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Public summary of opinion on orphan designation

Imetelstat sodium for the treatment of myelodysplastic syndromes

On 27 July 2020, orphan designation EU/3/20/2305 was granted by the European Commission to Parexel International GmbH, Germany, for imetelstat sodium for the treatment of myelodysplastic syndromes.

What are myelodysplastic syndromes?

Myelodysplastic syndromes are a group of disorders in which the red blood cells, white blood cells and platelets produced by the bone marrow (the spongy tissue inside large bones) do not mature normally. Patients with myelodysplastic syndromes can develop tiredness or weakness due to anaemia (low red blood cell counts), infections due to low white blood cell counts, and bruising or abnormal bleeding due to low platelet counts.

Myelodysplastic syndromes are long-term debilitating and life-threatening diseases because they can lead to severe anaemia, infections or bleeding, 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, myelodysplastic syndromes affected approximately 2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 104,000 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, several medicines were authorised in the EU for the treatment of myelodysplastic syndromes including azacitidine, lenalidomide and imatinib. The choice of treatment depended on a number of factors, including the type and the extent of the disease, whether it had been treated before, and the patient's age, symptoms and general state of health. The main treatments included medicines that stimulate production of blood cells, chemotherapy (medicines to

*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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treat cancer), blood transfusions and stem cell transplantation. Stem cell transplantation is a procedure where the patient's bone marrow is cleared of cells and replaced with stem cells from a donor to form new bone marrow that produces healthy blood cells.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with myelodysplastic syndromes, because early data showed that some patients did not need blood transfusions or needed fewer transfusions after treatment with the medicine. 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?

The medicine is expected to work by blocking the activity of an enzyme called telomerase that is involved in protecting the cell's genetic material during cell growth and division. When telomerase is not active, the genetic material in the abnormal blood cells become damaged, the cells cannot divide, and they die. This is expected to slow down progression of the disease.

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 myelodysplastic syndromes were ongoing.

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

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 18 June 2020, 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

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.

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 myelodysplastic syndromes
Bulgarian	Имителстат натрий	Лечение на миелодиспластичен синдром
Croatian	Imetelstatnatrij	Liječenje mijelodisplastičnih sindroma
Czech	Imetelstatum natrium	Léčba myelodysplastického syndromu
Danish	Imetelstatnatrium	Behandling af myelodysplastiske syndromer
Dutch	Imetelstatnatrium	Behandeling van myelodysplastische syndromen
Estonian	Naatriummetelstaat	Müelodüsplastiliste sündroomide ravi
Finnish	Imetelstaattinatrium	Myelodysplastisten syndroomien hoito
French	Imételstat sodium	Traitement des syndromes myélodysplasiques
German	Imetelstat Natrium	Behandlung der myelodysplastischen Syndrome
Greek	Νατριούχος ιμετελστάτη	Θεραπεία των μυελοδυσπλαστικών συνδρόμων
Hungarian	Imetelstat nátrium	Myelodysplasias syndroma kezelése
Italian	Imetelstat sodico	Trattamento delle sindromi mielodisplastiche
Latvian	Imetelstata nātrija sāls	Mielodisplastisko sindromu ārstēšana
Lithuanian	Natrio imetelstatas	Mielodisplastinių sindromų gydymas
Maltese	Imetelstat sodium	Kura tas-sindromi mjelodisplastiċi
Polish	Imetelstat sodu	Leczenie zespołów mielodysplastycznych
Portuguese	Imetelstato de sódio	Tratamento dos síndromes mielodisplásicos
Romanian	Imetelstat sodic	Tratamentul sindromului mielodisplazic
Slovak	Imetelstat sodný	Liečba myelodysplastického syndrómu
Slovenian	Natrijev imetelstat	Zdravljenje mielodisplastičnega sindroma
Spanish	Imetelstat sódico	Tratamiento de los síndromes mielodisplásicos
Swedish	Natriumimetelstat	Behandling av myelodysplastiska syndrom
Norwegian	Natriumimetelstat	Behandling av myelodysplastisk syndrom
Icelandic	Natríumímetelstat	Til meðferðar við mergmisþroskaheilkenni

[1] At the time of designation