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Daybu (*trofinetide*)

A plain-language overview of Daybu and why it is authorised in the EU

What is Daybu and what is it used for?

Daybu is a medicine used for the treatment of neurobehavioural symptoms of Rett syndrome in adults and children aged 5 years and older. Rett syndrome is a disorder that affects the way the brain develops. It affects mainly girls and women and leads to intellectual disability as well as loss of speech and previously learned skills between 6 and 18 months of age.

Neurobehavioural symptoms in Rett syndrome include repetitive hand movements, restlessness, general mood problems, anxiety, sleep and communication problems.

Rett syndrome is rare, and Daybu was designated an 'orphan medicine' (a medicine used in rare diseases) on 10 August 2015 for the treatment of Rett syndrome. Further information on the orphan designation can be found on the Agency's website: ema.europa.eu/en/medicines/human/orphan-designations/eu-3151534.

Daybu contains the active substance trofinetide.

How is Daybu used?

Daybu can only be obtained with a prescription.

It is available as a liquid to be drunk or given via a gastric (through the stomach) feeding tube twice a day.

For more information about using Daybu, see the package leaflet or contact your doctor or pharmacist.

How does Daybu work?

Insulin-like growth factor 1 (IGF-1) is a hormone that is important for the normal development and functioning of the nervous system. In Rett syndrome, levels of IGF-1 in the brain are lower than normal, which is thought to affect nerve function.

The active substance in Daybu, trofinetide, is made up of a molecule derived from IGF-1. The way that trofinetide works in Rett syndrome is unclear.

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What benefits of Daybu have been shown in studies?

A main study showed that Daybu treatment can improve neurobehavioral symptoms of people with Rett syndrome.

The study involved 187 girls from 5 years of age and women with Rett syndrome who received either Daybu or placebo (a dummy treatment) every day for 12 weeks. The main measures of effectiveness were changes in patients' scores on two standard scales: the Rett syndrome behaviour questionnaire (RSBQ) scale, which measures a patient's behavioural, emotional and physical symptoms based on their caregiver's responses, and the clinical global impression of improvement (CGI-I) scale, which measures a patient's overall health improvement as observed by their clinician.

The total possible score on RSBQ is 90 with higher scores indicating more problems with behaviour and emotions. On the RSBQ scale, the score dropped by 4.9 points on average with Daybu compared with a reduction of 1.7 points on average with placebo.

The CGI-I scale measures overall health improvement, including neurobehavioral symptoms, on a 7-point scale, where a score of 1 indicates that the patient's condition is very much improved since treatment initiation and a score of 7 indicates that the patient's condition is very much worse. After 12 weeks, the average CGI-I score was 3.5 with Daybu compared with 3.8 with placebo.

Studies carried out with Daybu are described in more detail in the medicine's assessment report.

What are the side effects and restrictions with Daybu?

For the full list of side effects and restrictions with Daybu, see the package leaflet.

The most common side effects with Daybu include diarrhoea, which may affect 4 out of 5 people, and vomiting which may affect 1 in 4 people. Weight loss is also very common and may affect more than 1 in 10 people.

Why is Daybu authorised in the EU?

Although the main study on Daybu does not provide evidence on some of the key symptoms of Rett syndrome, such as seizures, the data from the RSBQ and CGI-I scales showed some improvement in neurobehavioural symptoms. The Agency considered that although these improvements were small, they were relevant in the context of a rare and serious disorder such as Rett syndrome.

In terms of safety, Daybu causes diarrhoea and vomiting; although these side effects are generally not serious, they occur frequently which may lead to patients stopping treatment. Daybu also causes weight loss, which could affect the growth of young patients with Rett syndrome. The product information for Daybu includes information on these side effects to support their management.

The European Medicines Agency decided that Daybu's benefits are greater than its risks and it can be authorised for use in the EU.

What measures are being taken to ensure the safe and effective use of Daybu?

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Daybu have been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Daybu are continuously monitored. Suspected side effects reported with Daybu are carefully evaluated and any necessary action is taken to protect patients.

Other information about Daybu

Daybu received a marketing authorisation valid throughout the EU on 20 August 2026.

Further information on Daybu, including the package leaflet and assessment report, can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/daybu.

For information about the availability of this medicine in your country, contact your national competent authority.

This overview was last updated in 08-2026.