NEWS RELEASE

# Ascendis Pharma Receives Orphan Drug Exclusivity in the U.S. for YORVIPATH® (Palopegteriparatide) for the Treatment of Hypoparathyroidism in Adults

2024-09-11

– U.S. FDA Orphan Drug exclusivity provides seven years of market exclusivity for YORVIPATH in the United States for the treatment of hypoparathyroidism in adults

COPENHAGEN, Denmark, Sept. 11, 2024 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND) today announced that the United States Food & Drug Administration (FDA) has granted Orphan Drug exclusivity to YORVIPATH® (palopegteriparatide, developed as TransCon PTH), providing seven years of market exclusivity for YORVIPATH in the United States for the treatment of hypoparathyroidism in adults. YORVIPATH is a prodrug of parathyroid hormone (PTH [1-34]), administered once daily, designed to provide continuous exposure to released PTH over the 24-hour dosing period. Hypoparathyroidism is a rare endocrine disease caused by insufficient levels of parathyroid hormone that impact multiple organs and affects an estimated 70,000 to 90,000 people in the United States.

“YORVIPATH has now been granted orphan exclusivity in the U.S., European Union, and other countries, reflecting the uniqueness of YORVIPATH to address this rare disease,” said Jan Mikkelsen, Ascendis Pharma’s President and Chief Executive Officer. “As the first and only FDA-approved treatment of hypoparathyroidism in adults, we believe that YORVIPATH has great potential to address the underlying disease and look forward to making it available to patients in the U.S. as quickly as possible.”

TransCon PTH (palopegteriparatide) originally received Orphan Drug Designation from the U.S. FDA in June 2018. The FDA grants orphan designation to drugs that are intended for the safe and effective treatment, diagnosis, or prevention of rare diseases or disorders that affect fewer than 200,000 people in the United States, and that

potentially may be safer or more effective than already approved products. Subject to certain exceptions, orphan designation provides a drug developer with certain benefits and incentives, including a seven-year period of U.S. marketing exclusivity upon approval of the product in the orphan-designated indication, waiver of FDA user fees, and tax credits for clinical research. The granting of orphan designation does not alter the FDA's regulatory requirements to establish safety and effectiveness of a drug through adequate and well-controlled studies to support approval and commercialization, nor does it provide any advantage during the regulatory review and approval processes.

About Hypoparathyroidism

Hypoparathyroidism is an endocrine disease caused by insufficient levels of parathyroid hormone (PTH), the primary regulator of calcium and phosphate balance in the body, acting directly on bone and kidneys and indirectly on the intestine. 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 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) YORVIPATH's potential to address the underlying disease of hypoparathyroidism, (ii) Ascendis' intent to make YORVIPATH available to patients in the U.S. as quickly as possible, (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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