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Public summary of opinion on orphan designation

Magrolimab for the treatment of myelodysplastic syndromes

On 26 June 2020, orphan designation EU/3/20/2288 was granted by the European Commission to Granzer Regulatory Consulting & Services, Germany, for magrolimab 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 less than 2 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 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, decitabine and lenalidomide. 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 magrolimab might be of significant benefit for patients with myelodysplastic syndromes. Early data on use of the medicine in patients with myelodysplastic syndromes at intermediate or high risk of developing leukaemia indicated that treatment with magrolimab in combination with azacitidine may lead to a long-lasting response and be more effective than azacitidine taken alone. 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?

Magrolimab is a monoclonal antibody (a type of protein) designed to recognise and attach to a protein called CD47 that is widely found on the surface of the abnormal cells seen in myelodysplastic syndromes. CD47 is used by these cells to avoid detection by the body’s immune (defence) system. By binding to CD47, magrolimab is thought to help the immune system detect and kill the abnormal blood cells.

What is the stage of development of this medicine?

The effects of magrolimab have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with magrolimab in patients with myelodysplastic syndromes were ongoing.

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

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 20 May 2020, 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

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	Magrolimab	Treatment of myelodysplastic syndromes
Bulgarian	Магролимаб	Лечение на миелодиспластичен синдром
Croatian	Magrolimab	Liječenje mijelodisplastičnih sindroma
Czech	Magrolimab	Léčba myelodysplastického syndromu
Danish	Magrolimab	Behandling af myelodysplastiske syndromer
Dutch	Magrolimab	Behandeling van myelodysplastische syndromen
Estonian	Magrolimab	Müelodüsplastiliste sündroomide ravi
Finnish	Magrolimabi	Myelodysplastisten syndroomien hoito
French	Magrolimab	Traitement des syndromes myélodysplasiques
German	Magrolimab	Behandlung der myelodysplastischen Syndrome
Greek	Μαγκρολιμάμπη	Θεραπεία των μυελοδυσπλαστικών συνδρόμων
Hungarian	Magrolimab	Myelodysplasias syndroma kezelése
Italian	Magrolimab	Trattamento delle sindromi mielodisplastiche
Latvian	Magrolimabs	Mielodisplastisko sindromu ārstēšana
Lithuanian	Magrolimabas	Mielodisplastinių sindromų gydymas
Maltese	Magrolimab	Kura tas-sindromi mjelodisplastiċi
Polish	Magrolimab	Leczenie zespołów mielodysplastycznych
Portuguese	Magrolimab	Tratamento dos síndromes mielodisplásicos
Romanian	Magrolimab	Tratamentul sindromului mielodisplazic
Slovak	Magrolimab	Liečba myelodysplastického syndrómu
Slovenian	Magrolimab	Zdravljenje mielodisplastičnega sindroma
Spanish	Magrolimab	Tratamiento de los síndromes mielodisplásicos
Swedish	Magrolimab	Behandling av myelodysplastiska syndrom
Norwegian	Magrolimab	Behandling av myelodysplastisk syndrom
Icelandic	Magrolimab	Til meðferðar við mergmisþroskaheilkenni

[1] At the time of designation