



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Efmody (*hydrocortisone*)

An overview of Efmody and why it is authorised in the EU

What is Efmody and what is it used for?

Efmody is a medicine used to treat an inherited condition called congenital adrenal hyperplasia (CAH) in patients 12 years old and above.

CAH is rare, and Efmody was designated as an 'orphan medicine' (a medicine used in rare diseases) on 27 July 2005. Further information on the orphan designation can be found here: ema.europa.eu/medicines/human/orphan-designations/eu305296.

Efmody contains the active substance hydrocortisone and is a 'hybrid medicine'. This means that it is similar to a 'reference medicine' containing the same active substance, but Efmody has a different use and is available in different strengths as capsules formulated to release the active substance over a prolonged period. The reference medicine for Efmody capsules is Hydrocortone tablets.

How is Efmody used?

Efmody is available as modified-release capsules and can only be obtained with a prescription. Treatment should be started by a doctor experienced in the management of CAH.

The recommended dose of Efmody in adults and adolescents who have finished growing is 15 to 25 mg daily. In adolescents who have not finished growing, the dose is based on their weight and height. The daily dose can be adjusted as necessary based on the individual response. Two-thirds to three-quarters of the daily dose is taken in the evening at bedtime, at least 2 hours after the last meal, and the rest in the morning at least 1 hour before a meal.

Patients may have to take an additional hydrocortisone medicine during periods of mental or physical stress including surgery and infections.

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

How does Efmody work?

People with CAH are unable to make enough of the natural corticosteroid hormone cortisol (and sometimes another hormone, aldosterone). Cortisol is normally made in the adrenal glands (small glands just above the kidneys) and helps regulate other hormones and the body's response to stress

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as well as the balance of salts and water in the body. They also suffer an increase in male sex hormones that can result in growth and fertility problems.

Efmody contains hydrocortisone, a synthetic form of cortisol, and slowly releases it in the intestines to replace the natural hormone in the body, in a pattern similar to natural daily secretion of cortisol. This helps to restore a more normal hormone balance and minimise other aspects of the condition.

What benefits of Efmody have been shown in studies?

The benefits of Efmody have been shown in a main study involving 122 patients with CAH. Efmody was compared with standard treatment involving other corticosteroid medicines. The main measure of effectiveness was a score based on levels of 17-OHP, a hormonal substance that indicates increased male sex hormones in uncontrolled CAH. A fall in this score over the course of the study showed better control. Over the 24 weeks of the study this score fell by 0.403 in patients treated with Efmody, compared with 0.172 in those given standard treatment. Although this difference was not sufficient to clearly show that Efmody worked better than standard treatment, measurements also suggested a better control of morning 17-OHP levels.

Supportive data from an ongoing continuation study indicated that control of CAH could be maintained with Efmody longer term.

What are the risks associated with Efmody?

The most common side effect with Efmody (which may affect more than 1 in 10 people) is tiredness. Other common side effects with Efmody (which may affect up to 1 in 10 people) are headache, increased appetite, dizziness and weight gain. The most common serious effect is acute adrenal insufficiency (when a corticosteroid medicine cannot supply enough hydrocortisone to respond to increased cortisol needs during stress or infection, which may be shown by vomiting or feeling very unwell).

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

Why is Efmody authorised in the EU?

Efmody provided adequate control of CAH and the overall data suggested an improved hormone balance. The long-term data suggested this could be maintained, in some cases using lower doses of corticosteroid than before and thus reducing the risk of side effects from long-term treatment. Modified-release hydrocortisone was considered to offer clinical value by allowing dosing that resembles the daily rhythm of natural cortisol secretion. The reported side effects of Efmody are in line with those expected for hydrocortisone taken by mouth. Thus, the European Medicines Agency decided that Efmody'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 Efmody?

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

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

Other information about Efmody

Efmody received a marketing authorisation valid throughout the EU on 27 May 2021.

Further information on Efmody can be found on the Agency's website:

ema.europa.eu/medicines/human/EPAR/efmody.

This overview was last updated in 05-2021.