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EPAR summary for the public

Alofisel

darvadstrocel

This is a summary of the European public assessment report (EPAR) for Alofisel. 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 Alofisel.

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

What is Alofisel and what is it used for?

Alofisel is a medicine that is used to treat complex anal fistulas in adults with Crohn's disease (an inflammatory condition of the gut) when a conventional or biological medicine has not worked well enough.

Fistulas are abnormal passages between the lower parts of the gut and the skin near the anus. Complex fistulas are those with several abnormal passages and openings, or with passages that go deep inside the body, or where there are other complications such as collection of pus.

Alofisel contains the active substance darvadstrocel, which comprises stem cells removed from fat tissue of adult donors. It is a type of advanced therapy medicine called a 'somatic cell therapy product'. This is a type of medicine that contains cells or tissues that have been modified so that they can be used to cure, diagnose or prevent a disease.

Because the number of patients with anal fistula is low, the disease is considered 'rare', and Alofisel was designated an 'orphan medicine' (a medicine used in rare diseases) on 8 October 2009.

How is Alofisel used?

Alofisel should be given only by specialist doctors experienced in the diagnosis and treatment of the condition for which it is used. The medicine can only be obtained with a prescription.

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Alofisel is given just once. The patient is given an anaesthetic (either to put the patient to sleep or to numb the treatment area). After preparing the fistulas for treatment in an operating room, the contents of two vials (each containing 30 million cells) are injected around the internal openings and two further vials through the external openings into the walls of the fistula.

For further information, see the package leaflet.

How does Alofisel work?

Alofisel is made up of 'mesenchymal stem cells' from the fat tissue of a donor. To make this medicine, the cells are selected and cultivated in the laboratory to increase their number. When injected into the walls of the fistula, these cells can help to reduce inflammation and support the growth of new tissue. This encourages the fistula to heal and close.

What benefits of Alofisel have been shown in studies?

One main study, involving 212 patients with Crohn's disease and complex anal fistulas, found Alofisel more effective than placebo (a dummy treatment) 24 weeks after treatment. Treatment with conventional or biological medicines had not worked in these patients. The main measure of effectiveness, called 'combined remission', was the closing of abnormal external openings together with lack of fluid collections of more than 2 cm associated with internal passages (since these are likely to re-open the fistula). Of the patients treated with Alofisel, combined remission occurred in almost 50% of patients (53 out of 107); this compared with 34% of patients (36 out of 105) receiving placebo.

What are the risks associated with Alofisel?

The most common side effects with Alofisel (which may affect up to 1 in 10 people) are anal abscess (a swollen area with a collection of pus), proctalgia (anal pain), anal fistula and pain during treatment.

Alofisel must not be used in patients with hypersensitivity (allergy) to bovine serum (the clear liquid in blood from cattle) or to any of the ingredients of Alofisel.

Why is Alofisel approved?

The European Medicines Agency decided that Alofisel's benefits are greater than its risks and recommended that it be approved for use in the EU. Alofisel is of value in the treatment of complex anal fistulas that have not responded well to other treatments. Data on the safety of Alofisel are limited but they provide enough information on the pattern of side effects.

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

The company that markets Alofisel will provide educational material for healthcare professionals on how to give the medicine correctly and on the possibility of passing on an infection to the patient. The company will also complete a study to continue to collect information on the effectiveness and safety of Alofisel.

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

Other information about Alofisel

The European Commission granted a marketing authorisation valid throughout the European Union for Alofisel on 23 March 2018

The full EPAR for Alofisel 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 Alofisel, read the package leaflet (also part of the EPAR) or contact your doctor or pharmacist.

The summary of the opinion of the Committee for Orphan Medicinal Products for Alofisel can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/Rare disease designation.

This summary was last updated in 03-2018.