CareSource Rx Innovations

PHARMACY POLICY STATEMENT
Indiana Medicaid

DRUG NAME	Daybue (trofinetide)
BENEFIT TYPE	Pharmacy
STATUS	Prior Authorization Required

Daybue is an insulin-like growth factor-1 (IGF-1) analog of glycine-proline-glutamate (GPE) that was FDA – approved for the treatment of Rett syndrome in March 2023. Rett syndrome is a rare, debilitating neurodevelopmental disorder that affects the way the brain develops. The condition is characterized by a progressive loss of motor skills affecting the ability to speak, walk, eat, and breathe. Daybue is the first FDA-approved treatment for Rett syndrome in adults and children 2 years of age or older.

Daybue (trofinetide) will be considered for coverage when the following criteria are met:

Rett Syndrome

For **initial** authorization:

1. Member is at least 2 years of age or older; AND
2. Medication must be prescribed by or in consultation with a neurologist, geneticist, or a physician with expertise in Rett syndrome; AND
3. Member has a confirmed diagnosis of Typical or Classic Rett syndrome with a period of regression followed by recovery or stabilization, confirmed by ALL of the following:
   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 to walk;
   d) Hand wringing/squeezing/clapping/tapping, mouthing, and/or washing/rubbing that seems habitual or uncontrollable; AND
4. Member must have documentation of a baseline evaluation with the Rett Syndrome Behavior Questionnaire (RSBQ) or the Clinical Global Impression-Improvement (CGI-I) score; AND
5. Member does not have ANY of the following:
   a) History of brain injury secondary to trauma, neurometabolic disease, or severe infection that causes neurological problems; OR
      a. Grossly abnormal psychomotor development in first 6 months of life
6. **Dosage allowed/Quantity limit:**

Patient Weight	DAYBUE Dosage	DAYBUE Volume
9 kg to less than 12 kg	5,000 mg twice daily	25 mL twice daily
12 kg to less than 20 kg	6,000 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

If all the above requirements are met, the medication will be approved for 3 months.

For **reauthorization**:

1. Chart notes must show improvement or stabilized signs and symptoms of disease demonstrated by improvement or stabilization on the Rett Syndrome Behavior Questionnaire (RSBQ) or the Clinical Global Impression-Improvement (CGI-I) score

If all the above requirements are met, the medication will be approved for an additional 12 months.

CareSource considers Daybue (trofinetide) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
05/01/2023	New policy for Daybue created.

References:

1. International Rett Syndrome Foundation. Rett Syndrome Diagnosis. International Rett Syndrome Foundation. February 2023.
2. Center for Drug Evaluation and Research. FDA approves first treatment for Rett syndrome. US Food and Drug Administration. March 2023.
3. National Institute of Neurological Disorders and Stroke. Rett syndrome. National Institute of Neurological Disorders and Stroke. April 2023.
4. Acadia Pharmaceuticals. Daybue (trofinetide) oral solution. Accessdata FDA. March 2023.
5. Neul JL, Percy AK, Benke TA, et al. Design and outcome measures of Lavender, a phase 3 study of trofinetide for Rett Syndrome. Elsevier. October 2021.
6. Fu C, Armstrong D, Marsh E, et al. Consensus guidelines on managing Rett syndrome across the lifespan. BMJ Paediatr Open. 2020;4(1):e000717. Published 2020 Sep 13. doi:10.1136/bmjpo-2020-000717

Effective date: 10/01/2023
Revised date: 05/01/2023