

Frequently Asked Questions



Q What is DAYBUE™ approved to treat?

A DAYBUE is a prescription medicine approved to treat Rett syndrome in adults and children 2 years of age and older. It is not known if DAYBUE is safe and effective in children under 2 years of age.

Q Can boys take DAYBUE?

A Yes, DAYBUE is indicated for the treatment of Rett syndrome in adults and children 2 years of age and older. It is not known if DAYBUE is safe and effective in children under 2 years of age.

Q How do we know that DAYBUE works?

A During a 12-week clinical trial of 187 females ages 5 to 20, treatment with DAYBUE demonstrated improvements in the signs and symptoms of Rett syndrome when compared to placebo. The following signs and symptoms were assessed by the RSBQ scale in the clinical trial: breathing, hand movements or stereotypies, repetitive behaviors, night-time behaviors, vocalizations, facial expressions, eye gaze, and mood. Individual patients may respond differently to DAYBUE treatment. It is important to take DAYBUE as prescribed by your child's doctor.

Important Safety Information:


What are the possible side effects of DAYBUE?

- **Diarrhea:** Diarrhea is a common side effect of DAYBUE that can sometimes be severe. Diarrhea may cause you to lose too much water from your body (dehydration). Before starting treatment with DAYBUE, stop taking laxatives. Tell your healthcare provider if you have diarrhea while taking DAYBUE. Your healthcare provider may ask you to increase the amount you drink or take anti-diarrheal medicine as needed.
- **Weight loss:** DAYBUE can cause weight loss. Tell your healthcare provider if you notice you are losing weight at any time during treatment with DAYBUE.
- **The most common side effects of DAYBUE include diarrhea and vomiting.** Other side effects include fever, seizure, anxiety, decreased appetite, tiredness, and the common cold. These are not all the possible side effects of DAYBUE. Tell your healthcare provider if you have any side effects that bother you or do not go away.


See additional Important Safety Information throughout.

Please read the full [Prescribing Information](#), including [Patient Information](#).

How should I decide if DAYBUE is right for my child?


 When considering a new medication, it's best to discuss it with your doctor. Together, you can determine if DAYBUE is right for your child.

What is Acadia Connect®?


 Acadia Connect is a patient and family support program that connects you and your family with educational support and resources throughout the DAYBUE treatment journey. The team will help you with:

- Understanding and verifying insurance coverage
 - Information on appropriate financial assistance options
 - Support and education throughout the DAYBUE treatment journey
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When will DAYBUE be available?

 DAYBUE was FDA approved on March 10, 2023. By the end of April 2023, DAYBUE will be available by prescription through your doctor. Once you receive your child's prescription for DAYBUE, you'll be connected to the dedicated support team at Acadia Connect. The Acadia Connect team will be at your side every step of the way to support you in starting treatment with DAYBUE.

How can I get access to DAYBUE for my child?

 Once your doctor prescribes DAYBUE for your child, the next step is to enroll in Acadia Connect. This can happen in 1 of 3 ways:

- You can sign the Prescription and Enrollment Form at your doctor's office before they submit the prescription
- If you could not sign the Prescription and Enrollment form, you will need to sign and submit the DAYBUE Patient Consent Form available on the Acadia Connect website
- You can call Acadia Connect at 1-844-737-2223 and a Nurse Care Coordinator will help you enroll in the program

Acadia Connect will work with you to get access as quickly as possible.

Important Safety Information


What should I tell my healthcare provider before taking DAYBUE?

Before taking DAYBUE, tell your healthcare provider about all of your medical conditions, including if you:


- have kidney problems.
- are pregnant or plan to become pregnant. It is not known if DAYBUE will harm your unborn baby.
- are breastfeeding or plan to breastfeed. It is not known if DAYBUE passes into your breast milk. Talk to your healthcare provider about the best way to feed your baby while taking DAYBUE.

See additional Important Safety Information throughout.


How much will DAYBUE cost?

-  We believe cost should never be a barrier to treatment. The cost for DAYBUE will depend on your individual insurance coverage. The Acadia Connect team will confirm your information and help you understand your out-of-pocket insurance cost. If you are unable to afford DAYBUE, Acadia Connect can provide information on appropriate financial assistance options you may be eligible for.
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How can I find out if DAYBUE is covered by my insurance?

-  Acadia Connect is here to help you understand your insurance coverage for DAYBUE. Once enrolled in Acadia Connect, you will be assigned a Nurse Care Coordinator who will verify and help you understand your insurance coverage. If needed, they will provide information on financial assistance options available to you.
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How will my child's DAYBUE prescription be delivered to me?

