications of the disease. TransCon PTH has been granted Orphan drug status in the United States, European Union, and Japan.

About Hypoparathyroidism (HP)^{2,3,4,5,6,7}

HP is a rare endocrine disorder characterized by insufficient levels of parathyroid hormone (PTH) which plays a critical role in controlling systemic calcium, phosphate, and calcitriol (active vitamin D) levels and is essential to many key biological functions. HP affects approximately 400,000 patients in the United States, Europe, Japan, South Korea and Greater China, the majority of whom develop the condition following damage or accidental removal of the parathyroid glands during thyroid surgery. Patients often experience decreased quality of life. In the short term, symptoms include weakness, severe muscle cramps (tetany), abnormal sensations such as tingling, burning and numbness (paresthesia), memory loss, impaired judgment and headache. Over the long term, this complex disorder can increase risk of major complications, such as extraskeletal calcium depositions occurring within the brain, lens of the eye, and kidneys, which can lead to impaired renal function.

HP remains among the few hormonal insufficiency states without an approved replacement therapy that restores the missing hormone at physiologic levels. Standard of care with active vitamin D analogs and calcium supplementation does not fully control the disease and may contribute to risk of renal disease. As a result, patients with HP have an estimated 4-fold to 8-fold greater risk of renal disease compared to healthy controls.

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 uses its TransCon technologies to create new and potentially best-in-class therapies.

Ascendis is headquartered in Copenhagen, Denmark, and has additional facilities in Heidelberg and Berlin, Germany; Palo Alto and Redwood City, California; and Princeton, New Jersey. Please visit www.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) Ascendis' design of TransCon PTH as a once daily injection to restore physiologic levels of PTH, 24-hours a day; (ii) Ascendis' belief that BMD data observed indicate that TransCon PTH has the potential to be able to normalize calcium metabolism in the body over time; (iii) Ascendis' ability to apply its platform technology to build a leading, fully integrated biopharma company, (iv) Ascendis' product pipeline and expansion into additional therapeutic areas and (v) 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 TransCon hGH, the SKYTROFA[®] Auto-Injector and other study drug for commercial sales in the U.S. and clinical studies; unforeseen safety or efficacy results in its oncology programs, TransCon hGH, TransCon PTH and TransCon CNP or other development programs; unforeseen expenses related to commercialization of lonapegsomatropin-tcgd in the U.S. and the further development of TransCon hGH, 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 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.

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Footnotes:

- ¹ Karpf DB, et al. *J Bone Miner Res*. 2020; x:1-11.
- ² Mannstadt M, et al. *Nature Reviews* 2017, 3: 17055
- ³ Ascendis Pharma HP Patient Experience Research.
- ⁴ Hadker N, et al. *Endo Pract*. 2014, 20(7):671-679.
- ⁵ Powers J, et al. *J Bone Miner Res* 2013, 28: 2570-2576.
- ⁶ Mitchell DM, et al. *J Clin Endocrinol Metab* 2012, 97(12): 4507-4514
- ⁷ Underbjerg L, et al. *J Bone Miner Res* 2013, 28: 2277-2285



Source: Ascendis Pharma