

**DAYBUE
(trofinetide) oral solution****RATIONALE FOR INCLUSION IN PA PROGRAM****Background**

Daybue (trofinetide) is indicated for the treatment of Rett syndrome. Rett syndrome is a rare genetic neurological disorder that occurs almost exclusively in girls, more rarely in boys, and leads to severe impairments, including their ability to speak, walk, eat, and even breathe easily. The hallmark of Rett syndrome is near constant repetitive hand movements. Rett syndrome is usually recognized in children between 6 and 18 months as they begin to miss developmental milestones or lose abilities they had gained. Rett syndrome is caused by mutations on the X chromosome on a gene called MECP2. The mechanism of action by which Daybue exerts therapeutic effects in patients with Rett syndrome is unknown (1-2).

Regulatory Status

FDA-approved indication: Daybue is indicated for the treatment of Rett syndrome in adults and pediatric patients 2 years of age and older (1).

Daybue contains warnings regarding diarrhea, weight loss, and vomiting (1).

Patients should be advised to stop laxatives before starting Daybue. Interrupt, reduce the dosage, or discontinue Daybue if severe diarrhea occurs, if dehydration is suspected, or if significant weight loss occurs (1).

The safety and effectiveness of Daybue in pediatric patients less than 2 years of age have not been established (1).

Summary

Daybue (trofinetide) is indicated for the treatment of Rett syndrome, a rare genetic neurological disorder that occurs almost exclusively in girls. Prescribers should monitor patients being treated with Daybue for diarrhea and weight loss. The safety and effectiveness of Daybue in pediatric patients less than 2 years of age have not been established (1).

Prior authorization is required to ensure the safe, clinically appropriate, and cost-effective use of Daybue while maintaining optimal therapeutic outcomes.



**BlueCross
BlueShield**

Federal Employee Program.

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References

1. Daybue [package insert]. San Diego, CA: Acadia Pharmaceuticals Inc.; September 2024.
2. About Rett Syndrome: International Rett Syndrome Foundation. 2023.
<https://www.rett syndrome.org/about-rett-syndrome/>