

DAYBUE (trofinetide)

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

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

II. 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. Medical records documenting clinical manifestations of disease
3. Documentation of at least one of the following baseline assessment scores:
 - a. Rett Syndrome Behavior Questionnaire (RSBQ)
 - b. Clinical Global Impression-Severity (CGI-S) of ≥ 4

B. Continuation requests:

1. Documentation of improvement in baseline assessment score

III. PRESCRIBER SPECIALTIES

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of Rett syndrome:

- A. Geneticist
- B. Pediatrician who specializes in childhood neurological or developmental disorders
- C. Neurologist

IV. CRITERIA FOR INITIAL APPROVAL

Rett Syndrome

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

- A. Member is 2 years of age or older and weighs ≥ 9 kg
- B. The diagnosis is confirmed by a mutation in the *MECP2* gene
- C. Member exhibits clinical manifestations of disease (e.g., hand wringing, apraxia, gait abnormalities, developmental delays) as documented by:
 1. **RSBQ OR**

2. CGI-S score ≥ 4

V. CONTINUATION OF THERAPY

Authorization of 6 months may be granted for continued treatment of Rett syndrome in members who are experiencing benefit from therapy (e.g, stabilization or improvement in repetitive movements, mood dysfunction/disruptive behavior, vocalization, ambulation) as documented by one of the following:

1. ≥ 3 point reduction in overall RSBQ total score from baseline
2. A current CGI-S score < 4