Reference number(s)
4908-A

SPECIALTY GUIDELINE MANAGEMENT

SKYTROFA (lonapegsomatropin-tcgd)

POLICY

I. 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 Indication
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.

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

II. DOCUMENTATION

The following information is necessary to initiate the prior authorization review for both initial and continuation of therapy requests (where applicable):

A. Medical records supporting the diagnosis of neonatal growth hormone (GH) deficiency
B. Pretreatment growth hormone provocative test result(s) (laboratory report or medical record documentation)
C. Growth chart
D. Pretreatment IGF-1 level (laboratory report or medical record documentation)*
E. The following information must be provided for all continuation of therapy requests:
    1. Total duration of treatment (approximate duration is acceptable)
    2. Date of last dose administered
    3. Approving health plan/pharmacy benefit manager
    4. Date of prior authorization/approval
    5. 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.

III. CRITERIA FOR INITIAL APPROVAL

Pediatric growth hormone (GH) deficiency
Authorization of 12 months may be granted to members with pediatric GH deficiency in members 1 year of age and older when EITHER criteria A. or B. below is met:

A. Member was diagnosed with GH deficiency as a neonate. Medical records must be available to support the diagnosis of neonatal GH deficiency (e.g., hypoglycemia with random GH level, evidence of multiple pituitary hormone deficiency, chart notes, or magnetic resonance imaging [MRI] results).
B. Member meets ALL of the following:
    1. Member has EITHER:

i. Two pretreatment pharmacologic provocative GH tests with both results demonstrating a peak GH level < 10 ng/mL, OR
ii. A documented pituitary or CNS disorder (refer to Appendix A) and a pretreatment IGF-1 level > 2 standard deviations (SD) below the mean

2. 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
3. For members ≥ 2.5 years of age at initiation of treatment:
   i. Pretreatment height is > 2 SD below the mean and 1-year height velocity is > 1 SD below the mean, OR
   ii. Pretreatment 1-year height velocity is > 2 SD below the mean
4. Epiphyses are open

IV. CONTINUATION OF THERAPY

Pediatric GH deficiency
Authorization of 12 months may be granted for continuation of therapy when ALL of the following criteria are met:

A. Epiphyses are open (confirmed by X-ray or X-ray is not available)
B. 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)

V. APPENDIX

Examples of Hypothalamic/Pituitary/CNS Disorders

1. Congenital genetic abnormalities
   a. Known mutations in growth-hormone-releasing hormone (GHRH) receptor, GH gene, GH receptor, or pituitary transcription factors
   b. Perinatal insults
2. Congenital structural abnormalities
   a. Optic nerve hypoplasia/septo-optic dysplasia
   b. Agenesis of corpus callosum
   c. Empty sella syndrome
   d. Ectopic posterior pituitary
   e. Pituitary aplasia/hypoplasia
   f. Pituitary stalk defect
   g. Anencephaly or prosencephaly
   h. Other mid-line defects
   i. Vascular malformations
3. Acquired structural abnormalities (or causes of hypothalamic/pituitary damage)
   a. CNS tumors/neoplasms (e.g., craniopharyngioma, glioma, pituitary adenoma)
   b. Cysts (Rathke cleft cyst or arachnoid cleft cyst)
   c. Surgery
   d. Radiation
   e. Chemotherapy
   f. CNS infections
   g. CNS infarction (e.g., Sheehan's syndrome)
   h. Inflammatory lesions (e.g., autoimmune hypophysitis)
   i. Infiltrative lesions (e.g., sarcoidosis, histiocytosis)
   j. Head trauma/traumatic brain injury
   k. Aneurysmal subarachnoid hemorrhage

VI. REFERENCES

1. Skytrofa [package insert]. Palo Alto, CA: Ascendis Pharma, Inc.; August 2021.
2. 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/nicemedia/live/12992/48715/48715.pdf. Accessed January 28, 2019.
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.