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

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

References:

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
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 -000717

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