Skytrofa® (lonapegsomatropin-tcgd) – New orphan drug approval

- On August 25, 2021, Ascendis Pharma announced the FDA approval of Skytrofa (lonapegsomatropin-tcgd), 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).

- GH deficiency is a disease characterized by short stature and metabolic complications.

    — Skytrofa is the first once-weekly formulation of GH, somatropin.

- The efficacy of Skytrofa was established in an open-label, active-controlled study in 161 treatment-naïve, prepubertal pediatric patients with GH deficiency. Patients received once-weekly Skytrofa or daily Genotropin® (somatropin). The primary endpoint was annualized height velocity at week 52.

    — Treatment with once-weekly Skytrofa for 52 weeks resulted in an annualized height velocity of 11.2 cm/year. Patients treated with daily somatropin achieved an annualized height velocity of 10.3 cm/year after 52 weeks of treatment (estimate of treatment difference: 0.9, 95% CI: 0.2, 1.5).

- Skytrofa is contraindicated in patients with:

    — Acute critical illness after open heart surgery, abdominal surgery or multiple accidental trauma, or those with 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
    — Closed epiphyses
    — Active malignancy due to the risk of malignancy progression
    — Active proliferative or severe non-proliferative diabetic retinopathy because treatment with somatropin may worsen this condition
    — 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.

- Warnings and precautions for Skytrofa include increased mortality in patients with acute critical illness; severe hypersensitivity; increased risk of neoplasms; glucose intolerance and diabetes mellitus; intracranial hypertension; fluid retention; hypoadrenalism; hypothyroidism; slipped capital femoral epiphysis; progression of preexisting scoliosis; pancreatitis; lipoatrophy; sudden death in pediatric patients with Prader-Willi syndrome; and laboratory tests.

- The most common adverse reactions (≥ 5%) with Skytrofa use were viral infection, pyrexia, cough, nausea and vomiting, hemorrhage, diarrhea, abdominal pain, and arthralgia and arthritis.

- The recommended dose of Skytrofa for treatment-naïve patients and patients switching from daily somatropin therapy is 0.24 mg/kg body weight, given subcutaneously once-weekly. The dosage should be individualized and titrated based on response.

    — Therapy with Skytrofa should be supervised by a physician who is experienced in the diagnosis and management of pediatric patients with growth failure due to GH deficiency.
    — Refer to the Skytrofa drug label for complete dosing and administration recommendations.

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- Ascendis Pharma’s launch plans for Skytrofa are pending. Skytrofa will be available as a 3 mg, 3.6 mg, 4.3 mg, 5.2 mg, 6.3 mg, 7.6 mg, 9.1 mg, 11 mg and 13.3 mg lyophilized powder in single-dose, prefilled cartridges.