Ngenla™ (somatrogon-ghla) – New orphan drug approval

- On June 28, 2023, Pfizer announced the FDA approval of Ngenla (somatrogon-ghla), for the treatment of pediatric patients aged 3 years and older who have growth failure due to an inadequate secretion of endogenous growth hormone.

- Growth hormone deficiency is a rare disease characterized by the inadequate secretion of growth hormone from the pituitary gland. Without treatment, affected children will have persistent growth attenuation and a very short height in adulthood.

    - Growth hormone deficiency affects one in approximately 4,000 to 10,000 children.

- Ngenla is a human growth hormone that works by replacing the lack of growth hormone in the body.

- The efficacy of Ngenla was established in a randomized, open-label, active-controlled study in 224 treatment-naïve, prepubertal pediatric subjects with growth hormone deficiency. Patients received Ngenla 0.66 mg/kg/week or 0.034 mg/kg/day daily somatropin. The primary endpoint was annualized height velocity at week 52.

    - Treatment with once-weekly Ngenla resulted in an annualized height velocity of 10.1 cm/year vs. 9.8 cm/year with daily somatropin (least square mean treatment difference 0.3, 95% CI: -0.2, 0.9).

- Ngenla 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 somatropin
    - Hypersensitivity to somatrogon-ghla or any of the excipients in Ngenla
    - Closed epiphyses
    - Active malignancy due to the risk of malignancy progression
    - 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.

- Warnings and precautions for Ngenla 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 Ngenla use were injection site reactions, nasopharyngitis, headache, pyrexia, anemia, cough, vomiting, hypothyroidism, abdominal pain, rash, and oropharyngeal pain.

- The recommended dose of Ngenla is 0.66 mg/kg based on actual body weight administered once weekly by subcutaneous (SC) injection.

    - The dosage should be individualized for each patient based on the growth response.

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  - — Ngenla treatment should be supervised by a healthcare provider who is experienced in the diagnosis and management of pediatric patients aged 3 years and older with growth failure due to growth hormone deficiency.

- Pfizer plans to launch Ngenla in August 2023. Ngenla will be available as 24 mg/1.2 mL and 60 mg/1.2 mL single-patient-use prefilled pens.

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