Effective Date: 8/01/2022
Reviewed: 05/2022, 3/2023, 12/2023, 03/2024, 04/2025 Scope: Medicaid

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 contraindications or exclusions to the prescribed therapy.

A. FDA-Approved Indications
1. Pediatric patients 1 year and older who weigh at least 11.5kg and have growth failure due to inadequate secretion of endogenous growth hormone.

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

II. PRESCRIBER SPECIALTIES

Therapy must be prescribed by or in consultation with any of the following specialists:
A. Endocrinologist
B. Pediatric endocrinologist

III. INITIAL CRITERIA FOR APPROVAL

Growth charts are required for pediatric patients with growth hormone (GH) deficiency.

A. Pediatric GH Deficiency

Authorization of 12 months may be granted to members with pediatric GH deficiency when the following criteria is met:
1. Documentation that the member meets one of the following:
   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:
      i. Member has EITHER:
         a. Two pretreatment pharmacologic provocative GH tests with both results demonstrating a peak GH level < 10 ng/mL, OR
         b. A documented pituitary or CNS disorder (refer to Appendix A) and a pretreatment IGF-1 level > 2 standard deviations (SD) below the mean

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

2. Documentation that member is at least 1 year of age and weighs at least 11.5kg
3. Documentation that the member does not have a diagnosis of intracranial tumor growth, psychosocial dwarfism, idiopathic short stature, or history or presence of malignant disease.
4. Documentation that the member has had a treatment failure with at least 2 daily growth hormone products (e.g., based on claims review of inadequate adherence or documentation of injection site reactions)

IV. CONTINUATION OF THERAPY

A. Pediatric GH Deficiency

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

1. Documentation of the member's diagnosis must be provided.
2. Documentation that the 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, hypopituitarism)
3. Documentation that the epiphyses are open (confirmed by X-ray or X-ray is not available)

V. APPENDICES

A. Appendix A: 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

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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.
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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.