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EPAR summary for the public

Nplate

romiplostim

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

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

What is Nplate and what is it used for?

Nplate is used in patients from 1 year of age with long-term immune thrombocytopenic purpura (ITP), a disease in which the patient's immune system destroys platelets (components in the blood that help it to clot). Patients with ITP have low platelet counts and are at risk of bleeding.

Nplate is used when treatment with medicines such as corticosteroids or immunoglobulins has not worked. Nplate can be used in patients whose spleen has been removed to control the disease and in those who still have a spleen. The spleen, an organ behind the stomach, is involved in the removal of platelets from the blood.

Because the number of patients with ITP is low, the disease is considered 'rare', and Nplate was designated an 'orphan medicine' (a medicine used in rare diseases) on 27 May 2005.

Nplate contains the active substance romiplostim.

How is Nplate used?

Nplate can only be obtained with a prescription and treatment with it should be supervised by a doctor who has experience in treating blood disorders.

Nplate is a powder that is made up into a solution for injection. It is given once a week as an injection under the skin. The starting dose depends on the patient's weight, and is then adjusted to maintain platelet counts at the target levels. Treatment should be stopped if the platelet count does not rise

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enough to reduce the risk of bleeding after 4 weeks of treatment with the maximum dose of Nplate. Adults whose platelet levels are stable may inject the medicine themselves after they have been properly trained.

For further information, see the package leaflet.

How does Nplate work?

The active substance in Nplate, romiplostim, stimulates the production of platelets. In the body, a hormone called 'thrombopoietin' normally stimulates the production of platelets in the bone marrow. Romiplostim has been designed to attach to and stimulate the same targets (receptors) as thrombopoietin. By mimicking the action of thrombopoietin, romiplostim stimulates the production of platelets, thereby increasing blood platelet counts and reducing the risk of bleeding.

What benefits of Nplate have been shown in studies?

Two main studies in adults and a third one in children found Nplate effective for the treatment of long-standing ITP. All studies compared Nplate with placebo (a dummy treatment). Patients were treated for 24 weeks and the main measure of effectiveness was the increase in platelet count above a threshold of 50 million platelets per millilitre of blood during at least 6 of the last 8 weeks of treatment. A platelet count below 30 million per millilitre puts patients at risk of bleeding while the normal count is 150 to 400 million per millilitre.

The first study involved 63 patients whose disease was not controlled despite removal of their spleen. Platelet count rose above the threshold in 38% of patients who received Nplate (16 out of 42) compared with none of the 21 patients receiving placebo.

The second study involved 62 patients whose ITP had been treated previously (but who did not have their spleen removed). Platelet count rose above the threshold in 61% of patients who received Nplate (25 out of 41) compared with 5% receiving placebo (1 out of 21).

The study in children involved 62 patients aged 1 to less than 18 years whose ITP had been treated previously (including some whose spleen had been removed). Platelet count rose above the threshold in 52% of patients who received Nplate (22 out of 42) compared with 10% receiving placebo (2 out of 20).

Long-term studies involving over 1,000 patients, some treated for longer than 5 years, confirmed that Nplate remained effective both in patients whose spleen had been removed and those who had their spleen.

What are the risks associated with Nplate?

The most common side effects with Nplate in adults (seen in more than 1 patient in 10) include headache, infections of the nose and throat and allergic (hypersensitivity) reactions such as rash, itching and rapid swelling under the skin. The most common side effects in children include infections of the nose and throat, runny nose, cough, fever, mouth and throat pain, abdominal (belly) pain, diarrhoea, rash and bruising. For the full list of side effects reported with Nplate, see the package leaflet.

Nplate must not be used in people who are hypersensitive (allergic) to romiplostim, any of the other ingredients, or proteins produced by *Escherichia coli* (a bacterium).

Why is Nplate approved?

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

The Agency noted that Nplate was effective in patients who had had their spleen removed, as well as in patients who had not had their spleen removed. The improvement in platelet count was long-lasting and clinically relevant in both groups, although the treatment only manages symptoms and is not a cure. Therefore, in patients with intact spleens, the possibility of removing the spleen should be evaluated periodically.

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

The company that markets Nplate will provide doctors a 'dosing calculator' to help them calculate the sometimes very small volumes of Nplate that need to be injected. Doctors can also receive a home administration training pack, which includes materials on training patients who will inject themselves with Nplate, and materials for patients on how to prepare the medicine for injection.

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

Other information about Nplate

The European Commission granted a marketing authorisation valid throughout the European Union for Nplate on 4 February 2009.

The full EPAR for Nplate 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 Nplate, 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 Nplate can be found on the Agency's website: ema.europa.eu/Find medicine/Human medicines/Rare disease designation.

This summary was last updated in 12-2017.