-  Your DAYBUE prescription can only be filled by a specialty pharmacy. We partner with AnovoRx, our exclusive specialty pharmacy to process and fill prescriptions. Once AnovoRx finalizes your child's prescription details, they will schedule delivery to the location most convenient for you. Acadia Connect will also reach out to you regularly to coordinate shipping refills.
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How do you take DAYBUE?

-  DAYBUE is a strawberry-flavored oral medication that is taken twice daily, in the morning and evening. DAYBUE can be taken with or without food. DAYBUE may be swallowed or given via gastrostomy (G) tube. If your child has a gastrojejunal (GJ) tube, DAYBUE must be given through the G-port. The amount of DAYBUE your child will need to take will depend on their weight. Your child's doctor will instruct you on the proper dose and when to take it. If you miss a dose of DAYBUE, skip that dose and take your next dose at your usual time. Do not take 2 doses to make up the missed dose.
- If you vomit after taking a dose of DAYBUE, do not take another dose to make up for that dose. Wait and take the next dose at your usual time.
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Important Safety Information (continued)

What should I tell my healthcare provider before taking DAYBUE? (continued)


Tell your healthcare provider about all of the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements. Taking DAYBUE with certain medicines may affect how the other medicines work and can cause serious side effects.

See additional Important Safety Information throughout.


My child uses a feeding tube. Can they take DAYBUE?

-  Yes, DAYBUE may be taken orally or via gastrostomy (G) tube. If your child has a gastrojejunal (GJ) tube, DAYBUE must be given through the G-port.
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How does my child transition from an Acadia-sponsored clinical trial to the FDA-approved DAYBUE product?

-  If your child is currently enrolled in an Acadia-sponsored clinical trial, please discuss your desire to transition to FDA-approved DAYBUE with your Clinical Trial Coordinator or doctor. They will begin the process by submitting a Prescription and Enrollment Form to Acadia Connect. Upon enrollment, your dedicated Nurse Care Coordinator will reach out to welcome you to the program, explain the next steps, and introduce you to your Family Access Manager. Together, they will provide support every step of the way.
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Where can I get more information on DAYBUE?

-  For more information on DAYBUE, please visit [DAYBUE.com](https://daybue.com) and speak with your child's doctor to determine if DAYBUE is right for your child.

Join us on March 28, 2023 from 7:00 PM - 8:00 PM ET for our Introducing DAYBUE Webinar for Caregivers. This educational webinar will provide more information about treatment with DAYBUE and support services offered by Acadia Connect. You can register for this upcoming event at DaybueEvents.com.

Important Safety Information

What are the possible side effects of DAYBUE?

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- **Weight loss:** DAYBUE can cause weight loss. Tell your healthcare provider if you notice you are losing weight at any time during treatment with DAYBUE.
- **The most common side effects of DAYBUE include diarrhea and vomiting.** Other side effects include fever, seizure, anxiety, decreased appetite, tiredness, and the common cold. These are not all the possible side effects of DAYBUE. Tell your healthcare provider if you have any side effects that bother you or do not go away.

See additional Important Safety Information throughout.

Is DAYBUE approved outside of the US?

A At this time, DAYBUE is only approved in the US for the treatment of Rett syndrome in adults and children 2 years of age and older. If you have a child who is living with Rett syndrome, we understand your urgency to learn what possible treatments may be available for your child. While Acadia Pharmaceuticals Inc. owns the rights to trofinetide in North America (Canada, Mexico and the US), Neuren Pharmaceuticals owns the rights outside of North America.

If you live in the US:

DAYBUE was FDA approved on March 10, 2023. By the end of April 2023, DAYBUE will be available by prescription through your doctor. Once you receive your child's prescription for DAYBUE, you'll be connected to the dedicated support team at Acadia Connect. The Acadia Connect team will be at your side every step of the way to support you in starting treatment with DAYBUE. You can reach Acadia Connect at 1-844-737-2223.

If you live in Canada:

With the US approval complete, we are in the midst of developing a regulatory and commercial plan to commercialize trofinetide in Canada, and will provide further details on the timeline as we progress. For any additional inquiries, please do not hesitate to contact Acadia Medical Information at 1-844-4ACADIA (1-844-422-2342) Option 1 or medicalinformation@acadia-pharm.com.

If you live in Mexico:

At this time, it is premature for us to comment on plans for Mexico. We will provide updates as soon as we are able.

If you live in any country outside North America:

At this time, if you are seeking information and live outside of North America, please contact Neuren Pharmaceuticals directly at enquiries@neurenpharma.com.

Important Safety Information (continued)

What are the possible side effects of DAYBUE?

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- **Weight loss:** DAYBUE can cause weight loss. Tell your healthcare provider if you notice you are losing weight at any time during treatment with DAYBUE.

See additional Important Safety Information throughout.

What is DAYBUE?

