



An introduction to DAYBUE™

We're adding more of her sparkle to the world around us

DAYBUE is the first and only FDA-approved treatment for Rett syndrome

Maddy, age 21, living with **Rett syndrome**, with her mom

Indication

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.



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 antidiarrheal medicine as needed.

See additional Important Safety Information on page 18. Please read the full [Prescribing Information](#) including [Patient Information](#).



An opportunity to help spark meaningful improvements in the signs and symptoms of Rett syndrome

For the loved one you've always known is inside, DAYBUE is the first and only FDA-approved prescription medicine for the treatment of Rett syndrome in adults and children 2 years of age and older.

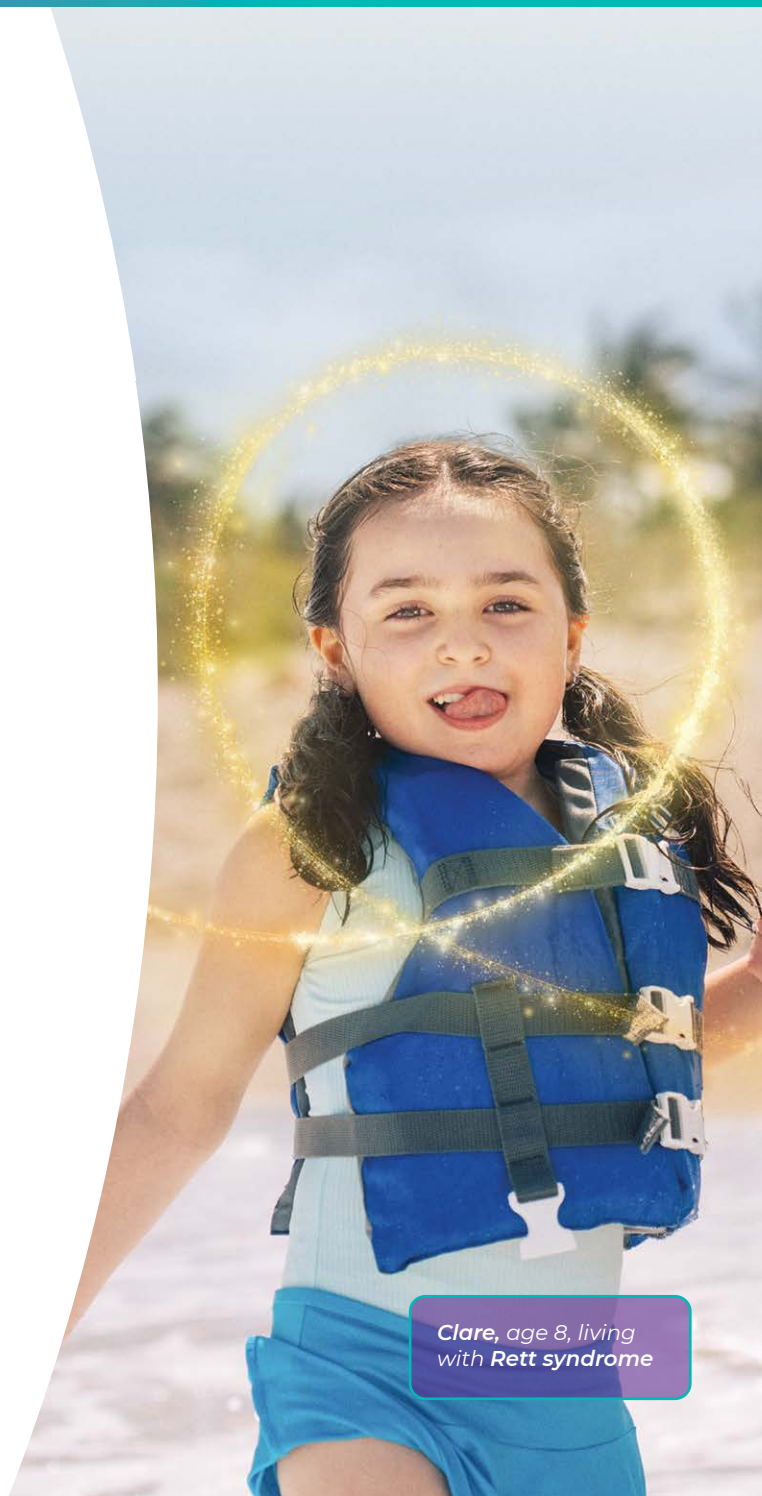
Over 1500 people living with Rett syndrome have started DAYBUE—including people with a variety of Rett symptoms, adults, girls, and boys.

Important Safety Information (continued)

What are the possible side effects of DAYBUE? (continued)

- **Vomiting:** Vomiting is a common side effect of DAYBUE. Sometimes vomit can get into your lungs (aspiration), which could cause an infection (aspiration pneumonia). Tell your healthcare provider if you have severe vomiting or if vomiting happens often.

See additional Important Safety Information on page [18](#).



