Trofinetide

Also called: Daybue; NNZ-2566

What it is

An oral synthetic tripeptide analogue derived from IGF-1, approved as Daybue in March 2023 for Rett syndrome, a rare genetic neurodevelopmental disorder.

Approval status

FDA Approved. Approved in the US as Daybue (trofinetide), NDA 217026, Acadia Pharmaceuticals, on March 10, 2023, for Rett syndrome in adults and children aged 2 years and older (Drugs@FDA via openFDA and the Daybue label, read October 3, 2026).

Dosing, as printed in the page's Recommended Dosing box

Manufacturer's recommended dosing (FDA label)

From the prescribing information. Other doses studied, if any, are in the full dosing data below.

Protocol and source	Dose	Frequency	Duration	Route
Label: by weight band **[Approved label]** Daybue (trofinetide) oral solution US prescribing information (DailyMed set 67e6f2d9-21f6-466f-9def-826c6a4b8257, effective September 4, 2026)	5,000 mg (9–12 kg) up to 12,000 mg (50 kg or more)	Twice daily	Ongoing	Oral or gastrostomy

Used in human trials

No manufacturer dose exists for this use: these are amounts given in published trials, not recommendations.

Protocol and source	Dose	Frequency	Duration	Route
Paediatric phase 2 (2019) **[Human study]** PMID 30918097	50, 100 or 200 mg/kg	Twice daily	42 days	Oral

Educational information, not medical advice. Each dose above is tagged with its source: an approved label, a published human study, animal data, or amounts reported by users. None is a recommendation for you. An unapproved compound has no established safe or effective human dose, and products sold for "research use only" are not made or tested for people. Talk to a doctor before acting on anything on this site, including before you start, stop or change any medicine or dose.

Key human evidence

The first 3 of the 13 human studies carded on the page (15 study cards in all):

• **LAVENDER phase 3, 187 patients** (Human, 2023). Over 12 weeks, the Rett Syndrome Behaviour Questionnaire improved by 4.9 points on trofinetide against 1.7 on placebo, and the clinician's global improvement score was 3.5 against 3.8 (lower is better); both co-primary end points were met, with effect sizes of 0.37 and 0.47. Diarrhoea affected 80.6% against 19.1%. **PMID 37291210**

• **Communication in LAVENDER** (Human, 2023). A caregiver-rated communication score improved by 1.0 point more on trofinetide than placebo (effect size 0.43), and a clinician rating of non-verbal choice-making improved nominally; verbal communication did not change. **PMID 38232652**

• **LILAC: 40-week extension** (Human, 2024). In 154 participants aged 5 to 21, behaviour scores improved by about 7 points from the original baseline by week 40; diarrhoea affected 74.7% and was the commonest reason for stopping (21.4%). **PMID 38917793**

Interactions

From the FDA label's drug-interaction section (Acadia. DAYBUE (trofinetide) US prescribing information, section 7. DailyMed version of September 4, 2026, read October 3, 2026):

• With sensitive oral CYP3A and/or P-gp substrates: trofinetide can raise their levels; closely monitor for adverse reactions when a small increase could be serious (FDA label)

Side effects and cautions

Reported side effects	Contraindications and cautions
• Diarrhoea, in about four in five patients in the trials • Vomiting • Weight loss • Diarrhoea was the most common reason for stopping in the 40-week LILAC extension, where it affected 74.7% • Vomiting, especially in the youngest children (53.3% in DAFFODIL) (1 more on the page)	• Diarrhoea: stop laxatives before starting and manage dehydration • Weight loss: monitor weight • Vomiting • Tested athletes — see the Sport (WADA) row below

Five questions to ask

1. Is Trofinetide right for me, and is the labelled dose on this sheet right for my weight, age, kidney and liver function?
2. Here are the medicines and supplements I take. Do any of them interact with Trofinetide? This sheet lists the interactions on record.
3. Which side effects should make me stop and call you, for example diarrhoea?
4. Do any of the cautions on this sheet apply to me, such as: Diarrhoea: stop laxatives before starting and manage dehydration?
5. What should we check before I start and while I am on it (blood tests, blood pressure, weight), and how often?

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