

Overview of DAYBUE® (trofinetide)

Indication

DAYBUE is indicated for the treatment of Rett syndrome in adults and pediatric patients 2 years of age and older.¹

Program Details

The program is scheduled on:

Thursday, April 9, 2026, 6:30 PM CST

Presenting live

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Pediatric Nurse Practitioner

Wichita, KS

Cask n Cork

Upstairs Private Room

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Presenter is a paid consultant.

Program Objectives:

- Review Rett syndrome diagnostic criteria, characteristics, and potential caregiver impact
- Explore DAYBUE clinical trial design, efficacy, and safety data from the pivotal trial, LAVENDER™, and results from the long-term open-label extension trials, LILAC-1™ and LILAC-2™
- Review management strategies for gastrointestinal adverse events
- Summarize the interim data from the postapproval, observational, ongoing real-world study LOTUS
- Gain insights from patients' experiences with RTT and DAYBUE

This is a promotional program, and no CME credits are offered.

Use the link below or scan the QR code with your device to register for this event.

Enter 450291 at <https://AcadiaPrograms.com>.

Online registration is preferred.

This presentation is available to participate virtually or in-person; please choose preferred method of attendance when registering. Virtual log-in instructions provided after registering. You may also contact Michael Middlebrooks at 210-845-2112 or email Mike.Middlebrooks@Acadia-Pharm.com. Please note: This program is subject to cancellation.

Important Safety Information

• Warnings and Precautions

- **Diarrhea:** In a 12-week study and in long-term studies, 85% of patients treated with DAYBUE experienced diarrhea. In those treated with DAYBUE, 49% either had persistent diarrhea or recurrence after resolution despite dose interruptions, reductions, or concomitant antidiarrheal therapy. Diarrhea severity was mild or moderate in 96% of cases. In the 12-week study, antidiarrheal medication was used in 51% of patients treated with DAYBUE.

Advise patients to stop laxatives before starting DAYBUE. If diarrhea occurs, patients should notify their healthcare provider, consider starting antidiarrheal treatment, and monitor hydration status and increase oral fluids, if needed. Interrupt, reduce dose, or discontinue DAYBUE if severe diarrhea occurs or if dehydration is suspected.

Please see additional Important Safety Information on page 2.

Please read the accompanying full Prescribing Information.



Important Safety Information (cont'd)

• Warnings and Precautions (cont'd)

- **Vomiting:** In a 12-week study, vomiting occurred in 29% of patients treated with DAYBUE and in 12% of patients who received placebo.

Patients with Rett syndrome are at risk for aspiration and aspiration pneumonia. Aspiration and aspiration pneumonia have been reported following vomiting in patients being treated with DAYBUE. Interrupt, reduce dose, or discontinue DAYBUE if vomiting is severe or occurs despite medical management.

- **Weight Loss:** In the 12-week study, 12% of patients treated with DAYBUE experienced weight loss of greater than 7% from baseline, compared to 4% of patients who received placebo. In long-term studies, 2.2% of patients discontinued treatment with DAYBUE due to weight loss. Monitor weight and interrupt, reduce dose, or discontinue DAYBUE if significant weight loss occurs.

- **Adverse Reactions:** The common adverse reactions ($\geq 5\%$ for DAYBUE-treated patients and at least 2% greater than in placebo) reported in the 12-week study were diarrhea (82% vs 20%), vomiting (29% vs 12%), fever (9% vs 4%), seizure (9% vs 6%), anxiety (8% vs 1%), decreased appetite (8% vs 2%), fatigue (8% vs 2%), and nasopharyngitis (5% vs 1%).

• Drug Interactions: Effect of DAYBUE on other Drugs

- DAYBUE is a weak CYP3A4 inhibitor; therefore, plasma concentrations of CYP3A4 substrates may be increased if given concomitantly with DAYBUE. Closely monitor when DAYBUE is used in combination with orally administered CYP3A4 sensitive substrates for which a small change in substrate plasma concentration may lead to serious toxicities.
- Plasma concentrations of OATP1B1 and OATP1B3 substrates may be increased if given concomitantly with DAYBUE. Avoid the concomitant use of DAYBUE with OATP1B1 and OATP1B3 substrates for which a small change in substrate plasma concentration may lead to serious toxicities.

• Use in Specific Population: Renal Impairment

- DAYBUE is not recommended for patients with severe renal impairment.

DAYBUE is available as an oral solution (200 mg/mL).

Please read the accompanying full Prescribing Information, also available at DAYBUEHCP.com.

A meal may be offered. Alcohol will not be provided.

Acadia Pharmaceuticals is pleased to sponsor this program to provide information consistent with industry guidelines. This program is not an accredited CME program and is not designed to meet any training and/or educational requirements. In accordance with the PhRMA Code on Interactions With Health Care Professionals, attendance at this educational program is limited to only health care professionals (physicians, nurse practitioners, physician assistants, RNs, clinical pharmacists, social workers). Accordingly, attendance by guests or spouse is not permitted.

To comply with certain federal, state, and local laws that prohibit or limit the provision of meals to health care professionals and/or state employees, Acadia Pharmaceuticals does not offer a complimentary meal to individuals who are subject to such restrictions. Attendees to whom such restrictions apply should not avail themselves of the complimentary meal. Please note that Acadia Pharmaceuticals is required to report the value of a provided meal pursuant to applicable federal and state laws.

This invitation is non-transferrable.

[Privacy Policy](#)

Reference: 1. Acadia Pharmaceuticals Inc. DAYBUE [Package Insert]. San Diego, CA, 2024.



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