Clare, age 8, living with Rett syndrome



Henry, age 4, living with Rett syndrome



Kate, age 9, living with Rett syndrome, with her dad



Maddy, age 21, living with Rett syndrome, with her mom

Rett syndrome: A complex genetic disorder with a range of symptoms—but limited treatment options



Rett syndrome is a rare neurodevelopmental disorder. **Your child's experience with Rett syndrome is unique to them**—the symptoms can affect every individual in different ways, at different times, and at different stages of the disorder. However, **Rett syndrome is not neurodegenerative**, meaning that brain cells do not become damaged or die. **Changes in symptoms may be possible at any age.**



As varied as these symptoms can be, your child can also experience unexpected setbacks called regressions. **This may leave you and your family continually seeking new solutions to the ongoing changes in your child.**



Along with a variation in symptoms, the ability to think and understand may also vary among children. Many children with Rett syndrome are cognitively aware but are unable to effectively act on their desire to communicate. This may leave them feeling frustrated and misunderstood.

In Rett syndrome, a majority of cases are caused by a mutation, or change, in the *MECP2* gene.

MECP2=methyl-CpG-binding protein 2.



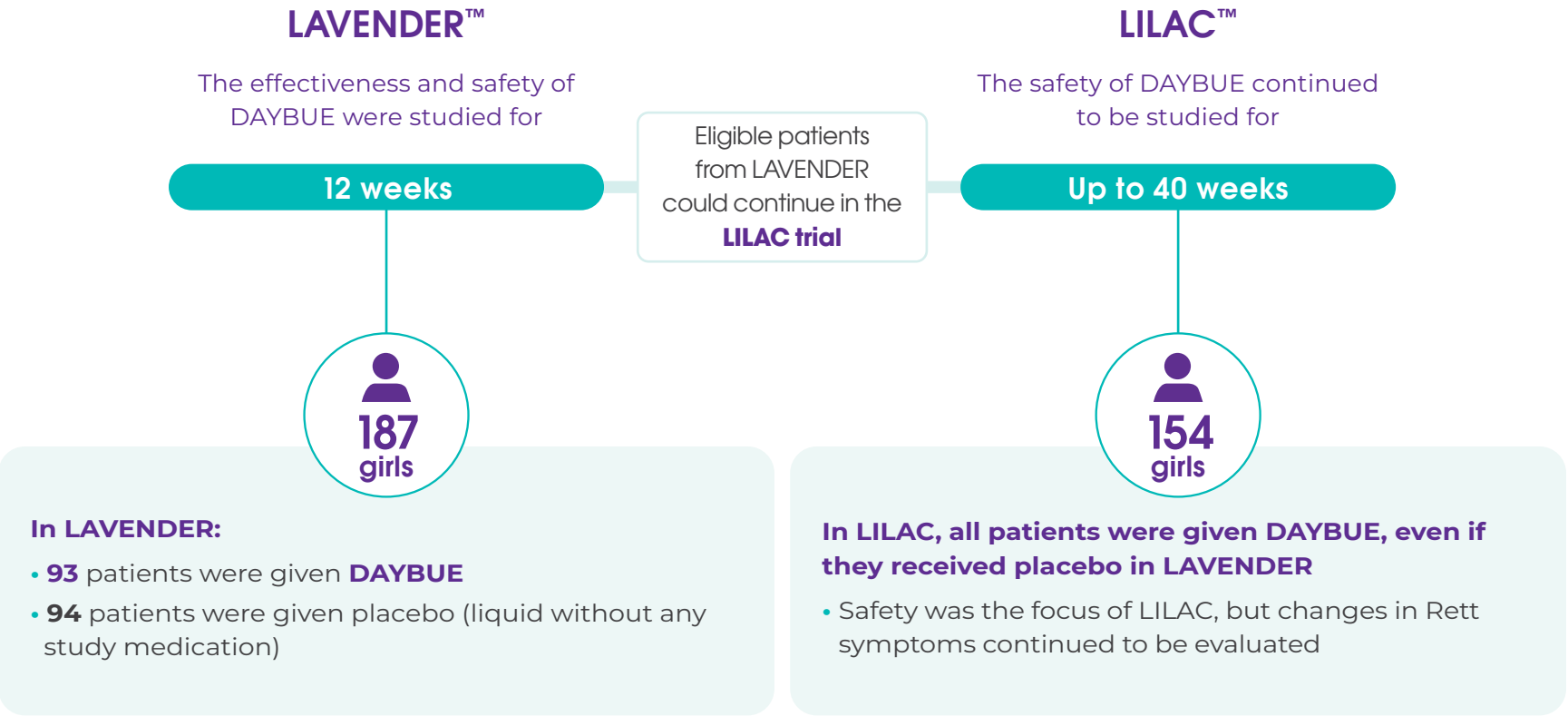
MECP2 is thought to control how many genes work, including those important for brain development.



In Rett syndrome, mutations in *MECP2* are thought to disrupt the activity of these genes which may be associated with symptoms of Rett syndrome.

DAYBUE was studied in the largest clinical trial for Rett syndrome to date

The effectiveness and safety of DAYBUE were evaluated in the 12-week LAVENDER™ clinical trial of 187 females aged 5 to 20 with Rett syndrome



Patients in LAVENDER all had a diagnosis of typical Rett syndrome according to the Rett Syndrome Diagnostic Criteria, and had a documented mutation in the *MECP2* gene. Patients in both groups of the study had a range of symptoms and disease severity, reflecting how unique Rett syndrome is for each person.

Because caregivers possess such a deep knowledge of their child, they contributed directly to the evaluation of the overall effectiveness of DAYBUE. Doctors evaluated if patients improved or got worse, while caregivers looked for changes in the signs and symptoms of Rett syndrome in day-to-day life.



