25 June 2026
EMADOC-1829012207-57009
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion[1] (initial authorisation)

Daybu

trofinetide

On 25 June 2026, the Committee for Medicinal Products for Human Use (CHMP), following a re-examination procedure, adopted a positive opinion recommending the granting of a marketing authorisation for the medicinal product Daybu[2], intended for the treatment of neurobehavioural symptoms of Rett syndrome.

The applicant for this medicinal product is Acadia Pharmaceuticals (Netherlands) B.V.

Daybu will be available as a 200 mg/ml oral solution. The active substance of Daybu is trofinetide, which belongs to the group of other nervous system drugs (ATC code: N07XX24). Trofinetide is a synthetic analogue of the N-terminal tripeptide of insulin-like growth factor 1. Its mechanism of action in the treatment of Rett syndrome is not fully understood.

The benefits of Daybu are primarily based on the pivotal study 003, which was a 12-week, placebo-controlled study. Statistically significant improvements were observed in the co-primary and key secondary endpoints. Efficacy was demonstrated for behavioural aspects of Rett syndrome and on the Clinical Global Impression-Improvement (CGI-I) scale. Although the effect sizes were small, their clinical relevance was considered acceptable based on the totality of data. The most common side effects with Daybu include diarrhoea, vomiting and weight loss.

The full indication is:

> Daybu is indicated for the treatment of neurobehavioural symptoms of Rett syndrome in adults and paediatric patients aged 5 years and older.

Daybu should be prescribed by physicians experienced in the treatment of Rett syndrome.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published on the EMA website in all official European Union languages after the marketing authorisation has been granted by the European Commission.

[1] Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion.

[2] This product was designated as an orphan medicine during its development. EMA will now review the information available to date to determine if the orphan designation can be maintained.

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union