Reference number(s)
5833-A

SPECIALTY GUIDELINE MANAGEMENT

DAYBUE (trofinetide)

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
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 for initial requests:
A. Genetic testing results confirming a mutation in the *MECP2* gene.
B. Medical records documenting clinical manifestations of disease.

III. PRESCRIBER SPECIALTIES

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

IV. CRITERIA FOR INITIAL APPROVAL

Rett Syndrome
Authorization of 12 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. 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).

V. CONTINUATION OF THERAPY

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in Section IV who are experiencing benefit from therapy (e.g., stabilization or improvement in repetitive movements, mood dysfunction/disruptive behavior, vocalization, ambulation).

VI. REFERENCES

1. Daybue [package insert]. San Diego, CA: Acadia Pharmaceuticals, Inc.; March 2023.

2. Neul JL, Percy AK, Benke TA, et al. Design and outcome measures of LAVENDER, a phase 3 study of trofinetide for Rett syndrome. Contemp Clin Trials. 2022;114:106704.
3. Neul JL, Eskind AS. Rett syndrome: NORD. National Organization for Rare Disorders. https://rarediseases.org/rare-diseases/rett-syndrome/#complete-report Published March 15, 2023. Accessed February 2, 2024.