Maddy, age 21, living with Rett syndrome, (center), with her family

Caregivers evaluated changes in a range of symptoms using the Rett Syndrome Behaviour Questionnaire (RSBQ)

RSBQ is a behavior questionnaire completed by caregivers in the clinical trial to evaluate the range of symptoms of Rett syndrome.

What does the RSBQ measure?



Breathing



Hand movements or stereotypies



Repetitive behaviors



Nighttime behaviors



Vocalizations



Facial expressions



Eye gaze



Mood

Important Safety Information (continued)

What are the possible side effects of DAYBUE? (continued)

- **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 on page 18.

Understanding RSBQ

- Caregivers evaluated 45 items on the RSBQ. Each item was scored as not true (0), somewhat or sometimes true (1), or very true or often true (2)
- The scores were added up to determine the **RSBQ total score** across symptoms
- Researchers looked at the change in average RSBQ total score from the start of the trial to measure how DAYBUE impacted the signs and symptoms of Rett at Week 12 when compared to patients receiving placebo

At 12 weeks, caregivers observed:

Significant improvement in the signs and symptoms of Rett syndrome

as measured by average change in RSBQ total score with DAYBUE vs placebo

- At 12 weeks, the group of patients receiving DAYBUE (n=76) saw a decrease of 4.9 in average RSBQ total score from the start of the trial when compared with a decrease of 1.7 in the placebo group (n=85)
- The difference between the two groups was -3.2. A lower score meant signs and symptoms of Rett syndrome were less severe
- Before starting treatment, the average RSBQ total score for the DAYBUE group was 43.7. For the placebo group, the average RSBQ total score before treatment was 44.5
- Results varied between patients in the clinical trial. Individual results may vary with DAYBUE

Important Safety Information (continued)

What are the possible side effects of DAYBUE? (continued)






















- **The most common side effects of DAYBUE were 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 on page 18.



What improvements could DAYBUE help spark?

Caregiver’s description of improvements with DAYBUE:

Symptom areas (RSBQ)	 Quinn 5 years old Taking DAYBUE since 2021	 Clare 8 years old Taking DAYBUE since 2021	 Kate 9 years old Taking DAYBUE since 2021	 Ellie 15 years old Taking DAYBUE since 2020	 Maddy 21 years old Taking DAYBUE since 2020	Symptom areas (RSBQ)
 Hand movements or stereotypes	More control of hands. Can hold objects longer. Not grasping her body and face as much	Hands are more fluid and open, can hold a fork or cup, taps on fridge when hungry	More purposeful hand use. Able to use a spoon and point at and touch her tablet	Quiet, calmer hands. Can hold a cup, fork and grasp and reach for things	Less aggressive hand movements. More control of hands	Hand movements or stereotypes 
 Repetitive behaviors	Teeth grinding much improved				Hardly grinds her teeth anymore	Repetitive behaviors 
 Breathing	Less frequent breath holding and less hyperventilating				Fewer “blue spells” (breath holding)	Breathing 
 Nighttime behaviors		Less frequent night terrors allow her to sleep better			Fewer night terrors and laughing spells—now family gets more sleep	Nighttime behaviors 
 Vocalizations		Increased nonverbal vocalizations, able to let people know when she wants something		She makes a lot of chatty sounds. Says “fun, funny, and food”	No words, but makes more sounds to respond	Vocalizations 
 Facial expressions					Doesn’t stick her tongue out as often	Facial expressions 
 Eye gaze	Can hold her gaze to participate and engage, especially in the classroom	Uses eye gaze more to convey feelings, needs, and wishes. Allows her to show family what she wants for a snack	Able to use her eye-gaze device better to convey wishes	Uses her eyes to say yes or no and to make choices	Makes choices more quickly with her eyes. More of an interest in using her eye-gaze device	Eye gaze 
 Mood	Her moods are more predictable and she cries a lot less	Less screaming and tantrums. Able to take an interest in the things around her	More peaceful at home, in school, and in groups	She’s able to regulate her emotions a little better. She’s happier than she used to be	Mood is better, happier, much less crying for no apparent reason	Mood 

Important Notes:

