



Ascendis Pharma A/S Announces U.S. Commercial Launch of SKYTROFA® (Lonapegsomatropin-tcgd), the First and Only FDA Approved Once-Weekly Treatment for Pediatric Growth Hormone Deficiency

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COPENHAGEN, Denmark, Oct. 15, 2021 (GLOBE NEWSWIRE) -- Ascendis Pharma A/S (Nasdaq: ASND), today announced the U.S. commercial launch of SKYTROFA (lonapegsomatropin-tcgd), its once-weekly treatment for the treatment of pediatric patients one year and older who weigh at least 11.5 kg (25.4 lb) and have growth failure due to inadequate secretion of endogenous growth hormone (GH). SKYTROFA (lonapegsomatropin-tcgd) is available by prescription and distributed through a network of specialty pharmacies across the United States.

"SKYTROFA offers patients, caregivers, and physicians the potential to replace daily somatropin injections that have been the standard of care for more than 30 years," said Jan Mikkelsen, Ascendis Pharma's President and Chief Executive Officer. "As the first and only FDA-approved once-weekly therapy for pediatric growth hormone deficiency, SKYTROFA represents one of the most important innovations for these patients in decades."

Reflecting its commitment to patients, Ascendis Pharma has also launched the Ascendis Signature Access Program™, a personalized patient support program in the U.S. dedicated to working with families, caregivers, and physicians from decision to treat through long-term therapy adherence. The program is staffed by nurses and offers a full suite of services including, but not limited to, prior authorization support, out of pocket assistance, and training on proper injection procedures.

The full commercial launch of SKYTROFA (lonapegsomatropin-tcgd) marks an important milestone as the Company continues to deliver on its Vision 3x3 strategic roadmap through 2025 to build a leading global biopharma company by achieving sustainable growth through multiple approaches.

The Following Information is Intended for the U.S. Audience Only

INDICATION

SKYTROFA® is a human growth hormone indicated for the treatment of pediatric patients 1 year and older who weigh at least 11.5 kg and have growth failure due to inadequate secretion of endogenous growth hormone (GH).

IMPORTANT SAFETY INFORMATION

- SKYTROFA is contraindicated in patients with:
 - Acute critical illness after open heart surgery, abdominal surgery or multiple accidental trauma, or if you have acute respiratory failure due to the risk of increased mortality with use of pharmacologic doses of somatropin.
 - Hypersensitivity to somatropin or any of the excipients in SKYTROFA. Systemic hypersensitivity reactions have been reported with post-marketing use of somatropin products.
 - Closed epiphyses for growth promotion.
 - Active malignancy.
 - Active proliferative or severe non-proliferative diabetic retinopathy.
 - Prader-Willi syndrome who are severely obese, have a history of upper airway obstruction or sleep apnea or have severe respiratory impairment due to the risk of sudden death.
- Increased mortality in patients with acute critical illness due to complications following open heart surgery, abdominal surgery or multiple accidental trauma, or those with acute respiratory failure has been reported after treatment with pharmacologic doses of somatropin. Safety of continuing SKYTROFA treatment in patients receiving replacement doses for the approved indication who concurrently develop these illnesses has not been established.
- Serious systemic hypersensitivity reactions including anaphylactic reactions and angioedema have been reported with post-marketing use of somatropin products. Do not use SKYTROFA in patients with known hypersensitivity to somatropin or any of the excipients in SKYTROFA.
- There is an increased risk of malignancy progression with somatropin treatment in patients with active malignancy. Preexisting malignancy should be inactive with treatment completed prior to starting SKYTROFA. Discontinue SKYTROFA if there is evidence of recurrent activity.

