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Public summary of opinion on orphan designation

Asunercept for the treatment of myelodysplastic syndromes

On 23 August 2017, orphan designation (EU/3/17/1900) was granted by the European Commission to Apogenix AG, Germany, for asunercept 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 several symptoms including 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 103,000 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, some medicines were authorised in the EU for the treatment of myelodysplastic syndromes. 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 treat cancer), blood transfusions and stem cell transplantation. Stem cell transplantation is a procedure where the patient's bone marrow is cleared of

*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 515,700,000 (Eurostat 2017).

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

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 this medicine might be of significant benefit for patients with myelodysplastic syndromes. Observational data showed that the medicine led to improvements in patients with low or intermediate risk disease who are not specifically targeted by current 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?

Asunercept works by attaching to a protein known as the CD95 ligand, which triggers a natural process that leads to cell death (apoptosis). The CD95 ligand plays an important role in controlling the production of blood cells and is found at excess levels in the blood cells of patients with myelodysplastic syndromes. By attaching to the protein, the medicine is expected to block the triggering of apoptosis, allowing blood cells to grow and mature normally.

What is the stage of development of this medicine?

The effects of asunercept have been evaluated in experimental models.

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

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 July 2017 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:

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

[1] At the time of designation