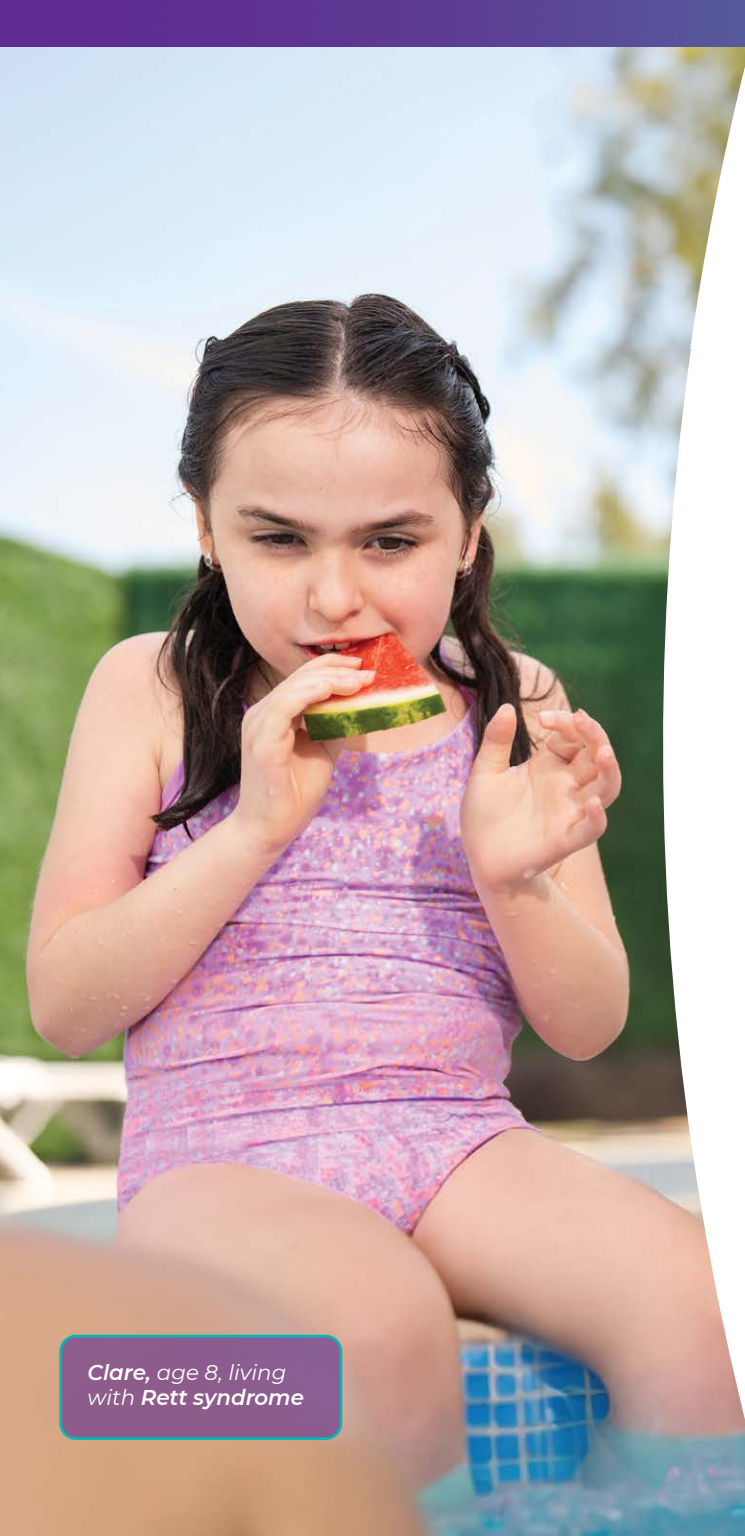
- **Not all patients in the clinical trial responded to treatment with DAYBUE. These patients were also receiving other types of therapy, like physical, occupational, or speech therapy. See pages 10 to 15 for more details**
 - **The families of these patients were compensated for participating in photo shoots and sharing their stories. These stories were created in 2023 and 2024**
- **In the clinical trial, some of these patients experienced side effects, including diarrhea, behavioral issues, or gas. See pages 10 to 15 to learn more about their DAYBUE experience**
 - **Always speak with a healthcare provider to learn what to expect when starting DAYBUE, including side effects**

Important Safety Information (continued)

What are the possible side effects of DAYBUE? (continued)

Diarrhea and vomiting are the most common side effects of DAYBUE that can sometimes be severe. DAYBUE can also cause weight loss. Other possible side effects include fever, seizure, anxiety, decreased appetite, tiredness, and the common cold.

See additional Important Safety Information on page 18.



Clare, age 8, living with Rett syndrome

At 12 weeks, doctors observed:



Significant improvements in the signs and symptoms of Rett syndrome in patients taking DAYBUE vs placebo

The CGI-I (Clinical Global Impression-Improvement) scale was used by doctors to evaluate improvement or worsening of Rett symptoms

- 38% of patients (n=77) had improvement in symptoms with DAYBUE vs 15% of patients (n=86) taking placebo
- 61% of patients had no improvements in symptoms with DAYBUE vs 81% of patients taking placebo. Individual results may vary with DAYBUE

How soon are improvements seen with DAYBUE?

In LAVENDER, caregivers and doctors saw improvements in the signs and symptoms of Rett syndrome as soon as 12 weeks after starting DAYBUE when compared to placebo. Not all patients in the study responded to treatment with DAYBUE. Individuals results may vary.

The time to treatment response may vary from person to person. It may take time to see improvements in symptoms. Work closely with your doctor to understand your child's baseline symptoms and how to track them over time.



Tips for tracking signs and symptoms of Rett syndrome:

- Write down signs and symptoms before starting DAYBUE
- Ask everyone in your child's care team to notice and report any changes
- Track hand movements, eye gaze, nighttime behaviors—any aspect of your child's life that is impacted by Rett syndrome
- Notice impact of even small changes

Important Safety Information (continued)

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.

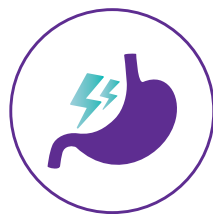
See additional Important Safety Information on page 18.

Understanding the possible side effects of DAYBUE

As with any medication, it's important to talk to your child's doctor about the possibility of side effects with DAYBUE. Not every person will experience the same side effects, and for some people, certain side effects, such as diarrhea and vomiting, may be more severe than others. **Side effects may include:**



Diarrhea: Diarrhea is a common side effect of DAYBUE that can sometimes be severe. Diarrhea may cause your child to lose too much water from their body (dehydration). Before starting treatment with DAYBUE, stop taking laxatives. Tell your doctor if your child has diarrhea while taking DAYBUE. Your doctor may ask you to increase the amount your child drinks or give antidiarrheal medicine as needed.



