Ascendis Pharma A/S Announces Presentations for TransCon™ PTH and TransCon™ CNP at the American Society for Bone & Mineral Research 2021 Annual Meeting

September 30, 2021 at 4:01 PM EDT

– Late-breaking oral presentation will feature a comprehensive review of 58-week results from the company's Phase 2 PaTH Forward Trial –

COPENHAGEN, Denmark, Sept. 30, 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 presentations featuring new data about its investigational TransCon PTH product candidate at the American Society for Bone & Mineral Research (ASBMR) 2021 Annual Meeting, being held October 1-4 virtually and in-person in San Diego. Presentations will also include an overview of baseline demographics from the Ascendis Pharma-sponsored natural history study of children with achondroplasia.

"We are excited to partner with our investigators to share the latest data showing continued advances in our hypoparathyroidism and achondroplasia development programs," said Aimee Shu, M.D., Ascendis Pharma's Vice President of Clinical Development, Endocrine Medical Sciences. "In particular, we are excited to have a late-breaking oral presentation by Dr. Aliya Khan, a recognized expert in parathyroid disease, who will present results from what we believe to be the most extensive data set generated to date (58 subjects) of continuous long-term delivery of parathyroid hormone (PTH) at physiologic levels in patients with hypoparathyroidism."

"Over 58 weeks in PaTH Forward, the data from study subjects show continued trends toward normal calcium homeostasis in the absence of standard of care therapies," said Aliya Khan, M.D., Clinical Professor of Medicine and Director of the Calcium Disorders Clinic at St. Joseph's Healthcare, McMaster University. "This was reflected by normalization of key biomarkers and bone mineral density. Equally important, patients reported a marked reduction in their symptom burden and improvements in their health-related quality of life. The majority of adverse events were mild and unrelated to study drug, and no adverse events led to discontinuation of the study drug or dropouts from the trial. These are very encouraging signs that physiological parathyroid hormone replacement is well tolerated and could help address the unmet needs these patients face."

Oral Presentation	Date/Time
Sustained Benefit of TransCon PTH, an Investigational Hormone Replacement Therapy for Adult Patients with Hypoparathyroidism, at Week 58 in the PaTH Forward Trial Oral presentation by Aliya Khan, M.D.	Late-Breaking Oral Presentations: Clinical Treatment *#LB-1114* Monday, October 4 11:30am-12:45pm PST

Poster Presentations	Date/Time
Baseline Demographics of the ACHieve Study: A Five-Year, Multi-National Observational Cohort Study of Children with Achondroplasia Presenting author: Leanne M. Ward, M.D., FRCPC	Virtual Plenary Poster Presentation Friday, October 1 & Saturday, October 2 *Poster #A21023623*
A Single Dose of TransCon PTH in Subjects with Impaired Renal Function: A Phase 1 Trial Presenting author: Aimee Shu, M.D.	Poster Session I Saturday, October 2 1:00- 3:00pm PST *Poster #SAT-272*

Ascendis Pharma is also proud to sponsor two continuing medical education (CME) programs at this year's ASBMR Annual Meeting:

Ascendis-Sponsored CME Programs	**Date/Time**
Hypoparathyroidism: Changing the Treatment Landscape Bart Clarke & Lars Rejnmark	Friday, October 1 6:30am-7:45am PST *Virtual presentation with live Q&A*
Achondroplasia: Beyond Short Stature Janet Legare & Michael Bober	Saturday, October 2 6:30am-7:45am PST *Virtual presentation with live Q&A*

About Hypoparathyroidism[1-6]
Hypoparathyroidism (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 Achondroplasia

Achondroplasia is the most common form of dwarfism, affecting approximately 250,000 people worldwide. Individuals living with achondroplasia may experience severe skeletal complications and comorbidities. For example, abnormal development of the vertebra can lead to sleep apnea, chronic back and leg pain from lower spine impingement and sudden infant death from cervical compression. Chronic ear infections due to abnormal eustachian tubes can lead to hearing loss and speech delay.[7]

The condition is caused by an autosomal dominant activating mutation in the fibroblast growth factor receptor 3 (FGFR3) gene that leads to an imbalance in the effects of the FGFR3 and CNP signaling pathways. Preclinical and clinical data show that the CNP pathway stimulates growth. Increased CNP counteracts the effects of the FGFR3 mutation downstream, thus promoting bone growth.[8]

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) whether physiological parathyroid hormone replacement could help address the unmet needs these patients face, (ii) Ascendis' ability to apply its platform technology to build a leading, fully integrated biopharma company, (iii) Ascendis' product pipeline and expansion into additional therapeutic areas and (iv) 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.

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

End Notes:

[1] Mannstadt M, et al. *Nature Reviews* 2017, 3: 17055
[2] Ascendis Pharma HP Patient Experience Research.
[3] Hadker N, et al. *Endo Pract.* 2014, 20(7);671-679.
[4] Powers J, et al. *J Bone Miner Res* 2013, 28: 2570-2576.
[5] Mitchell DM, et al. *J Clin Endocrinol Metab* 2012, 97(12): 4507-4514
[6] Underbjerg L, et al. *J Bone Miner Res* 2013, 28: 2277-2285
[7] Ireland PJ, Pacey V, et al. *Appl Clin Genet*. 2014;7: 117–25.
[8] Horton WA, et al. *Lancet*. 2007;370(9582):162–172.

Source: Ascendis Pharma