Division: Pharmacy Policy	Subject: Prior Authorization Criteria
Original Development Date: Original Effective Date: Revision Date:	June 27, 2023 November 8, 2023, May 29, 2025

Daybue™ (trofinetide)

LENGTH OF AUTHORIZATION: Initial therapy: 3 months
Continuation of therapy: 6 months

REVIEW CRITERIA:

- Patient must be ≥ 2 years of age.
- Patient must have a diagnosis of Rett syndrome confirmed by the following:
  - Documentation of molecular genetic testing confirming heterozygous methyl-CpG binding protein-2 (MECP2) pathogenic variant gene mutations; **AND**
  - Baseline disease severity of behavior and/or functionality using an objective measure or tool (e.g., Clinical Global Impression-Improvement [CGI-I] score, Motor-Behavior Assessment [MBA], Interval History Form, Clinical Severity Scale, Rett Syndrome Gross Motor Scale); **OR**
  - Documented, detailed baseline clinical presentation of Rett syndrome including, but not limited to the following:
    - Abnormal muscle tone/dystonia
    - Abnormal respiration pattern
    - Feeding difficulties
    - Intellectual disability (i.e., I.Q. score < 70)
    - Loss of mobility or gait abnormalities
    - Partial or complete loss of acquired hand skills
    - Partial or complete loss of speech
    - Seizures
    - Stereotypic hand movements such as hand wringing/squeezing, clapping/tapping, mouthing, and washing/rubbing automatisms
- Prescriber attests patient does not have severe renal impairment (estimated glomerular filtration rate (eGFR) < 30 ml/min for adults or < 30 ml/min/1.73m^2 for pediatrics).
- Patient does not have progressive weight loss prior to therapy initiation.

CONTINUATION OF THERAPY:

- Patient met initial review criteria;
- Documentation of a positive response to therapy from pre-treatment baseline as demonstrated by:
  - Disease stability; **OR**
  - Clinically significant improvement in core symptoms; **OR**
  - An objective measure or tool (e.g., Clinical Global Impression-Improvement [CGI-I] score, Motor-Behavior Assessment [MBA], Interval History Form, Clinical Severity Scale, Rett Syndrome Gross Motor Scale).
- Patient has not experienced any treatment-restricting adverse effects (e.g., severe diarrhea or dehydration, significant weight loss).

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DOSING AND ADMINISTRATION:

- Refer to product labeling at https://www.accessdata.fda.gov/scripts/cder/daf/
- Available as a 200 mg/mL oral solution, 450 ml bottle. Store upright and refrigerate once opened. Discard any unused portion after 14 days of first opening the bottle.