Vomiting: Vomiting is a common side effect of DAYBUE. Sometimes vomit can get into your child's lungs (aspiration), which could cause an infection (aspiration pneumonia). Tell your doctor if your child has severe vomiting or if vomiting happens often.



Weight loss: DAYBUE can cause weight loss. Tell your doctor if you notice your child is losing weight at any time during treatment with DAYBUE.

See additional Important Safety Information on page 18.

Common side effects in the 12-week LAVENDER trial

Side Effects	DAYBUE (% of 93 patients)	Placebo (% of 94 patients)
Diarrhea	82%	20%
Vomiting	29%	12%
Fever	9%	4%
Seizure	9%	6%
Anxiety	8%	1%
Decreased appetite	8%	2%
Tiredness	8%	2%
Common cold	5%	1%

See page 9 for helpful tips for managing diarrhea.

Preparing
for potential
GI side
effects
before
starting
DAYBUE

Before your child starts treatment, talk to your doctor about a management plan to address the possible side effects while on treatment with DAYBUE.

Many people with Rett syndrome have gastrointestinal (GI) issues like constipation or reflux. If you are used to managing constipation, diarrhea may be new to you.

Here are some tips before your child starts DAYBUE:

- ✓ Tell your doctor about any existing GI issues like vomiting, swallowing difficulty, or acid reflux to make a management plan
- ✓ Keep a log to track stool consistency/frequency for 1 week before starting treatment
- ✓ Tell your doctor if you are giving your child stool softeners and other medicines or dietary supplements for the treatment of constipation
- ✓ Stop use of laxatives
- ✓ Work with your doctor to form a management plan that addresses diarrhea and learn about any recommended dietary changes including potential use of supplemental fiber

Starting DAYBUE if diarrhea is a concern:

- Based on your doctor’s clinical judgment and experience, they may decide to start with a lower dose and increase to the FDA-recommended weight-based dose over time
- Improvements may not occur until your child reaches the recommended dose and continues treatment
- The impact of this approach on diarrhea was not studied in LAVENDER. Individual patient response may vary

The LAVENDER study findings were based on starting patients at the FDA-recommended weight-based dose of DAYBUE and continuing treatment for 12 weeks.



If diarrhea occurs:

- ✓ Tell your doctor if your child has diarrhea
- ✓ Keep an eye on your child’s fluid intake and watch for signs of dehydration; increase oral fluids, if needed. Your doctor may also suggest a fiber supplement, if appropriate
- ✓ It is recommended that you have antidiarrheal medications such as loperamide (IMODIUM®) at home so you can manage diarrhea as needed
- ✓ Your doctor may reduce the dose of DAYBUE or may ask you to stop giving your child DAYBUE until the diarrhea improves. Do not stop giving DAYBUE before talking to your doctor



General tips to manage vomiting:

- ✓ Tell your doctor if your child has vomiting. Your doctor may suggest they take medication to help manage vomiting
- ✓ Have meals somewhere calm. Your child’s stomach can be sensitive to stress. Finding somewhere relaxed to eat can reduce the likelihood of vomiting
- ✓ Avoid meals before bedtime or nap time
- ✓ Keep a meal journal. A journal can help you track connections between certain foods and vomiting. By removing foods one-by-one you can figure out if a particular food is causing vomiting
- ✓ Talk to your doctor if vomiting does not stop

This is not a complete list of ways to manage vomiting. Talk to your doctor about making a plan that is tailored to your child’s unique needs.

Important Safety Information (continued)

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 antidiarrheal medicine as needed.

See additional Important Safety Information on page 18.



Clare’s family has seen meaningful improvements in the signs and symptoms of Rett syndrome since starting DAYBUE



Hand movements or stereotypies

Clare’s hands are more fluid and open. She can even hold her own ice cream cone



Nighttime behaviors

Clare has night terrors less often, which allows her to sleep better



Vocalizations

Clare is vocalizing more and she is babbling more to let people know she wants something



Eye gaze

Clare’s eye gaze has improved, which allows her to convey feelings, needs, and wishes



Mood

Clare is able to take an interest in the things around her. She also is screaming less and has fewer tantrums

Important Safety Information (continued)

What are the possible side effects of DAYBUE? (continued)

- **Vomiting:** Vomiting is a common side effect of DAYBUE. Sometimes vomit can get into your lungs (aspiration), which could cause an infection (aspiration pneumonia). Tell your healthcare provider if you have severe vomiting or if vomiting happens often.

See additional Important Safety Information on page 18.



Diagnosis and symptoms

Clare was diagnosed with Rett syndrome at age 4. **Some of her signs and symptoms have included:**

- Difficulty controlling her hands—her mom says “her hands would get in the way of themselves”
- Loss of communication abilities
- Frequent screaming spells and night terrors

Other treatments

Attends occupational therapy, physical therapy, speech therapy, horse therapy, swim lessons, gymnastics classes; sees a physiatrist for muscle tone checks; wears shoe inserts; sees a gastroenterologist and nutritionist.

