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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Ruxolitinib for the treatment of polycythaemia vera

First publication	31 March 2014
Rev.1: sponsor's change of address	6 February 2015
Rev.2: withdrawal from the Community Register	17 April 2015
Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in February 2015 on request of the Sponsor.

On 19 February 2014, orphan designation (EU/3/14/1244) was granted by the European Commission to Novartis Europharm Limited, United Kingdom, for ruxolitinib for 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).

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

At the time of designation, polycythaemia vera affected less than 5 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 256,000 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).

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.

The sponsor has provided sufficient information to show that ruxolitinib might be of significant benefit for patients with polycythaemia vera because early studies indicate that it might improve the outcome of patients whose disease does not respond to hydroxycarbamide. 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?

Ruxolitinib is a medicine that is already authorised under the name of Jakavi for the treatment of myelofibrosis. It works by blocking a group of enzymes known as Janus kinases (JAKs), which are involved in the production and growth of blood cells. In polycythaemia vera, these enzyme are overactive, leading to the production of too many red blood cells. By blocking these enzymes, ruxolitinib is expected to decrease the abnormal production of red blood cells, relieving the symptoms of polycythaemia vera.

What is the stage of development of this medicine?

The effects of ruxolitinib have been evaluated in experimental models.

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

At the time of submission, ruxolitinib was authorised in the EU for the treatment of myelofibrosis.

At the time of submission, ruxolitinib was not authorised anywhere in the EU for polycythaemia vera. Orphan designation of ruxolitinib had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 January 2014 recommending the granting of this designation.

*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 28), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 512,900,000 (Eurostat 2014).

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	Ruxolitinib	Treatment of polycythaemia vera
Bulgarian	Руксолитиниб	Лечение на полицитемия вера
Czech	Ruxolitinib	Léčba polycythemia vera
Croatian	Ruksolitinib	Liječenje policitemije vere
Danish	Ruxolitinib	Behandling af polycytæmia vera
Dutch	Ruxolitinib	Behandeling van polycythaemia vera
Estonian	Ruksolitiniib	Polycythemia vera ravi
Finnish	Ruksolitinibi	Polysytemia veran hoito
French	Ruxolitinib	Traitement de la Polyglobulie de Vaquez
German	Ruxolitinib	Behandlung von Polycythemia vera
Greek	Ρουξολιτινίμπη	Θεραπεία της αληθούς πολυκυτταραιμίας
Hungarian	Ruxolitinib	Polycythaemia vera kezelése
Italian	Ruxolitinib	Terapia della policitemia vera
Latvian	Ruksolitinibs	Polycythemia vera ārstēšanai
Lithuanian	Ruksolitinibas	Tikrosios policitemijos (Polycythemia vera) gydymas
Maltese	Ruxolitinib	Kura tal-poliċitemija vera
Polish	Ruksolitynib	Leczenie czerwienicy prawdziwej
Portuguese	Ruxolitinib	Tratamento da policitemia vera
Romanian	Ruxolitinib	Tratamentul policitemiei vera
Slovak	Ruxolitinib	Liečba pravej polycytémie
Slovenian	Ruksolitinib	Zdravljenje prave policitemije
Spanish	Ruxolitinib	Tratamiento de la policitemia vera
Swedish	Ruxolitinib	Behandling av polycytemia vera
Norwegian	Ruksolitinib	Behandling av polycythemia vera
Icelandic	Rúxólitíníb	Til meðferðar á polycythemia vera

[1] At the time of designation