

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

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** baseline glomerular filtration rate (GFR) > 45ml/min/m².
- 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.