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Agamree (*vamorolone*)

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

What is Agamree and what is it used for?

Agamree is a medicine for treating Duchenne muscular dystrophy in patients from 4 years of age. Duchenne muscular dystrophy is a genetic disease that gradually causes weakness and loss of muscle function.

Duchenne muscular dystrophy is rare, and Agamree was designated an 'orphan medicine' (a medicine used in rare diseases) on 22 August 2014. Further information on the orphan designation can be found on the EMA website.

Agamree contains the active substance vamorolone.

How is Agamree used?

The medicine can only be obtained with a prescription. Treatment should only be started by a specialist doctor with experience in managing Duchenne muscular dystrophy.

The medicine is available as a suspension to be taken by mouth once a day. The doctor will prescribe the dose based on the patient's bodyweight.

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

How does Agamree work?

The active substance in Agamree, vamorolone, is a modified corticosteroid medicine and reduces inflammation by blocking the production of certain inflammatory substances called cytokines. The way it works in patients with Duchenne muscular dystrophy is not fully understood.

What benefits of Agamree have been shown in studies?

A main study showed that Agamree was more effective than placebo (a dummy treatment) in treating Duchenne muscular dystrophy in patients between the ages of 4 and 7 years who were able to walk. The study, involving 121 patients, looked at their TTSTAND velocity (time to stand), which is the speed at which they can stand up from a lying position.

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

After 24 weeks of treatment, the average TTSTAND velocity increased from 0.19 to 0.24 rises per second in patients who took Agamree, while it decreased slightly from 0.20 to 0.19 rises per second in those who had placebo. This effect was maintained up to week 48.

What are the risks associated with Agamree?

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

The most common side effects with Agamree (which may affect more than 1 in 10 people) include Cushingoid features (features caused by long-term use of a corticosteroid, such as fat build-up on the face and bruising), vomiting, increased weight and irritability.

The medicine must not be used in patients who have severely impaired liver function or who have recently received a live vaccine (a vaccine that uses a weakened form of the organism).

Why is Agamree authorised in the EU?

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

The main study in patients between 4 and 7 years of age showed that Agamree is effective in improving their ability to move. Given that vamorolone works in the same way as currently used corticosteroids, the Agency concluded that it can be used in older patients as well.

In terms of safety, Agamree compares well with conventional corticosteroids and does not cause some of the side effects that conventional corticosteroids cause (such as effects on the bone and growth).

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

The company that markets Agamree will provide patients with an alert card with information about the need for daily treatment and the risk of adrenal crisis, a side effect which can occur in patients abruptly stopping corticosteroid treatment.

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

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

Other information about Agamree

Agamree received a marketing authorisation valid throughout the EU on 14 December 2023.

Further information on Agamree can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/agamree

This overview was last updated in 12-2023.