Clinical Oversight Review Board (CORB) Criteria for Prescribing/ Criteria-Based Consultation (CBC) Criteria for Coverage

trofinetide (Daybue)

Notes:

- Quantity Limits: Yes

Non-Formulary **trofinetide (Daybue)** requires a clinical review. Appropriateness of therapy will be based on the following criteria:

Initiation (new start) criteria and criteria for *new members* entering Kaiser Permanente already taking the medication who have not been reviewed previously:
Non-formulary **trofinetide (Daybue)** will be covered on the prescription drug benefit for 12 months when the following criteria are met:

- Classic/typical Rett syndrome with documented disease-causing mutation in the MECP2 gene
- Age 5 to 10 years old
- Patient is a female
- Patient has failed behavioral, rehabilitative and/or pharmacological therapies targeting Rett syndrome related characteristics (e.g., physical therapy, occupational therapy, applied behavioral analysis [ABA], behavioral health treatment [BHT], and/or anxiolytics)
- Patient weighs at least 12 kg, and does not have progressive weight loss
- Patient has stable disease (i.e., at least six months “post regression” [no further loss or degradation in ambulation, hand function, speech, nonverbal communicative or social skills])
- Patient has a Clinical Global Impression Scale-Severity (CGI-S) score of at least 4
- Has a stable pattern of seizures or has not had a seizure within eight weeks of treatment initiation
- Normal eGFR and QTc within previous year
- Patient has been reviewed by the Kaiser Permanente Interregional Consultative Physician Panel, with recommendation to use medication

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Continued use criteria (12 months after initiation): Non-formulary **trofinetide (Daybue)** will continue to be covered on the prescription drug benefit when the following criteria are met:

- Patient must have clinically meaningful benefit
  - at least one point improvement from baseline in at least one of the clinical domains in the CGI-S.
    If patient achieved improvement, but there has been a lack of measurable progress over one year, a gradual temporary withdrawal trial of trofinetide is recommended to determine if trofinetide is providing any benefit.