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EPAR summary for the public

Vedrop

tocofersolan

This is a summary of the European public assessment report (EPAR) for Vedrop. It explains how the Agency assessed the medicine to recommend its authorisation in the EU and its conditions of use. It is not intended to provide practical advice on how to use Vedrop.

For practical information about using Vedrop, patients should read the package leaflet or contact their doctor or pharmacist.

What is Vedrop **and what is it used for?**

Vedrop is a medicine used to treat or prevent vitamin E deficiency (low vitamin E levels). It is used in children up to the age of 18 years who have congenital or hereditary chronic cholestasis and who cannot absorb vitamin E from the gut. Congenital or hereditary chronic cholestasis is an inherited disease causing problems with the flow of bile from the liver to the gut. Bile is a fluid produced in the liver that helps to absorb fats from the gut.

Vedrop contains the active substance tocofersolan.

How is Vedrop used?

Vedrop can only be obtained with a prescription and treatment should be started and supervised by a doctor who has experience in treating patients with congenital or hereditary chronic cholestasis.

Vedrop is available as a solution to be given by mouth. The recommended daily dose is 0.34 ml per kilogram body weight. This dose should be adjusted according to the amount of vitamin E in the patient's blood. This should be checked regularly.

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How does Vedrop work?

Vitamin E is a natural substance that cannot be made by the body and is therefore needed in the diet. It has a number of actions in the body, including protecting the nervous system from damage. Because vitamin E dissolves in fats but not in water, it can only be absorbed from the gut into the body alongside fat particles. Patients with cholestasis may have low vitamin E levels because they have problems absorbing fats from the gut.

The active substance in Vedrop, tocofersolan, is vitamin E that has been made water soluble by attaching it to a chemical called polyethylene glycol. Tocofersolan can be absorbed from the gut in children who have difficulty absorbing fats and vitamin E from the diet. This can increase vitamin E levels in the blood and help to prevent damage to the nervous system due to vitamin E deficiency.

What benefits of Vedrop have been shown in studies?

To support the use of Vedrop, the company presented information from the scientific literature, including the results of three studies in a total of 92 children and adolescents with chronic cholestasis who were given tocofersolan for about two years. All the patients had vitamin E deficiency that was not responding to other vitamin E treatments given by mouth. The main measures of effectiveness were the level of vitamin E in the blood and the number of children whose neurological symptoms improved or stayed the same. The studies showed that Vedrop could correct vitamin E levels in patients with chronic cholestasis and that it might improve or prevent neurological symptoms, especially in patients aged below three years.

The company also presented information on the use of Vedrop in patients with cystic fibrosis, but withdrew its application for this disease during the evaluation of the medicine.

What **are** the **risks associated with Vedrop?**

The most common side effect with Vedrop (which may affect up to 1 in 10 people) is diarrhoea. Vedrop must not be used in premature babies. For the full list of all side effects and restrictions with Vedrop, see the package leaflet.

Why is Vedrop approved?

The European Medicines Agency decided that Vedrop's benefits are greater than its risks and recommended that Vedrop be given marketing authorisation.

Vedrop has been authorised under 'exceptional circumstances'. This is because it has not been possible to obtain complete information about Vedrop due to the rarity of the disease. Every year, the European Medicines Agency will review any new information that becomes available and this summary will be updated as necessary.

What information is still awaited for Vedrop?

Since Vedrop has been approved under exceptional circumstances, the company that markets the medicine is required to provide further data on the safety and effectiveness of Vedrop in patients with congenital or hereditary chronic cholestasis.

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

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

Other information about Vedrop

The European Commission granted a marketing authorisation valid throughout the European Union for Vedrop on 24 July 2009.

The full EPAR for Vedrop can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European public assessment reports. For more information about treatment with Vedrop, read the package leaflet (also part of the EPAR) or contact your doctor or pharmacist.

This summary was last updated in 08-2017.