Reference number(s)
4908-A

Specialty Guideline Management Skytrofa

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Skytrofa	lonapegsomatropin-tcgd

Indications

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-approved Indications[1]

Skytrofa is 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).

All other indications are considered experimental/investigational and not medically necessary.

Documentation

Submission of the following information is necessary to initiate the prior authorization review (where applicable):

Both initial and continuation of therapy requests:

- Growth chart
- Pretreatment insulin-like growth factor-1 (IGF-1) level (laboratory report or medical record

documentation)

Initial requests:

- Support for the diagnosis of neonatal growth hormone (GH) deficiency (medical documentation, laboratory report, or imaging report)
- Pretreatment growth hormone provocative test result(s) (laboratory report or medical record documentation)

Continuation of therapy requests:

- Total duration of treatment (approximate duration is acceptable)
- Date of last dose administered
- Approving health plan/pharmacy benefit manager
- Date of prior authorization/approval
- Prior authorization approval letter

IGF-1 levels vary based on the laboratory performing the analysis. Laboratory-specific values must be provided to determine whether the value is within the normal range.

Coverage Criteria

Pediatric Growth Hormone (GH) Deficiency[1-6]

Authorization of 12 months may be granted to members with pediatric growth hormone (GH) deficiency 1 year of age and older when EITHER of the following criteria is met:

- Member has a documented diagnosis of GH deficiency as a neonate (e.g., hypoglycemia with random GH level, evidence of multiple pituitary hormone deficiency, magnetic resonance imaging [MRI] results).
- Member meets ALL of the following criteria:
    - Member has either of the following:
        - Two pretreatment pharmacologic provocative GH tests with both results demonstrating a peak GH level < 10 ng/mL
        - A documented pituitary or central nervous system (CNS) disorder (see Appendix A) and a pretreatment IGF-1 level > 2 standard deviations (SD) below the mean
    - For members < 2.5 years of age at initiation of treatment, the pretreatment height is > 2 SD below the mean and growth velocity is slow
    - For members ≥ 2.5 years of age at initiation of treatment, member has either of the following:
        - Pretreatment height is > 2 SD below the mean and 1-year height velocity is > 1 SD below the mean
        - Pretreatment 1-year height velocity is > 2 SD below the mean
    - Epiphyses are open

Continuation of Therapy

Pediatric Growth Hormone (GH) Deficiency

Authorization of 12 months may be granted for continuation of therapy for pediatric growth hormone (GH) deficiency when ALL of the following criteria are met:

- Member is currently receiving the requested medication or another growth hormone product (e.g., Norditropin) indicated for pediatric GH deficiency
- Epiphyses are open[1,2] (confirmed by X-ray or X-ray is not available)
- Member's growth rate is > 2 cm/year unless there is a documented clinical reason for lack of efficacy (e.g., on treatment less than 1 year, nearing final adult height/late stages of puberty)

Appendix

Examples of Hypothalamic/Pituitary/CNS Disorders

- Congenital genetic abnormalities
  - Transcription factor defects (PIT-1, PROP-1, LHX3/4, HESX-1, PITX-2)
  - Growth hormone releasing hormone (GHRH) receptor gene defects
  - GH secretagogue receptor gene defects
  - GH gene defects
- Congenital structural abnormalities
  - Optic nerve hypoplasia/septo-optic dysplasia
  - Agenesis of corpus callosum
  - Empty sella syndrome
  - Ectopic posterior pituitary
  - Pituitary aplasia/hypoplasia
  - Pituitary stalk defect
  - Holoprosencephaly
  - Encephalocele
  - Hydrocephalus
  - Anencephaly or prosencephaly
  - Arachnoid cyst
  - Other mid-line facial defects (e.g., single central incisor, cleft lip/palate)
  - Vascular malformations

- Acquired structural abnormalities (or causes of hypothalamic/pituitary damage)
    - CNS tumors/neoplasms (e.g., craniopharyngioma, glioma/astrocytoma, pituitary adenoma, germinoma)
    - Cysts (Rathke cleft cyst or arachnoid cleft cyst)
    - Surgery
    - Radiation
    - Chemotherapy
    - CNS infections
    - CNS infarction
    - Inflammatory processes (e.g., autoimmune hypophysitis)
    - Infiltrative processes (e.g., sarcoidosis, histiocytosis, hemochromatosis)
    - Head trauma/traumatic brain injury
    - Aneurysmal subarachnoid hemorrhage
    - Perinatal or postnatal trauma
    - Surgery of the pituitary or hypothalamus

References

1. Skytrofa [package insert]. Princeton, NJ: Ascendis Pharma Endocrinology, Inc.; May 2024.
2. Gharib H, Cook DM, Saenger PH, et al. American Association of Clinical Endocrinologists Growth Hormone Task Force. Medical guidelines for clinical practice for growth hormone use in adults and children 2003 Update. Endocr Pract. 2003;9(1):64-76.
3. National Institute for Clinical Excellence: Guidance on the use of human growth hormone (somatropin) for the treatment of growth failure in children. May 2010. http://www.nice.org.uk/guidance/ta188. Accessed January 14, 2025.
4. Wilson TA, Rose SR, Cohen P, et al. Update of Guidelines for the Use of Growth Hormone in Children: The Lawson Wilkins Pediatric Endocrinology Society Drug and Therapeutics Committee. J Pediatr. 2003;143:415-421.
5. Franklin SL, Geffner ME. Growth hormone: the expansion of available products and indications. Pediatr Clin North Am. 2011;58:1141-1165.
6. Grimberg A, DiVall SA, Polychronakos C, et al. Guidelines for growth hormone and insulin-like growth factor-I treatment in children and adolescents: growth hormone deficiency, idiopathic short stature, and primary insulin-like growth factor-I deficiency. Horm Res Paediatr. 2016;86:361-397.