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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Pegylated proline-interferon alpha-2b for the treatment of polycythaemia vera

On 9 December 2011, orphan designation (EU/3/11/932) was granted by the European Commission to AOP Orphan Pharmaceuticals AG, Austria, for pegylated proline-interferon alpha-2b 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 is equivalent to a total of around 152,000 people, and is below the 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).

*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 506,300,000 (Eurostat 2011).

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What treatments are available?

At the time of designation, hydroxycarbamide and busulfan were authorised in some EU 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) was recommended in some patients to reduce the risk of blood clot formation.

The sponsor has provided sufficient information to show that pegylated proline-interferon alpha-2b might be of significant benefit for patients with polycythaemia vera because early studies show that it might improve the outcome of patients with this condition and be safer than existing treatments. 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?

Pegylated proline-interferon alpha-2b belongs to the group 'interferons'. Interferons are natural substances produced by the body to help it fight against attacks such as viral infections. The exact way alpha interferons work is not fully understood, but it is thought that they act as immunomodulators (substances that modify how the immune system, the body's defence system, works). Interferon-alpha has been used in the management of patients with various blood disorders for more than two decades.

Pegylated proline-interferon alpha-2b is expected to be injected under the skin. The medicine is expected to work in polycythaemia vera by blocking the production of blood cells in the bone marrow.

In this medicine, interferon alfa-2b has been 'pegylated' (coated with a chemical called polyethylene glycol). This decreases the rate at which the substance is removed from the body and allows the medicine to be given less often. The interferon alfa-2b is produced by a method known as 'recombinant DNA technology': it is made by a bacterium that has received a gene (DNA), which makes it able to produce interferon alfa-2b. The replacement acts in same way as naturally produced interferon alpha.

What is the stage of development of this medicine?

The effects of pegylated proline-interferon alpha-2b have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with pegylated proline-interferon alpha-2b in patients with polycythaemia vera were ongoing.

At the time of submission, pegylated proline-interferon alpha-2b 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 7 October 2011 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:

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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.

Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Pegylated proline-interferon alpha-2b	Treatment of polycythaemia vera
Bulgarian	Пегилиран пролин-interferon алфа-2b	Лечение на полицитемия вера
Czech	Pegylovaný prolin-interferon alfa-2b	Léčba polycythemia vera
Danish	Pegyleret prolin-interferon alfa-2b	Behandling af polycythæmia vera
Dutch	Gepegyleerd proline-interferon-alfa-2b	Behandeling van polycythaemia vera
Estonian	Pegüleeritud proline-Interferoon alfa-2b	Polycythemia vera ravi.
Finnish	Pegyloitu proliini-interferoni-alfa-2b	Polysytemia veran hoitoon
French	Proline-interféron-alpha-2b pegylé	Traitement de la Polyglobulie de Vaquez
German	Pegyliertes prolin-interferon alpha-2b	Behandlung der Polycythemia vera
Greek	Πεγκυλιωμένη σε προλίνη-ιντερφερόνη-α-2b	Θεραπεία της αληθούς πολυκυτταραιμίας, ή ερυθραιμίας (Polycythaemia vera)
Hungarian	Pegilált prolin-interferon-alfa-2b	Polycythaemia vera kezelésére
Italian	Prolina-interferone-alfa-2b pegilato	Terapia della policitemia vera
Latvian	Pegilēts alfa 2b prolīna-interferons	Polycythemia vera ārstēšanai
Lithuanian	Pegiliuotas prolinas- alfa-2b interferono	Tikrosios policitemijos (Polycythemia vera) gydymas
Maltese	Proline-interferon-alfa-2b peġilat	Kura tal-poliċitemija vera
Polish	Pegylowana prolina-interferon alfa-2b	Leczenie czerwienicy prawdziwej
Portuguese	Prolina-interferão-alfa-2b peguilado	Tratamento da policitemia vera
Romanian	Prolin-interferon-alfa-2b pegilat	Tratamentul policitemiei vera
Slovak	Pegylovaný prolín-interferón alfa-2b	Liečba pravej polycytémie
Slovenian	Pegilirani prolin-interferon-alfa-2b	Zdravljenje prave policitemije
Spanish	Interferón alfa-2b pegilado sobre prolina	Tratamiento de la policitemia vera
Swedish	Pegylerad prolin-interferon-alfa-2b	Behandling av polycytemia vera
Norwegian	Pegylert prolin-interferon-alfa-2b	Behandling av polycythemia vera
Icelandic	Pegýlerað prólín-interferón-alfa-2b	Til meðferðar á polycythemia vera

[1] At the time of designation