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Veyvondi (*vonicog alfa*)

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

What is Veyvondi and what is it used for?

Veyvondi is a medicine used to control bleeding in adults and children with von Willebrand disease (an inherited bleeding disorder) who cannot be treated with desmopressin (another medicine to stop bleeding) or in whom desmopressin does not work.

In adults, it is used to prevent and treat bleeding episodes, including during surgery. In children, it is only used to treat bleeding episodes not related to surgery; it is not used to prevent bleeding episodes.

Veyvondi contains the active substance vonicog alfa.

How is Veyvondi used?

Veyvondi can only be obtained with a prescription and treatment should be supervised by a doctor experienced in treating patients with bleeding disorders.

Veyvondi is given as an injection into a vein. The dose and frequency of the injections depend on whether Veyvondi is used during surgery or to treat or prevent bleeding episodes.

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

How does Veyvondi work?

People with von Willebrand disease lack von Willebrand factor, a protein needed for normal clotting of the blood, and as a result they bleed more easily. The active substance in Veyvondi, vonicog alfa, is produced in the laboratory and works in the same way as natural von Willebrand factor. It replaces the missing protein, thereby helping the blood to clot and allowing the bleeding to be controlled.

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What benefits of Veyvondi have been shown in studies?

Studies in adults

Veyvondi has been shown to be effective at controlling bleeding episodes in three main studies in adults with von Willebrand disease. Veyvondi was not compared with any other treatments in these studies.

The first study involved 37 patients who were given Veyvondi for the treatment of bleeding episodes. The main measure of effectiveness was based on an assessment of how well the treatment worked. Treatment with Veyvondi was successful in 95% of patients (20 out of 22). A total of 193 bleeding episodes were recorded, and Veyvondi was rated 'excellent' or 'good' at treating around 98% of bleeds.

The second study involved 15 patients who were given Veyvondi to prevent bleeding during surgery, including major surgery such as knee replacement. In the 15 major and minor surgeries that occurred during the study, Veyvondi was rated as excellent or good at preventing bleeding episodes for all 15 surgeries.

A third study was carried out in 23 patients with severe von Willebrand disease who received Veyvondi as a preventive treatment to avoid bleeding episodes. Before the study, patients had either been using von Willebrand factor treatment on demand and had experienced at least 3 spontaneous bleeds requiring treatment in the last year, or they had been using a plasma-derived von Willebrand factor preventively for at least 12 months. Plasma-derived means the product was produced from human plasma (the liquid part of blood).

In the 13 patients who had previously been using on-demand treatment, preventive treatment with Veyvondi reduced the number of annual bleeds by about 92% compared with the average number in the year before Veyvondi treatment.

In the 10 patients who had been receiving a plasma-derived von Willebrand factor to prevent bleeds, preventive treatment with Veyvondi reduced the number of annual bleeds by 45% compared with the average number in the year before switching to Veyvondi.

Study in children

An ongoing study involving 25 children with severe von Willebrand disease showed that Veyvondi was effective at treating bleeding episodes unrelated to surgery in these patients. The study did not compare Veyondi with other treatments.

For all of the 18 children who had bleeding episodes, treatment with Veyvondi was considered successful. The treatment's effectiveness was evaluated for a total of 98 bleeding episodes of varying severity, and it was rated as 'excellent' or 'good' for all of them.

What are the risks associated with Veyvondi?

For the full list of side effects with Veyvondi, see the package leaflet.

The most common side effect with Veyvondi (which may affect more than 1 in 10 people) is headache. The following side effects may also occur during treatment with Veyvondi: hypersensitivity (allergic) reactions, thromboembolic events (problems due to the formation of blood clots in the blood vessels), development of inhibitors (antibodies) against von Willebrand factor causing the medicine to stop working and resulting in a loss of bleeding control.

Veyvondi must not be used in patients who have had allergic reactions to mouse or hamster proteins.

Why is Veyvondi authorised in the EU?

Veyvondi was shown to be effective in preventing and treating bleeding episodes in adults with von Willebrand disease. Veyvondi was also effective for preventing and treating bleedings related to surgery. In children, Veyvondi was shown to be effective in treating bleeding episodes that are not related to surgery.

Veyvondi should be used only when desmopressin (the main treatment for von Willebrand disease) cannot be used or is not working well enough. The European Medicines Agency noted that there are uncertainties related to the small number of patients involved in the studies and the lack of a direct comparator; however, this was considered acceptable given the rarity of the disease. The side effects with Veyvondi were considered typical for this type of medicine. The safety profile in children is similar to that seen in adults.

The Agency therefore decided that Veyvondi'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 Veyvondi?

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

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

Other information about Veyvondi

Veyvondi received a marketing authorisation valid throughout the EU on 31 August 2018.

Further information on Veyvondi can be found on the Agency's website: https://www.ema.europa.eu/en/medicines/human/EPAR/veyvondi.

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