TROFINETIDE

Included Products: Daybue (trofinetide)

Created: 07/13/2023 *Revised:* 03/14/2024 *Reviewed*: 03/14/2024 *Updated*: 04/01/2024

Statement of intent: Daybue should be initiated by a specialist with experience in diagnosing and treating Rett Syndrome, and other conditions must be ruled out. Due to the high incidence of vomiting and/or diarrhea and associated weight loss, a precise initial weight and weight monitoring is required. Baseline Rett Syndrome Behavior Questionnaire (RSBQ) filled out by the caregiver will provide a starting point to determine response. Daybue was studied in patients between 2 and 20 years of age, and is indicated for patients age 2 or older.

Rett Syndrome			
Initial Criteria: All Diagnoses		If yes	If no
1.	Is Daybue being prescribed by or in consultation with a neurologist with expertise in treating Rett Syndrome?	Continue to #2.	Do not approve.
2.	Is the member at least 2 years old?	Continue to #3.	Do not approve.
3.	Does member have a mutation of the MECP2 gene?	Continue to #4.	Do not approve.
4.	Does member have a diagnosis of classic/typical Rett syndrome as confirmed by ALL of the following diagnostic criteria? a. Partial or complete loss of acquired purposeful hand skills b. Partial or complete loss of acquired spoken language c. Gait abnormalities: Impaired or absence of ability d. Stereotypic hand movements such as hand wringing/squeezing, clapping/tapping, mouthing, and washing/rubbing automatisms	Continue to #5.	Do not approve.
5.	Is there a baseline weight documented and at least 12 kg?	Continue to #6.	Do not approve.
6.	Has the baseline Rett Syndrome Behavior Questionnaire (RSBQ) score been documented?	Continue to #7.	Do not approve.

7.	Have all laxatives been stopped before initiating Daybue?	Continue to #8.	Do not approve.
8.	Approve for 3 months		
Renewal Criteria		If yes	If no
1.	Has the provider documented a current weight that demonstrates weight maintenance while on Daybue? Note: Due to high incidence of GI side effects, significant weight loss is a potential risk and should be monitored.	Continue to #2.	Do not approve.
2.	Is there objective documentation (RSBQ and CGI-I) that the symptoms have been maintained or improved while on Daybue?	Continue to #3.	Do not approve.
3.	Approve for 12 months.		

REFERENCES

- Daybue FDA Drug Label 3/10/23