Managing side effects

After starting DAYBUE, Clare began experiencing behavioral outbursts and anger. She did not want to eat, didn’t sleep, and cried frequently. Clare’s parents worked with her doctor on how to manage these side effects. Clare’s dose was lowered, and her mood improved. Clare’s parents have been working with their doctors to increase the dose slowly. **The goal is to get back to the recommended dose.**

*Clare’s eye gaze has improved. **She looks right at me, which is amazing...She’s using her eyes more to convey her feelings, needs, and wishes.***

– Allison, Clare’s mom

Important Note: The effectiveness and safety of DAYBUE were evaluated in the 12-week LAVENDER™ clinical trial of 187 female patients aged 5 to 20 with Rett syndrome. Because each individual with Rett syndrome is unique—with a unique set of symptoms—the response to DAYBUE may be different for everyone. See the results from the clinical trial. Clare’s family was compensated for sharing their story and participating in a photo shoot. This story was created in March 2024.



Clare, age 8, living with Rett syndrome, with her mom



Ellie’s family has seen meaningful improvements in the signs and symptoms of Rett syndrome since starting DAYBUE



Hand movements or stereotypes

Ellie’s hands are calmer, she can use two hands to hold and carry things, like cups



Vocalizations

Ellie can use some words to express her feelings, needs, and wishes



Eye gaze

Ellie uses her eyes to convey yes or no and to make choices



Mood

Ellie is able to better regulate her emotions. She is happier than she used to be

Important Safety Information (continued)

What are the possible side effects of DAYBUE? (continued)

- **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 on page 18.



Diagnosis and symptoms

Ellie received her Rett syndrome diagnosis at age 7. **Some of her signs and symptoms have included:**

- Loss of communication abilities
- Repetitive hand movements
- Behavioral issues and outbursts

Other treatments

Ellie has been on different therapies since she was 2 years old, including speech therapy and yoga. Since her Rett syndrome diagnosis, she’s participated in occupational, horse, swim, massage, and regular physical therapy.

Managing side effects

After starting DAYBUE, Ellie began experiencing gas, and her doctor prescribed additional medication to help. Ellie’s family works closely with her doctor about any changes or side effects they notice.

*With DAYBUE, words didn’t happen right away. **But now, she says words like “funny,” “fun,” and “food” and is making new noises. She is vocalizing more with intent.***

– Heidi, Ellie’s mom

[Click here to watch Ellie's story](#)

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Ellie, age 15, living with Rett syndrome, (center), with her family



Henry, age 4, living with Rett syndrome

Henry’s family has seen meaningful improvements in the signs and symptoms of Rett syndrome since starting DAYBUE



Hand movements or stereotypies

Henry has more intentional hand movement and can hold a weighted spoon with assistance



Repetitive behaviors

Henry grinds his teeth and taps his fingers less



Vocalizations

Henry has a greater variety of vocalizations, especially when he wants something. When he sees a soccer ball, he says, “ba ba ba”



Eye gaze

Henry has been able to use his eyes to make progress with his eye-gaze device to communicate



Mood

Henry is less anxious, calmer, and yells less. He can sit calmly at story time and restaurants

Important Safety Information (continued)

What are the possible side effects of DAYBUE? (continued)

- **The most common side effects of DAYBUE were 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 on page 18.



Diagnosis and symptoms

Henry was diagnosed with Rett syndrome when he was almost 2 years old.

Some of his signs and symptoms have included:

- Failure to reach developmental milestones
- Repetitive hand movements and behaviors
- Loss of communication skills
- Bursts of anxiety and irritability
- Tremor

Other treatments

Henry has a comprehensive treatment plan developed by his neurologist. It includes physical and occupational therapy five times a week at the school he attends for children with complex needs. He also uses an eye-gaze communication device.

Managing side effects

For the first few weeks after starting DAYBUE, Henry experienced diarrhea. Then, with guidance from their doctor, Henry’s family decided to take a wait-and-see approach while adjusting his diet to include more bananas. Henry’s diarrhea is managed with the help of their doctor.

DAYBUE has helped Henry get to a place where **he can benefit more from his therapies.** The improvements we’ve seen have helped him participate more and get the most out of his school experience.

– Carrie, Henry’s mom

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Kate, age 9, living with Rett syndrome

Kate’s family has seen meaningful improvements in the signs and symptoms of Rett syndrome since starting DAYBUE



Hand movements or stereotypes

Kate is able to use a spoon and point at and touch her tablet



Eye gaze

Kate is better able to use her eye-gaze device to convey her wishes and needs



Mood

Her parents describe her as more peaceful—at home, at school, and in groups

Since being on DAYBUE, Kate is happier and more content going to events with us as a family. She’s able to sit there and enjoy her day.

– Jen, Kate’s mom

Important Safety Information (continued)

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 antidiarrheal medicine as needed.

See additional Important Safety Information on page 18.



Diagnosis and symptoms

Kate was diagnosed with Rett syndrome at age 3. **Some of her signs and symptoms have included:**

- Repetitive hand behaviors
- Loss of purposeful hand use
- Loss of communication abilities

Other treatments

No daily medications to manage symptoms; attends occupational therapy to strengthen hand function, as well as speech and physical therapy.

Managing side effects

Shortly after starting DAYBUE, Kate started having diarrhea. While it doesn’t happen every day, when it does occur, it can require multiple diaper changes and can happen multiple times a day. This was a different experience for her family, as Kate struggled with constipation before the trial and sometimes had to take laxatives. **When the diarrhea first started, Kate’s parents worked with her doctor to come up with strategies for managing the diarrhea.** In consultation with her doctor, Kate’s parents stopped giving her laxatives and instead started an antidiarrheal every day, which has helped make her bowel movements more solid.

