10 February 2021
EMADOC-628903358-3059

Public summary of opinion on orphan designation

1-(3-methylbutanoyl)-L-aspartyl-L-threonyl-L-histidyl-L-phenylalanyl-L-prolyl-(L-cystinyl-L-isoleucyl-[(N6-(S)-4-carboxy-4-palmitamidobutanoyl)-L-lysinyl]-L-phenylalanyl-L-glutamyl-L-prolyl-L-arginyl-L-serinyl-L-lysinyl-L-glycinyl-L-cystinyl)-L-lysinamide, disulfide, acetate for the treatment of polycythaemia vera

On 19 October 2020, orphan designation EU/3/20/2330 was granted by the European Commission to Scendea (NL) B.V., Netherlands, for 1-(3-methylbutanoyl)-L-aspartyl-L-threonyl-L-histidyl-L-phenylalanyl-L-prolyl-(L-cystinyl-L-isoleucyl-[(N6-(S)-4-carboxy-4-palmitamidobutanoyl)-L-lysinyl]-L-phenylalanyl-L-glutamyl-L-prolyl-L-arginyl-L-serinyl-L-lysinyl-L-glycinyl-L-cystinyl)-L-lysinamide, disulfide, acetate (also known as PTG-300) for the treatment of polycythaemia vera.

What is polycythaemia vera?

Polycythaemia vera is a disease in which the bone marrow (the spongy tissue inside the large bones where blood cells are produced) produces too many red blood cells. This makes the blood thicker and can result in reduced blood flow to the organs and occasionally the formation of blood clots. While some patients with polycythaemia vera do not have any symptoms, others may have itching, tiredness, headache, blurred vision and an enlarged liver and spleen. Patients who develop blood clots in the small blood vessels can also experience a wide range of symptoms including burning pains in the hands. Patients with blood clots in the arteries can have strokes.

Polycythaemia vera is a long-term debilitating and life-threatening condition because it may lead to the formation of blood clots and bleeding and can result in leukaemia (cancer of the white blood cells) and myelofibrosis (a disease of the bone marrow).

What is the estimated number of patients affected by the condition?

At the time of designation, polycythaemia vera affected approximately 3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 156,000 people*, and is below the

*For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union, Iceland, Liechtenstein, Norway and the United Kingdom. This represents a population of 519,200,000 (Eurostat 2020).

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ceiling for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of designation, several medicines were authorised for polycythaemia vera, including ruxolitinib which is authorised in the EU for use in adults who are resistant or intolerant to treatment with the medicine hydroxyurea. In addition, phlebotomy (removal of some of the blood from the body) and long-term treatment with low-dose aspirin were recommended in some patients to reduce the risk of blood clot formation.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with polycythaemia vera because early studies suggest that the medicine can reduce the amount of red blood cells in the blood and the need for phlebotomy in patients at low risk of complications. The medicine also seems effective in combination with other treatments in patients at high risk of complication.

This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

This medicine mimics the action of the liver enzyme hepcidin, which regulates the levels of iron in the body. It is expected to reduce the levels of iron available to the body by blocking the uptake of iron from food and by stopping the release of iron from iron-storage cells. Since iron is vital for the production of red blood cells, it is expected that by reducing the amount of iron available for this, the medicine will trigger a reduction in the production of red blood cells, which should reduce the symptoms of the disease.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the medicine in patients with polycythaemia vera were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for the treatment of polycythaemia vera or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 10 September 2020, recommending the granting of this designation.

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Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the EU) or insufficient returns on investment.

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information

Sponsor's contact details:

Contact details of the current sponsor for this orphan designation can be found on EMA website.

For contact details of patients' organisations whose activities are targeted at rare diseases see:

- Orphanet, a database containing information on rare diseases, which includes a directory of patients' organisations registered in Europe;
- European Organisation for Rare Diseases (EURORDIS), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.