DAYBUE is a prescription medicine used to treat Rett syndrome in adults and children 2 years of age and older. It is not known if DAYBUE is safe and effective in children under 2 years of age.

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What should I tell my healthcare provider before taking DAYBUE?

Before taking DAYBUE, tell your healthcare provider about all of your medical conditions, including if you:

- have kidney problems.
- are pregnant or plan to become pregnant. It is not known if DAYBUE will harm your unborn baby.
- are breastfeeding or plan to breastfeed. It is not known if DAYBUE passes into your breast milk. Talk to your healthcare provider about the best way to feed your baby while taking DAYBUE.

Tell your healthcare provider about all of the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements. Taking DAYBUE with certain medicines may affect how the other medicines work and can cause serious side effects

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call **1-800-FDA-1088**. You can also call Acadia Pharmaceuticals Inc. at 1-844-4ACADIA (1-844-422-2342).

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

Please read the full [Prescribing Information](#), including [Patient Information](#) also available at DAYBUE.com

PATIENT INFORMATION
DAYBUE™ (day-BYOO)
(trofinetide)
oral solution

What is DAYBUE?

- DAYBUE is a prescription medicine used to treat Rett syndrome in adults and children 2 years of age and older.
- It is not known if DAYBUE is safe and effective in children under 2 years of age.

Before taking DAYBUE, tell your healthcare provider about all of your medical conditions, including if you:

- have kidney problems.
- are pregnant or plan to become pregnant. It is not known if DAYBUE will harm your unborn baby.
- are breastfeeding or plan to breastfeed. It is not known if DAYBUE passes into your breast milk. Talk to your healthcare provider about the best way to feed your baby while taking DAYBUE.

Tell your healthcare provider about all of the medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements.

Taking DAYBUE with certain medicines may affect the way other medicines work and can cause serious side effects. Know the medicines you take. Keep a list of them to show your healthcare provider and pharmacist when you get a new medicine.

How should I take DAYBUE?

- Take DAYBUE exactly as your healthcare provider tells you to take it.
- If you take laxatives, stop taking them before starting treatment with DAYBUE.
- Your healthcare provider may change your dose or stop treatment with DAYBUE if needed.
- Your pharmacist should provide an oral syringe or dosing cup that is needed to measure your prescribed dose. **Do not** use a household measuring cup.
- DAYBUE may be taken by mouth or given through a gastrostomy (G) tube. If DAYBUE is given through a gastrojejunal (GJ) tube, the G-port must be used.
- DAYBUE may be taken with or without food.
- Throw away any unused DAYBUE after 14 days of first opening the bottle.
- If you vomit after taking a dose of DAYBUE, **do not take** another dose to make up for that dose. Wait and take the next dose at your usual time.
- If you miss a dose of DAYBUE, skip that dose and take your next dose at your usual time. **Do not** take 2 doses to make up the missed dose.

What are the possible side effects of DAYBUE?

DAYBUE may cause side effects, including:

- **Diarrhea.** Diarrhea is a common side effect of DAYBUE that can sometimes be severe. Diarrhea may cause you to lose too much water from your body (dehydration). Tell your healthcare provider if you have diarrhea while taking DAYBUE. Your healthcare provider may ask you to increase the amount you drink or take anti-diarrheal medicine as needed.
- **Weight loss.** DAYBUE can cause weight loss. Tell your healthcare provider if you notice you are losing weight at any time during treatment with DAYBUE.

The most common side effects of DAYBUE include diarrhea and vomiting.

These are not all the possible side effects of DAYBUE. Tell your healthcare provider if you have any side effects that bother you or do not go away. For more information, ask your healthcare provider or pharmacist. Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

How should I store DAYBUE?

- Store DAYBUE in the refrigerator between 36°F to 46°F (2°C to 8°C). **Do not** freeze.
- Keep DAYBUE in an upright position.
- Keep the child-resistant cap tightly closed.

Keep DAYBUE and all medicines out of the reach of children.

General Information about the safe and effective use of DAYBUE.

Medicines are sometimes prescribed for purposes other than those listed in a Patient Information leaflet. Do not use DAYBUE for a condition for which it was not prescribed. Do not give DAYBUE to other people, even if they have the same symptoms that you have. It may harm them. You can ask your pharmacist or healthcare provider for information about DAYBUE that is written for health professionals.

What are the ingredients in DAYBUE?

Active ingredient: trofinetide

Inactive ingredients: FD&C Red No. 40, maltitol, methylparaben sodium, propylparaben sodium, purified water, strawberry flavor, and sucralose.

Marketed by Acadia Pharmaceuticals Inc., San Diego, CA 92130 USA

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For more information, go to www.daybue.com or call 1-844-422-2342.

This Patient Information has been approved by the U.S. Food and Drug Administration

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