29 January 2016
EMA/COMP/777525/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Imetelstat sodium for the treatment of myelofibrosis

On 14 December 2015, orphan designation (EU/3/15/1593) was granted by the European Commission to Janssen-Cilag International N.V., Belgium, for imetelstat sodium for the treatment of myelofibrosis.

What is myelofibrosis?

Myelofibrosis is a disease in which the bone marrow (the spongy tissue inside the large bones where blood cells are produced) becomes dense and fibrous, and starts producing abnormal immature blood cells that replace the normal blood cells. In myelofibrosis, some immature blood cells migrate from the bone marrow to other organs, such as the spleen and liver, where they mature. This causes the organs to become enlarged. Patients with the disease can develop several symptoms, including bone pain, tiredness, weakness, weight loss, fever and bleeding.

Myelofibrosis is a debilitating disease that is long-lasting and life-threatening because it can lead to severe anaemia (low red blood cell counts) and infections, and can result in leukaemia (cancer of the white blood cells).

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

At the time of designation, myelofibrosis affected approximately 0.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 31,000 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, busulfan, hydroxycarbamide and ruxolitinib were authorised in the EU for myelofibrosis. In addition, medicines were authorised to treat the symptoms, including erythropoietin (a hormone that stimulates the production of red blood cells) to treat anaemia, and surgery was used to remove the enlarged spleen. In some patients, haematopoietic (blood) stem-cell transplantation was

*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. This represents a population of 512,900,000 (Eurostat 2015).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

used to treat the disease. This is a complex procedure where the patient receives stem cells from a matched donor to help restore the bone marrow.

The sponsor has provided sufficient information to show that imetelstat sodium might be of significant benefit for patients with myelofibrosis. The medicine works in a different way to existing treatments and early studies showed a reversal of bone marrow fibrosis, which is not achieved by authorised 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?

Imetelstat sodium works by blocking the activity of an enzyme called telomerase. Telomerase is involved in regulating cell growth and division. In cells dividing rapidly such as cancer cells, telomerase is very active, which enables cells to divide without control. By blocking the activity of telomerase, this medicine is expected to stop the uncontrolled division of abnormal immature blood cells, therefore slowing the progression of myelofibrosis.

What is the stage of development of this medicine?

The effects of imetelstat sodium have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with imetelstat sodium in patients with myelofibrosis were ongoing.

At the time of submission, imetelstat sodium was not authorised anywhere in the EU for myelofibrosis. Orphan designation of the medicine had been granted in the United States for myelofibrosis.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 November 2015 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 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, on the medicine's rare disease designations page.

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	Imetelstat sodium	Treatment of myelofibrosis
Bulgarian	Иметелстат натрий	Лечение на миелофиброза
Croatian	Imetelstatnatrij	Liječenje mijelofibroze
Czech	Imetelstatum natrium	Léčba myelofibrózy
Danish	Imetelstatnatrium	Behandling af myelofibrose
Dutch	Imetelstatnatrium	Behandeling van myelofibrose
Estonian	Naatriummetelstaat	Müelofibroosi ravi
Finnish	Imetelstaattinatrium	Myelofibroosin hoito
French	Imételstat sodium	Traitement de la myélofibrose
German	Imetelstat Natrium	Behandlung der Myelofibrose
Greek	Νατριούχος ιμετελστάτη	Θεραπεία της μυελοϊνωσης
Hungarian	Imetelstat nátrium	Myelofibrosis kezelése
Italian	Imetelstat sodico	Trattamento della mielofibrosi
Latvian	Imetelstata nātrija sāls	Mielofibrozes ārstēšana
Lithuanian	Natrio imetelstatas	Mielofibrozės gydymas
Maltese	Imetelstat sodium	Kura tal-mjelofibrożi
Polish	Imetelstat sodu	Leczenie mielofibrozy
Portuguese	Imetelstato de sódio	Tratamento da mielofibrose
Romanian	Imetelstat sodic	Tratamentul mielofibrozei
Slovak	Imetelstat sodný	Liečba myelofibrózy
Slovenian	Natrijev imetelstat	Zdravljenje mielofibroze
Spanish	Imetelstat sódico	Tratamiento de la mielofibrosis
Swedish	Natriumimetelstat	Behandling av myelofibros
Norwegian	Natriumimetelstat	Behandling av myelofibrose
Icelandic	Natríumímetelstat	Meðferð á mýelófíbrósu

[1] At the time of designation