- In childhood cancer survivors who were treated with radiation to the brain/head for their first neoplasm and who developed subsequent growth hormone deficiency (GHD) and were treated with somatropin, an increased risk of a second neoplasm has been reported. Intracranial tumors, in particular meningiomas, were the most common of these second neoplasms. Monitor all patients with a history of GHD secondary to an intracranial neoplasm routinely while on somatropin therapy for progression or recurrence of the tumor.
- Because children with certain rare genetic causes of short stature have an increased risk of developing malignancies, practitioners should thoroughly consider the risks and benefits of starting somatropin in these patients. If treatment with somatropin is initiated, carefully monitor these patients for development of neoplasms. Monitor patients on somatropin therapy carefully for increased growth, or potential malignant changes of preexisting nevi. Advise patients/caregivers to report marked changes in behavior, onset of headaches, vision disturbances and/or changes in skin pigmentation or changes in the appearance of preexisting nevi.
- Treatment with somatropin may decrease insulin sensitivity, particularly at higher doses. New onset type 2 diabetes mellitus has been reported in patients taking somatropin. Undiagnosed impaired glucose tolerance and overt diabetes mellitus may be unmasked. Monitor glucose levels periodically in all patients receiving SKYTROFA. Adjust the doses of antihyperglycemic drugs as needed when SKYTROFA is initiated in patients.
- Intracranial hypertension (IH) with papilledema, visual changes, headache, nausea, and/or vomiting has been reported in a small number of patients treated with somatropin. Symptoms usually occurred within the first 8 weeks after the initiation of somatropin and resolved rapidly after cessation or reduction in dose in all reported cases. Fundoscopic exam should be performed before initiation of therapy and periodically thereafter. If somatropin-induced IH is diagnosed, restart treatment with SKYTROFA at a lower dose after IH-associated signs and symptoms have resolved.
- Fluid retention during somatropin therapy may occur and is usually transient and dose dependent.
- Patients receiving somatropin therapy who have or are at risk for pituitary hormone deficiency(s) may be at risk for reduced serum cortisol levels and/or unmasking of central (secondary) hypoadrenalism. Patients treated with glucocorticoid replacement for previously diagnosed hypoadrenalism may require an increase in their maintenance or stress doses following initiation of SKYTROFA therapy. Monitor patients for reduced serum cortisol levels and/or need for glucocorticoid dose increases in those with known hypoadrenalism.
- Undiagnosed or untreated hypothyroidism may prevent response to SKYTROFA. In patients with GHD, central (secondary) hypothyroidism may first become evident or worsen during SKYTROFA treatment. Perform thyroid function tests periodically and consider thyroid hormone replacement.
- Slipped capital femoral epiphysis may occur more frequently in patients undergoing rapid growth. Evaluate pediatric patients with the onset of a limp or complaints of persistent hip or knee pain.
- Somatropin increases the growth rate and progression of existing scoliosis can occur in patients who experience rapid growth. Somatropin has not been shown to increase the occurrence of scoliosis. Monitor patients with a history of scoliosis for disease progression.
- Cases of pancreatitis have been reported in pediatric patients receiving somatropin. The risk may be greater in pediatric patients compared with adults. Consider pancreatitis in patients who develop persistent severe abdominal pain.
- When SKYTROFA is administered subcutaneously at the same site over a long period of time, lipoatrophy may result. Rotate injection sites when administering SKYTROFA to reduce this risk.
- There have been reports of fatalities after initiating therapy with somatropin in pediatric patients with Prader-Willi syndrome who had one or more of the following risk factors: severe obesity, history of upper airway obstruction or sleep apnea, or unidentified respiratory infection. Male patients with one or more of these factors may be at greater risk than females. SKYTROFA is not indicated for the treatment of pediatric patients who have growth failure due to genetically confirmed Prader-Willi syndrome.
- Serum levels of inorganic phosphorus, alkaline phosphatase, and parathyroid hormone may increase after somatropin treatment.
- The most common adverse reactions ($\geq 5\%$) in patients treated with SKYTROFA were: viral infection (15%), pyrexia (15%), cough (11%), nausea and vomiting (11%), hemorrhage (7%), diarrhea (6%), abdominal pain (6%), and arthralgia and arthritis (6%).

- SKYTROFA can interact with the following drugs:
- Glucocorticoids: SKYTROFA may reduce serum cortisol concentrations which may require an increase in the dose of glucocorticoids.
- Oral Estrogen: Oral estrogens may reduce the response to SKYTROFA. Higher doses of SKYTROFA may be required.
- Insulin and/or Other Hypoglycemic Agents: SKYTROFA may decrease insulin sensitivity. Patients with diabetes mellitus may require adjustment of insulin or hypoglycemic agents.
- Cytochrome P450-Metabolized Drugs: Somatropin may increase cytochrome P450 (CYP450)-mediated antipyrine clearance. Carefully monitor patients using drugs metabolized by CYP450 liver enzymes in combination with SKYTROFA.

You are encouraged to report side effects to FDA at (800) FDA-1088 or www.fda.gov/medwatch. You may also report side effects to Ascendis Pharma at 1-844-442-7236.

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

About SKYTROFA[®] (lonapegsomatropin-tcgd)

SKYTROFA[®] (lonapegsomatropin-tcgd) is a once-weekly prodrug designed to deliver somatropin over a one-week period. The released somatropin has the same 191 amino acid sequence as daily somatropin. SKYTROFA (lonapegsomatropin-tcgd) single-use, prefilled cartridges are available in nine dosage strengths, allowing for convenient dosing flexibility. They are designed for use only with the SKYTROFA[®] Auto-Injector and may be stored at room temperature for up to six months. The recommended dose of SKYTROFA (lonapegsomatropin-tcgd) for treatment-naïve patients and patients switching from daily somatropin is 0.24 mg/kg body weight, administered once weekly. The dose may be adjusted based on the child's weight and insulin-like growth factor-1 (IGF-1) SDS.

TransCon[™] hGH (known by its brand name SKYTROFA (Lonapegsomatropin-tcgh) in the U.S.) has been studied in over 300 treatment-naïve and treatment-experienced children with GHD across the Phase 3 program, which consists of the heiGHt Trial (for treatment-naïve patients), the fliGHt Trial (for treatment-experienced and treatment-naïve patients), and the enliGHten Trial (an ongoing long-term open-label extension trial). Patients who completed the heiGHt Trial or the fliGHt Trial were able to continue into the enliGHten Trial, and some have been treated with SKYTROFA (lonapegsomatropin-tcgd) for over four years.

TransCon hGH is currently under review by the European Medicines Agency (EMA) in Europe as a potential treatment for pediatric growth hormone deficiency. In addition, TransCon hGH is being evaluated for pediatric GHD in Phase 3 trials in Japan and the People's Republic of China, Ascendis Pharma is also conducting the global Phase 3 foresiGHt Trial in adults with GHD. TransCon hGH has been granted orphan designation for GHD in both the U.S. and Europe.

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' use of the Ascendis Signature Access Program[™] to support the use of SKYTROFA (lonapegsomatropin-tcgd) by families, caregivers, and physicians, (ii) Ascendis' use of its co-pay as well as a separate assistance program for patients and families facing financial need, (iii) Ascendis' ability to apply its platform technology 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 and distributors 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., the co-pay program, 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 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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