

PRESS RELEASE

FDA Approves SKYTROFA[®] (Lonapegsomatropin-tcgd) for the Once-Weekly Treatment of Adults with Growth Hormone Deficiency

- *First of many planned label expansions supporting Vision 2030 goal to become the leading endocrinology rare disease company*
- *On track to initiate basket trial for ISS, SHOX deficiency, Turner syndrome, and SGA*, as well as combination therapy trials in achondroplasia and hypochondroplasia, in Q4 2025*

COPENHAGEN, Denmark, July 28, 2025 (GLOBE NEWSWIRE) – Ascendis Pharma A/S (Nasdaq: ASND) today announced that the U.S. Food & Drug Administration (FDA) has approved SKYTROFA[®] (lonapegsomatropin-tcgd; developed as TransCon hGH) for the replacement of endogenous growth hormone in adults with growth hormone deficiency (GHD), a rare disorder resulting from decreased or total loss of growth hormone production. Lonapegsomatropin (approved by the FDA in 2021 for the treatment of pediatric GHD) is a prodrug of somatropin (human growth hormone, or hGH) administered once weekly, providing sustained release of active, unmodified somatropin.

The FDA’s approval of SKYTROFA for adult GHD was based on results from foresiGHt, a Phase 3 randomized, parallel-arm, placebo-controlled (double-blind) and active-controlled (open-label) clinical trial that compared the efficacy and safety of weekly TransCon hGH with weekly placebo and daily somatropin in adults with GHD.

“The abnormal body composition, dyslipidemia, and insulin resistance that are the hallmarks of adult GHD predispose affected individuals to serious medical complications such as metabolic syndrome, increased cardiovascular risk, and impaired quality of life – including cognitive dysfunction, depression, anxiety, sleep disturbance, and reduced physical and mental drive. Despite this, adherence to daily somatropin injections that have been the standard of care for more than 25 years remains a problem,” said Kevin Yuen, M.D., Barrow Neurological Institute, University of Arizona College of Medicine and Creighton University School of Medicine in Phoenix, Arizona. “It is no surprise that patients are looking for a less burdensome treatment regimen, and a new treatment option such as SKYTROFA, with once-weekly injection and its unique release of unmodified somatropin, is expected to help improve both real-world adherence and overall outcomes.”

“Our market research shows SKYTROFA is the treatment of choice for pediatric GHD among patients and physicians, and we are pleased to expand its availability in the United States for the treatment of adults initiating therapy or switching from another growth hormone therapy,” said Jan Mikkelsen, President and Chief Executive Officer at Ascendis Pharma. “This important milestone is the first of many planned label expansions supporting our goal to become the leading endocrinology rare disease company.”

The following information is intended for the U.S. Audience Only:

IMPORTANT SAFETY INFORMATION & USES

SKYTROFA is a prescription medicine used for:

- the replacement of growth hormone in children 1 year old or older who weigh at least 26 pounds (11.5 kilograms) with growth hormone deficiency (GHD)
- the replacement of growth hormone in adults with growth hormone deficiency (GHD)

Do not take SKYTROFA if:

- you have a critical illness caused by certain types of heart or stomach surgery, trauma or breathing problems
- you are allergic to somatropin or any of the ingredients in SKYTROFA
- you are a child with closed bone growth plates
- you have cancer or other tumors
- you have certain types of eye problems caused by diabetes
- you are a child with Prader-Willi syndrome who is severely obese or has breathing problems including sleep apnea (briefly stopping breathing during sleep)

Tell your healthcare provider if you are pregnant or plan to become pregnant, about all of your medical conditions, and about all the medicines you take. SKYTROFA may affect how other medicines work, and other medicines may affect how SKYTROFA works.

What are the possible side effects of SKYTROFA?

SKYTROFA may cause serious side effects, including:

- serious allergic reactions. Get medical help right away if you have swelling of the face, mouth, tongue, or throat or trouble breathing
- high risk of death in people who have critical illnesses caused by heart or stomach surgery, trauma, or serious breathing problems
- increased risk of growth of an existing tumor or cancer, or increased risk of a previous tumor or cancer returning in people who were treated with radiation to the brain or head as children and who developed low growth hormone problems
- Your healthcare provider will need to monitor for the growth or return of a tumor or cancer. Contact your healthcare provider if you start to have sudden changes in behavior, headaches, vision problems, or changes in moles, birthmarks, or skin color
- new or worsening high blood sugar or diabetes. Your blood sugar may need to be monitored during treatment with SKYTROFA
- increase in pressure in the skull. Contact your healthcare provider if headaches, eye problems, nausea, or vomiting occur

- too much fluid in the body (fluid retention). Contact your healthcare provider if you notice swelling in the hands and feet, pain in the joints or muscles, or nerve problems that cause pain, burning or tingling in the hands, arms, legs, and feet
- decrease in a hormone called cortisol. Your healthcare provider will do blood tests to check your cortisol levels
- decrease in thyroid hormone levels. Decreased thyroid hormone levels may affect how well SKYTROFA works. Your healthcare provider will do blood tests to check your thyroid hormone levels
- hip and knee pain or a limp in children (slipped capital femoral epiphysis). This may lead to a serious condition where bone tissue dies due to a lack of blood supply (osteonecrosis). Get medical help right away for children who develop a limp or have hip or knee pain
- worsening of curvature of the spine in children (scoliosis)
- severe and constant abdominal pain. This could be a sign of pancreatitis. Tell your healthcare provider about any new abdominal pain
- loss of fat and tissue weakness in the area of skin you or your child inject. Talk to your healthcare provider about rotating the areas where SKYTROFA is injected
- high risk of sudden death in children with Prader-Willi syndrome who are severely obese or have breathing problems, including sleep apnea
- increase in phosphate, alkaline phosphatase, and parathyroid hormone levels in your blood. Your healthcare provider will do blood tests to check this

The most common side effects of SKYTROFA in children include: viral infection, fever, cough, nausea and vomiting, bleeding, diarrhea, stomach area pain, joint pain and arthritis

The most common side effects of SKYTROFA in adults include: swelling due to fluid build-up and low thyroid hormone

These are not all of the possible side effects of SKYTROFA. Call your doctor for medical advice about side effects. **You are encouraged to report side effects to FDA at 1-800-FDA-1088 or at www.fda.gov/medwatch.** You may also report side effects to Ascendis Pharma at 1-844-442-7236.

Please click [here](#) for SKYTROFA full Prescribing Information.

About Ascendis Pharma A/S

Ascendis Pharma is a global biopharmaceutical company focused on applying our innovative TransCon technology platform to make a meaningful difference for patients. Guided by our core values of Patients, Science, and Passion, and following our algorithm for product innovation, we apply TransCon to develop new therapies that demonstrate best-in-class potential to address unmet medical needs. 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) Ascendis' plans to initiate basket trial for ISS, SHOX deficiency, Turner syndrome, and SGA, as well as combination therapy trials in achondroplasia and hypochondroplasia, in Q4 2025, (ii) SKYTROFA's ability to improve real-world adherence and overall outcomes, (iii) Ascendis' planned label expansions for SKYTROFA, (iv) Ascendis' goal of becoming the leading endocrinology rare disease company, (v) Ascendis' ability to apply its TransCon technology platform to make a meaningful difference for patients, and (vi) Ascendis' application of its TransCon technologies to develop new therapies that demonstrate best-in-class potential to address unmet medical needs. 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, including tariffs and trade policies. 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 12, 2025, 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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* *ISS = idiopathic short stature*

SHOX deficiency = short stature homeobox-containing gene deficiency

SGA = small for gestational age