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Maddy’s family has seen meaningful improvements in the signs and symptoms of Rett syndrome since starting DAYBUE



Hand movements or stereotypes

Maddy’s hands are less aggressive and she has more control of them



Repetitive behaviors

Maddy hardly sticks her tongue out and grinds her teeth less



Breathing

Maddy’s breath holding has improved significantly



Nighttime behaviors

Maddy has fewer night terrors and laughing spells, allowing her to sleep better



Vocalizations

Maddy still has no words, but vocalizes more when someone talks to her or asks her questions



Facial expressions

Maddy’s face is calmer and more relaxed. She doesn’t grimace as much



Eye gaze

Maddy is able to make quicker choices with her eyes (such as choosing what she wants to wear)



Mood

Maddy has experienced less anxiety in crowds and is happier

Important Safety Information (continued)

What are the possible side effects of DAYBUE? (continued)

- **Vomiting:** Vomiting is a common side effect of DAYBUE. Sometimes vomit can get into your lungs (aspiration), which could cause an infection (aspiration pneumonia). Tell your healthcare provider if you have severe vomiting or if vomiting happens often.

See additional Important Safety Information on page 18.



Diagnosis and symptoms

Maddy was diagnosed with Rett syndrome at age 3—just a few years after the discovery of the *MECP2* gene. **Some of her signs and symptoms have included:**

- Loss of purposeful hand use
- Breath holding
- Loss of communication abilities
- Waking up in the middle of the night with laughing spells or night terrors

Other treatments

Maddy attends physical, occupational, speech, massage, and music therapy.

Managing side effects

Maddy has had some reflux and vomiting with DAYBUE. Maddy’s parents told her doctor the first time it happened and together, **they developed a plan to help manage vomiting.** With this plan in place, her parents have felt this side effect has been manageable for Maddy.

We are happy with the breathing improvements we have noticed since starting DAYBUE.

– Amy, Maddy’s mom

[Click here to watch Maddy’s story](#)

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Maddy, age 21, living with Rett syndrome, (right), with her dad and brother



Quinn, age 5, living with Rett syndrome

Quinn’s family has seen meaningful improvements in the signs and symptoms of Rett syndrome since starting DAYBUE



Hand movements or stereotypies

Quinn has more control of her hands and she grabs at her face and body less



Repetitive behaviors

Quinn’s teeth grinding has improved



Breathing

Quinn still holds her breath occasionally, has fewer “Rett episodes,” and hyperventilates less



Eye gaze

Quinn can hold her gaze to participate and engage, especially in the classroom and in her therapies



Mood

Quinn’s happier, more predictable mood has allowed her family to go out more. She’s more willing to participate in therapies

Important Safety Information (continued)

What are the possible side effects of DAYBUE? (continued)

- **Vomiting:** Vomiting is a common side effect of DAYBUE. Sometimes vomit can get into your lungs (aspiration), which could cause an infection (aspiration pneumonia). Tell your healthcare provider if you have severe vomiting or if vomiting happens often.

See additional Important Safety Information on page 18.



Diagnosis and symptoms

Quinn was diagnosed with Rett syndrome when she was 15 months old.

Some of her signs and symptoms have included:

- Breath holding
- Repetitive behaviors (teeth grinding)
- Loss of purposeful hand use

Other treatments

At school, Quinn participates in physical, speech, and occupational therapy. Outside of school, Quinn does additional physical therapy, oral motor therapy, and swim therapy.

Managing side effects

After starting DAYBUE, Quinn began experiencing diarrhea. Some days, it occurs once or twice, other days as much as seven times. Quinn’s parents worked with their doctor on different tactics to help manage the diarrhea.

The improvements in Quinn’s symptoms, especially in her mood, have given us more confidence to have more flexibility as a family. Before DAYBUE, we didn’t go to many places. Now we can all go to parties, family cookouts, and our other kids’ sports events.

– Lindsay, Quinn’s mom

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Considering DAYBUE?

Talk to your doctor to see if DAYBUE may be right for your child

Important Safety Information (continued)

What are the possible side effects of DAYBUE? (continued)

- **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 were 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 on page [18](#).



A few facts when considering treatment with DAYBUE



DAYBUE is an oral medication that should be taken **twice daily**, morning and evening



DAYBUE is a **strawberry flavored** liquid formula that can be swallowed or given through a gastrostomy (G) tube



DAYBUE can be taken **with or without food**



If your child **vomits** after taking a dose of DAYBUE, do not give them another dose to make up for that dose. Wait and give them the next dose at their usual time. Call your doctor if vomiting does not stop



If your child **misses a dose** of DAYBUE, skip that dose and give the next dose at their usual time. Do not give two doses to make up the missed dose



If your child has a **gastrojejunal (GJ) tube**, DAYBUE must be given through the G-port



DAYBUE should be **stored upright in the refrigerator** before and after opening



Discard any unused DAYBUE after 14 days of opening the bottle



DAYBUE will be **shipped directly to your home** via a specialty pharmacy



Dedicated support for DAYBUE is available through Acadia Connect®

Educational support for families living with Rett syndrome

Whether you're considering treatment with DAYBUE or still trying to understand a Rett syndrome diagnosis, you're bound to have questions. **Patient and Community Educators** can help you better understand Rett and how DAYBUE may help.



Education that fits your schedule

Patient and Community Educators can help you to learn more about Rett syndrome and DAYBUE treatment, and can provide you all the time you need to discuss the facts and answer your questions.

Patient and Community Educators can meet with you one-on-one either virtually or in-person. Connect with a Patient and Community Educator for a partner in navigating your family's Rett syndrome journey.

