Daybue (trofinetide)

NOTICE

This policy contains information which is clinical in nature. The policy is not medical advice. The information in this policy is used by Wellmark to make determinations whether medical treatment is covered under the terms of a Wellmark member's health benefit plan. Physicians and other health care providers are responsible for medical advice and treatment. If you have specific health care needs, you should consult an appropriate health care professional. If you would like to request an accessible version of this document, please contact customer service at 800-524-9242.

BENEFIT APPLICATION

Benefit determinations are based on the applicable contract language in effect at the time the services were rendered. Exclusions, limitations or exceptions may apply. Benefits may vary based on contract, and individual member benefits must be verified. Wellmark determines medical necessity only if the benefit exists and no contract exclusions are applicable. This medical policy may not apply to FEP. Benefits are determined by the Federal Employee Program.

DESCRIPTION

The intent of the policy is to provide coverage consistent with product labeling, FDA guidance, standards of medical practice, evidence-based drug information, and/or published guidelines. The indications below including FDA-approved indications and compendial uses are considered covered benefits provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications

Daybue (trofinetide) is indicated for the treatment of Rett syndrome in adults and pediatric patients 2 years of age and older.

POLICY

Required Documentation

Submission of the following information is necessary to initiate the prior authorization review:

A. Initial requests:
   1. Genetic testing results confirming a mutation in the MECP2 gene
   2. Baseline assessment score (Rett Syndrome Behavioral Questionaire [RSBQ] or Clinical Global Impressions-Severity [CGI-S])

B. Continuation requests:
   1. Medical records documenting a positive clinical response to therapy (e.g., stabilization or improvement in repetitive movements, mood dysfunction/disruptive behavior, vocalization, ambulation)

2. Assessment scores (Rett Syndrome Behavioral Questionaire [RSBQ], Clinical Global Impressions-Severity [CGI-S], or Clinical Global Impressions-Improvement [CGI-I]) demonstrating a stabilization or improvement from baseline

Prescriber Specialties

This medication must be prescribed by or in consulation with a neurologist, geneticist, or developmental pediatrician experienced in the treatment of Rett syndrome.

Criteria for Initial Approval

Authorization of 6 months may be granted for treatment of Rett syndrome when all of the following criteria are met:

A. Member is 2 years of age or older
B. Diagnosis of classic or typical Rett Syndrome confirmed by meeting ALL of the following criteria:
    1. Partial or complete loss of acquired purposeful hand skills
    2. Partial or complete loss of acquired spoken language
    3. Gait abnormalities: impaired or absence of ability
    4. Stereotypic hand movements such as hand wringing/squeezing, clapping/tapping, mouthing, and washing/rubbing automatisms
C. Confirmed mutation in the MECP2 gene
D. Documentation of at least one of the following baseline assessment scores:
    1. Rett Syndrome Behavioral Questionnaire (RSBQ) score (see Appendix A)
    2. Clinical Global Impression-Severity (CGI-S) score (see Appendix B)

Continuation of Therapy

Authorization of 12 months may be granted for continued treatment of Rett syndrome in members who meet the Criteria for Initial Approval (A through C above)* AND when all of the following criteria are met:

A. Member is experiencing a positive clinical response to therapy as demonstrated by an improvement or stabilization of symptoms (e.g., stabilization or improvement of repetitive movements, mood dysfunction/disruptive behavior, vocalization, ambulation)
B. Member meets ONE of the following criteria:
    1. Improvement on the Rett Syndrome Behavioral Questionnaire (RSBQ) score from baseline (see Appendix A)
    2. Clinical Global Impression-Improvement (CGI-I) score of ≤ 4 (see Appendix B)
    3. Improvement on the Clinical Global Impression-Severity (CGI-S) score from baseline (see Appendix B)

    *Baseline RSBQ and CGI-S are not required if CGI-I score is provided for continuation of therapy.

Daybue (trofinetide) is considered **not medically necessary** for members who do not meet the criteria set forth above.

Dosing and Administration

Approvals may be subject to dosing limits in accordance with FDA-approved labeling, accepted compendia, and/or evidence-based practice guidelines.

Quantity Limits

Medication	FDA-recommended dosing		Standard Limit
	Weight	Volume	
Daybue (trofinetide) 200mg/mL (450 mL per bottle)	9 kg to < 12 kg	25 mL twice daily	8 bottles (3600 mL) per 30 days
	12 kg to < 20 kg	30 mL twice daily	
	20 kg to < 35 kg	40 mL twice daily	
	35 kg to < 50 kg	50 mL twice daily	
	≥ 50 kg	60 mL twice daily	

Appendices

Appendix A:

The Rett Syndrome Behavioral Questionnaire (RSBQ) consists of 45 questions across 8 categories associated with behavioral changes seen in typical Rett Syndrome. Each question is scored on a 3-point Likert scale where the questions are scored as a 0, 1, or 2 (0=never true, 1=sometimes true, and 2=always thrue). Scores range from zero to 90 where a higher score is indicative of more severe manifestation of Rett Syndrome symptoms.

RSBQ Category
General mood
Breathing problems
Hand behaviors
Repetitive face movements
Body rocking and expressionless face
Night-time behaviors
Fear/anxiety
Walking/standing

Appendix B:

The Clinical Global Impression (CGI) scale is used by the clinician to evaluate the severity (CGI-S) of Rett Syndrome symptoms and to evaluate the improvement in symptoms (CGI-I) after an intervention or therapy has been initiated. The CGI-S scale requires the clinician to evaluate the severity of illness as a relative measurement in comparison to other patients in the clinicians past experience with the same diagnosis. CGI-I is a scale that evaluates how a particular patient has either improved or worsened since the last evaluation of that patient's disease. The assessments are based on 7-point scales where a lower number reflects less severe disease (CGI-S) or greater improvement from baseline (CGI-I), and a higher number indicates more severe disease (CGI-S) or a clinical worsening of the condition (CGI-I).

Score	CGI-S	CGI-I
1	Normal, no illness	Very much improved
2	Borderline illness	Much improved
3	Mild illness	Minimally improved
4	Moderate illness	No change
5	Marked illness	Minimally worse
6	Severe illness	Much worse
7	Extreme illness	Very much worse

PROCEDURES AND BILLING CODES

To report provider services, use appropriate CPT* codes, Alpha Numeric (HCPCS level 2) codes, Revenue codes, and/or ICD diagnostic codes.

- N/A

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POLICY HISTORY

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