Trofinetide (DAYBUE)

Goal(s):

- Promote use that is consistent with medical evidence and product labeling in patients with Rett syndrome.

Length of Authorization:

- Up to 12 months

Requires PA:

- Trofinetide oral solution

Covered Alternatives:

- Current PMPDP preferred drug list per OAR 410-121-0030 at www.orpdl.org
- Searchable site for Oregon FFS Drug Class listed at www.orpdl.org/drugs/

Table 1. Recommended Weight-Based Trofinetide Oral Solution 200 mg/mL Dosing

Patient Weight	Trofinetide Dosage	Trofinetide Volume
9 kg to less than 12kg	5,000 mg twice daily	25 mL twice daily
12 kg to less than 20 kg	6000 mg twice daily	30 mL twice daily
20 kg to less than 35 kg	8,000 mg twice daily	40 mL twice daily
35 kg to less than 50 kg	10,000 mg twice daily	50 mL twice daily
50 kg or more	12,000 mg twice daily	60 mL twice daily
Abbreviations: kg = kilograms; mg = milligrams; mL = millilters		

Approval Criteria		
1. What diagnosis is being treated?	Record ICD10 code.	
2. Is the request for continuation of therapy previously approved by the FFS program?	**Yes:** Go to **Renewal Criteria**	**No:** Go to #3
3. Does the patient have a diagnosis of Rett syndrome?	**Yes:** Go to #4	**No:** Pass to RPh. Deny; medical appropriateness
4. Is there documentation of genetic testing to confirm Rett syndrome diagnosis?	**Yes:** Go to #5	**No:** Pass to RPh. Refer to Medical Director for review.
5. Is the requested medication prescribed by a neurologist or a provider with experience in treating Rett syndrome?	**Yes:** Go to #6	**No:** Pass to RPh. Deny; medical appropriateness
6. Is the request for an FDA approved age (e.g., 2 years of age and older)?	**Yes**: Go to #7	**No:** Pass to RPh. Deny; medical appropriateness
7. Is the request for an approved weight-based dosing regimen (see **Table 1**)?	**Yes:** Go to #8	**No:** Pass to RPh. Deny; medical appropriateness

Approval Criteria		
8. Has the provider documented specific and measurable goals of therapy? Note: Documentation should include what will be assessed, how progress will be measured, and timeline for assessment. Goals should be attainable within 6 months and relevant to the condition or health of the patient. Documentation of progress toward or achievement of therapeutic goals will be required for renewal.	**Yes:** Document Assessment and Date:_____ _____ Approve for 6 months. Note: The first 2 pharmacy fills are limited to 14 days each to assess tolerance to therapy. Initial fills can overlap to ensure adequate time for delivery. 1.Approve Initial Request for enough units up to 14 days. 2. Approve enough units to cover subsequent 14-28 days. 3. Approve enough units for up to 6 months (5 to 24 weeks).	**No:** Pass to RPh. Deny; medical appropriateness

Renewal Criteria		
1. Is there evidence of adherence and tolerance to therapy through pharmacy claims/refill history and/or provider assessment?	**Yes:** Go to #2	**No:** Pass to RPh; Deny; medical appropriateness.
2. Has the patient met the goals of therapy described in the initial authorization by the prescribing provider and provider attests to patient's stabilization on therapy?	**Yes:** Approve for 12 months. Document assessment and provider attestation received.	**No:** Pass to RPh; Deny; medical appropriateness.