Patient and Community Educators can't provide medical advice or treatment recommendations—those are decisions for you and your doctor. But your Patient and Community Educator helps you be **better prepared to have those discussions.**

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 antidiarrheal medicine as needed.

See additional Important Safety Information on page 18.



Your Patient and Community Educator can:

- ✓ Help you understand the details of Rett syndrome, comprehensive care, and information regarding DAYBUE, including possible side effects
- ✓ Provide education to others in your community, such as teachers and school nurses, on Rett syndrome and your child's unique needs
- ✓ Keep you informed on new learning resources, events, and more
- ✓ Connect you with other Rett caregivers through the DAYBUE Connect program
- ✓ The decision to pursue DAYBUE treatment is always between you and your doctor. If you do move forward, your Patient and Community Educator will help you transition to a member of our Acadia Connect team for ongoing treatment support

Ready to connect? Schedule an online meeting to start the conversation with your Patient and Community Educator. Once you're connected, future talks can happen online, by phone, or in person—whatever you prefer.

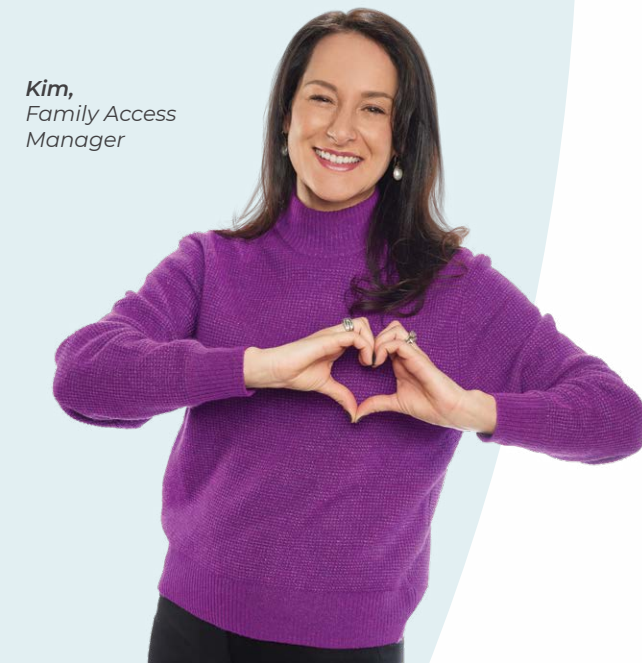
Book now at [DAYBUE.com](https://www.daybue.com)

Cheryl,
Patient and
Community Educator

Elisa,
Patient and
Community Educator

Support by your side

Once your child is prescribed DAYBUE, you'll have the team at Acadia Connect to help guide you through the treatment journey. Acadia Connect is a patient and family support program that connects you and your family with educational support and resources.



*Kim,
Family Access
Manager*

acadia
connect®

Acadia Connect team members are ready to help

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

Acadia Connect consists of a dedicated, experienced support team:

Nurse Care Coordinator who welcomes you to Acadia Connect and provides ongoing support throughout the DAYBUE treatment journey

Family Access Manager who helps you understand your insurance coverage, provides information about DAYBUE, and delivers education about potential side effects, tools, and resources for you and your child who is prescribed DAYBUE

Visit AcadiaConnect.com or call 1-844-737-2223, Monday–Friday, 8:00AM to 8:00PM ET to learn more about how Acadia Connect can provide you and your child with support throughout the DAYBUE treatment journey.

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 antidiarrheal medicine as needed.
- **Vomiting:** Vomiting is a common side effect of DAYBUE. Sometimes vomit can get into your lungs (aspiration), which could cause an infection (aspiration pneumonia). Tell your healthcare provider if you have severe vomiting or if vomiting happens often.
- **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 were 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.

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 (200 mg/mL).

The risk information provided here is not comprehensive. To learn more, talk with your healthcare provider and read the full [Prescribing Information](#), including [Patient Information](#), also available at DAYBUE.com.

See what meaningful improvements DAYBUE may help spark in your child

Over 1500 people living with Rett syndrome have started DAYBUE



Proven effective

By 12 weeks, treatment with DAYBUE demonstrated improvements in signs and symptoms of Rett syndrome, as measured by both caregivers and doctors



Twice-daily dosing

DAYBUE is a strawberry flavored oral medication taken twice a day by mouth or G/GJ tube



Common side effects

Diarrhea and vomiting are the most common side effects with DAYBUE. Your doctor can provide tips and strategies to help



Dedicated patient support

Once your child is prescribed DAYBUE, Acadia Connect is here to provide you with dedicated support and resources throughout the treatment journey



Visit the [Sparkle Gallery](#), where caregivers are sharing the improvements DAYBUE has helped spark for their child

Important Safety Information

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).

See additional Important Safety Information on page 18. Please read the full [Prescribing Information](#), including [Patient Information](#), also available at DAYBUE.com.



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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 gastrojejun (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. Call your healthcare provider if your vomiting does not stop.
- 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 antidiarrheal 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.
- **Vomiting.** Vomiting is a common side effect of DAYBUE. Sometimes vomit can get into your lungs (aspiration), which could cause an infection (aspiration pneumonia). Tell your healthcare provider if you have severe vomiting or if vomiting happens often.

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

DAYBUE is a trademark of Acadia Pharmaceuticals Inc.

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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

Approved 9/2024

DAY-0169-v2