

EMA/764628/2017  
EMA/H/C/004195

## EPAR summary for the public

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# Adynovi

## rurioctocog alfa pegol

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

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

### What is Adynovi and what is it used for?

Adynovi is a medicine used to treat and prevent bleeding in patients with haemophilia A, an inherited bleeding disorder caused by lack of a clotting protein called factor VIII. It can be used in adults and children from 12 years of age.

Adynovi contains the active substance rurioctocog alfa pegol.

### How is Adynovi used?

Adynovi can only be obtained with a prescription and treatment should be under the supervision of a doctor who has experience in the treatment of haemophilia.

Adynovi is available as a powder and solvent that are mixed together to make a solution for injection into a vein. The dose and frequency of treatment depend on whether it is used to treat or prevent bleeding, as well as on the seriousness of the haemophilia, the extent and location of the bleeding and the patient's condition and bodyweight. Patients or their carers may be able to inject Adynovi themselves at home once they have been trained appropriately.

For further details, see the package leaflet.

## **How does Adynovi work?**

Patients with haemophilia A lack factor VIII, a protein needed for normal clotting of the blood, and as a result, they bleed readily. The active substance in Adynovi, rurioctocog alfa pegol, works in the body in the same way as human factor VIII. It replaces the missing factor VIII, thereby helping the blood to clot and giving temporary control of the bleeding disorder.

## **What benefits of Adynovi have been shown in studies?**

Adynovi has been shown in two main studies to be effective at preventing and treating bleeding episodes in patients with severe haemophilia who were previously treated with other factor VIII products.

In a study involving 138 adults and adolescents aged 12 years or above, 120 patients given Adynovi as a twice-weekly preventive treatment had, on average, around 4 bleeding episodes a year, and 17 patients given Adynovi for treating bleedings 'on demand' had around 43 bleeding episodes a year. In addition, when bleeding did occur, Adynovi was rated excellent or good at treating around 96% of bleeding episodes. Around 96% of bleeding episodes resolved with one or two injections of Adynovi.

In the second study in 66 children aged below 12 years, all patients were given Adynovi as a twice-weekly preventive treatment for around 6 months. During this period, around 38% of patients (25 out of 66) had no bleeding episodes, and none of the patients developed antibodies against Adynovi, which can cause the medicine to stop working. When bleeding occurred, Adynovi was rated excellent or good at treating around 90% of episodes. Around 83% of bleeding episodes resolved with one injection.

## **What are the risks associated with Adynovi?**

Hypersensitivity (allergic) reactions are uncommon with Adynovi (affecting up to 1 in 100 people) and may include swelling, burning and stinging at the injection site, chills, flushing, itchy rash, headache, hives, low blood pressure, lethargy, nausea and vomiting, restlessness, a rapid heartbeat, tightness of the chest and wheezing. In some cases these reactions can become severe.

Following treatment with factor VIII products, including Adynovi, some patients may develop inhibitors (antibodies) against factor VIII, causing the medicine to stop working and resulting in a loss of bleeding control. In such cases, a specialised haemophilia centre should be contacted.

For the full list of all side effects see the package leaflet.

Adynovi must not be used in patients who are hypersensitive (allergic) to rurioctocog alfa pegol or the related substance octocog alfa or to any of the other ingredients of the medicine, nor in those with known allergy to mouse or hamster proteins.

## **Why is Adynovi approved?**

The European Medicines Agency decided that Adynovi's benefits are greater than its risks and recommended that it be approved for use in the EU. Studies show that Adynovi is effective at preventing and treating bleeding episodes in patients with haemophilia A and its safety is comparable to that of other factor VIII products. However, part of the active substance in Adynovi (called PEG) may accumulate in the body, including in a structure in the brain called the choroid plexus, following long-term treatment. Since this could potentially cause problems especially in children below 12 years of age, Adynovi is only approved for use in adults and children from 12 years of age.

## **What measures are being taken to ensure the safe and effective use of Adynovi?**

The company that markets Adynovi will conduct a study to investigate the potential effects of PEG accumulation in the choroid plexus of the brain and other organs.

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

## **Other information about Adynovi**

The European Commission granted a marketing authorisation valid throughout the European Union for Adynovi on 8 January 2018.

The full EPAR for Adynovi can be found on the Agency's website: [ema.europa.eu/Find\\_medicine/Human\\_medicines/European\\_public\\_assessment\\_reports](http://ema.europa.eu/Find_medicine/Human_medicines/European_public_assessment_reports). For more information about treatment with Adynovi, read the package leaflet (also part of the EPAR) or contact your doctor or pharmacist.

This summary was last updated in 01-2018.