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Public summary of opinion on orphan designation

Humanised IgG4 monoclonal antibody to the human toll-like receptor type 2 for the treatment of myelodysplastic syndromes

On 27 February 2017, orphan designation (EU/3/17/1837) was granted by the European Commission to Opsona Therapeutics Ltd, Ireland, for humanised IgG4 monoclonal antibody to the human toll-like receptor type 2 (also known as OPN-305) 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 grow and 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

*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).

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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 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 because early studies show that it may increase blood cell counts in patients with less aggressive forms of myelodysplastic syndromes and who cannot take other medicines for the condition or where other medicines have not worked. 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 a monoclonal antibody (a type of protein) that has been designed to attach to another protein called toll-like receptor type 2 (TLR2). TLR2 is part of the immune system (the body's natural defences) and may play a role in abnormal growth of blood cells. By attaching to TLR2, this medicine is expected to block its activity, thereby leading to normal blood cell growth.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

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

At the time of submission, the medicine was not authorised anywhere in the EU for myelodysplastic syndromes. Orphan designation of the medicine had been granted in the US for myelodysplastic syndromes.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 19 January 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	Humanised IgG4 monoclonal antibody to the human toll-like receptor type 2	Treatment of myelodysplastic syndromes
Bulgarian	Хуманизирано IgG4 моноклонално антитяло срещу човешкия Toll-like рецептор 2	Лечение на миелодиспластичен синдром
Croatian	Humanizirano IgG4 monoklonsko protutijelo za ljudski toll-like receptor tipa 2	Liječenje mijelodisplastičnih sindroma
Czech	Humanizovaná IgG4 monoklonální protilátka proti humánnímu Toll-like receptoru typu 2	Léčba myelodysplastického syndromu
Danish	Humaniseret monoklonalt antistof af typen IgG4 rettet mod den humane Toll-like receptor 2	Behandling af myelodysplastiske syndromer
Dutch	Gehumaniseerde monoklonale IgG4-antilichamen tegen humane Toll-like receptoren type 2	Behandeling van myelodysplastische syndromen
Estonian	Inimese 2. tüüpi toll-like retseptori humaniseeritud IgG4 monoklonaalne antikeha	Müelodüsplastiliste sündroomide ravi
Finnish	Humaanin ”Toll-like” reseptori tyyppi 2:n humanisoitu monoklonaalinen IgG4-luokan vasta-aine	Myelodysplastisten syndroomien hoito
French	Anticorps monoclonal IgG4 humanisé dirigé contre le récepteur toll-like de type 2 (TLR2) humain	Traitement des syndromes myélodysplasiques
German	Humanisierter monoklonaler IgG4-Antikörper gegen den humanen Toll-like-Rezeptor Typ 2	Behandlung der myelodysplastischen Syndrome
Greek	Εξανθρωπισμένο μονοκλωνικό αντίσωμα IgG4 στον ανθρώπινο υποδοχέα TLR2 (Toll-like receptor)	Θεραπεία των μυελοδυσπλαστικών συνδρόμων
Hungarian	2-es típusú, humán Toll-like receptor elleni humanizált IgG4 monoklonális antitest	Myelodysplasias syndroma kezelése
Italian	Anticorpo monoclonale IgG4 umanizzato verso il recettore Toll-like umano di tipo 2	Trattamento delle sindromi mielodisplastiche
Latvian	Humanizētas IgG4 monoklonālās antivielas pret cilvēka Toll-līdzīgo 2.tipa receptoru	Mielodisplastisko sindromu ārstēšana
Lithuanian	Humanizuotas IgG4 monokloninis antikūnas, veikiantis žmogaus „toll-like“ 2 tipo receptorius	Mielodisplastinių sindromų gydymas
Maltese	Antikorp monoklonali IgG4 umanizzat għar-riċettur tat-tip 2 “toll-like” uman	Kura tas-sindromi mjelodisplastiċi
Polish	Humanizowane przeciwciało monoklonalne IgG4 specyficzne względem ludzkiego receptora Toll-like typu 2-go	Leczenie zespołów mielodysplastycznych
Portuguese	Anticorpos monoclonais IgG4 humanizados para o receptor “Toll-like” humano tipo 2	Tratamento dos síndromes mielodisplásicos

[1] At the time of designation

Language	Active ingredient	Indication
Romanian	Anticorp monoclonal IgG4 umanizat îndreptat împotriva receptorului uman „Toll-like” de tip 2	Tratamentul sindromului mielodisplazic
Slovak	Humanizovaná IgG4 monoklonová protilátka proti ľudskému toll-like receptoru typu 2	Liečba myelodysplastického syndrómu
Slovenian	Humanizirano monoklonsko protitelo IgG4 proti človeškemu Toll-like receptorju tipa 2	Zdravljenje mielodisplastičnega sindroma
Spanish	Anticuerpo monoclonal humanizado IgG4 frente al receptor humano toll-like de tipo 2	Tratamiento de los síndromes mielodisplásicos
Swedish	Humaniserad IgG4 monoklonal antikropp mot human ”Toll-like” receptor 2	Behandling av myelodysplastiska syndrom
Norwegian	Humanisert IgG4 monoklonalt antistoff mot human “Toll-like” reseptor type 2	Behandling av myelodysplastisk syndrom
Icelandic	Mannaaðlagað IgG4 einstofna mótefni gegn manna „Toll-líkum” viðtaka af tegund 2	Til meðferðar við mergmisþroskaheilkenni