3 March 2010
EMA/COMP/834043/2009
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Givinostat for the treatment of polycythaemia vera

On 3 February 2010, orphan designation (EU/3/09/719) was granted by the European Commission to Italfarmaco S.p.A., Italy, for givinostat 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 in the body) 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 is equivalent to a total of around 151,000 people, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of designation, hydroxycarbamide, pipobroman and busulfan were authorised in some Member States to reduce the number of red blood cells in patients with polycythaemia vera. 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.

*Disclaimer: 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 (EU 27), Norway, Iceland and Liechtenstein. This represents a population of 504,800,000 (Eurostat 2009).

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The sponsor has provided sufficient information to show that givinostat might be of significant benefit for patients with polycythaemia vera because early studies indicate that it might improve the treatment of patients with this condition by reducing the symptoms of the disease. 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?

Patients with polycythaemia vera have abnormalities in a gene that is responsible for the production of an enzyme known as Janus kinase 2 (JAK2). JAK2 is involved in the reproduction and growth of red blood cells. In polycythaemia vera, JAK2 is overactivated. Givinostat is thought to work by reducing the levels of JAK2. This is expected to slow down the abnormal growth of red blood cells, reducing the symptoms of the disease.

What is the stage of development of this medicine?

The effects of givinostat have been evaluated in experimental models.

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

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 3 December 2009 recommending the granting of this designation.

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 Community) 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

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Translations of the active ingredient and indication in all official EU languages, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Givinostat	Treatment of polycythaemia vera
Bulgarian	Гивиностат	Лечение на полицитемия вера
Czech	Givinostat	Léčba polycythemia vera
Danish	Givinostat	Behandling af polycythæmia vera
Dutch	Givinostat	Behandeling van polycythaemia vera
Estonian	Givinostat	Polycythemia vera ravi.
Finnish	Givinostat	Polysytemia veran hoitoon
French	Givinostat	Traitement de la Polyglobulie de Vaquez
German	Givinostat	Behandlung von Polycythemia vera
Greek	Τζιβινοστάτη	Θεραπεία της αληθούς πολυκυτταραιμίας, ή ερυθραιμίας (Polycythaemia vera)
Hungarian	Givinostat	Polycythaemia vera kezelésére
Italian	Givinostat	Terapia della policitemia vera
Latvian	Givinostats	Polycythemia vera ārstēšanai
Lithuanian	Givinostatas	Tikrosios policitemijos (Polycythemia vera) gydymas
Maltese	Givinostat	Kura tal-policitemija vera
Polish	Givinostat	Leczenie czerwienicy prawdziwej
Portuguese	Givinostat	Tratamento da policitemia vera
Romanian	Givinostat	Tratamentul policitemiei vera
Slovak	Givinostat	Liečba pravej polycytémie
Slovenian	Givinostat	Zdravljenje prave policitemije
Spanish	Givinostat	Tratamiento de la policitemia vera
Swedish	Givinostat	Behandling av polycytemia vera
Norwegian	Givinostat	Behandling av polycythemia vera
Icelandic	Gívínóstat	Til meðferðar á polycythemia vera