27 May 2016
EMA/COMP/235594/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

N-carboxymethyl-glycyl-L-threonyl-L-histidyl-L-3,3-diphenylalanyl-L-piperidincarboxy-3-yl-L-arginyl-L-S-methylthio-cystyl-L-arginyl-L-tryptophyl-aminohexanyl-N-carboxamidomethyl-glycine N-hexadecylamide for the treatment of beta thalassaemia intermedia and major

On 28 April 2016, orphan designation (EU/3/16/1650) was granted by the European Commission to QRC Consultants Ltd, United Kingdom, for N-carboxymethyl-glycyl-L-threonyl-L-histidyl-L-3,3-diphenylalanyl-L-piperidincarboxy-3-yl-L-arginyl-L-S-methylthio-cystyl-L-arginyl-L-tryptophyl-aminohexanyl-N-carboxamidomethyl-glycine N-hexadecylamide for the treatment of beta thalassaemia intermedia and major.

What is beta thalassaemia intermedia and major?

Beta thalassaemia is an inherited disease in which patients are unable to make enough haemoglobin, the iron-rich protein found in red blood cells that carries oxygen around the body. Beta thalassaemia major is a severe form of the disease in which patients need frequent blood transfusions, while beta thalassaemia intermedia is a less severe form, which may get worse with age. Both types of beta thalassaemia are caused by defects in the gene responsible for the production of beta globin, one of the components of haemoglobin, which result in low levels of haemoglobin in the blood.

Beta thalassaemia intermedia and major are life-long debilitating diseases. They may be life threatening because of severe anaemia (low red blood cell count due to lack of haemoglobin), the need for repeated blood transfusions and the risk of complications associated with them.

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

At the time of designation, beta thalassaemia intermedia and major affected approximately 0.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 26,000 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).

*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 513,700,000 (Eurostat 2016).

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What treatments are available?

At the time of designation, the main treatments for beta thalassaemia intermedia and major were blood transfusion and the use of iron chelators (medicines for reducing the high iron levels in the body caused by repeated blood transfusions). In some cases, allogeneic haematopoietic (blood) stem cell transplantation was used to cure the disease. This is a complex procedure where the bone marrow of the patient is cleared of cells and replaced with healthy bone marrow cells from a matched donor, allowing the patient to produce red blood cells with normal haemoglobin.

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients because early data in experimental models suggest it may decrease the amount of excess iron that builds up in the body following repeated transfusions in patients with the condition. 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?

In patients with beta thalassaemia intermedia or major, excess iron can build up in the body following repeated transfusions and damage organs such as the heart and liver. This medicine is a small protein fragment that mimics the action of the hormone hepcidin, which is able to regulate the levels of iron in the body. Hepcidin reduces the amount of iron in the blood by blocking absorption of iron from food and by stopping the release of iron from iron-storage cells. The medicine is therefore expected to correct the iron overload and prevent organ damage due to iron accumulation in patients with beta thalassaemia intermedia or major.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of the medicine in experimental models was ongoing.

At the time of submission of the application for orphan designation, no clinical trials with the medicine in patients with beta thalassaemia intermedia and major had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for beta thalassaemia intermedia and major or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 23 March 2016 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	N-carboxymethyl-glycyl-L-threonyl-L-histidyl-L-3,3-diphenylalanyl-L-piperidincarboxy-3-yl-L-arginyl-L-S-methylthio-cystyl-L-arginyl-L-tryptophyl-aminohexanyl-N-carboxamidomethyl-glycine N-hexadecylamide	Treatment of beta thalassaemia intermedia and major
Bulgarian	N-карбоксиметил-глицил-L-треонил-L-хистидил-L-3,3-дифенилаланил-L-пиперидинкарбокси-3-ил-L-аргинил-L-S-метилтио-цистил-L-аргинил-L-триптофанил-аминохексанил-N-карбоксамидометил-глицин N-хексадециламин	Лечение на бета таласемия интермедия и майор
Croatian	N-carboxymethyl-glycyl-L-threonyl-L-histidyl-L-3,3-diphenylalanyl-L-piperidincarboxy-3-yl-L-arginyl-L-S-methylthio-cystyl-L-arginyl-L-tryptophyl-aminohexanyl-N-carboxamidomethyl-glycine N-hexadecylamide	Liječenje beta-talasemije intermedije i major
Czech	N-carboxymethyl-glycyl-L-threonyl-L-histidyl-L-3,3-diphenylalanyl-L-piperidincarboxy-3-yl-L-arginyl-L-S-methylthio-cystyl-L-arginyl-L-tryptophyl-aminohexanyl-N-carboxamidomethyl-glycine N-hexadecylamide	Léčení beta thalasémie intermedia a major
Danish	N-carboxymethyl-glycyl-L-threonyl-L-histidyl-L-3,3-diphenylalanyl-L-piperidincarboxy-3-yl-L-arginyl-L-S-methylthio-cystyl-L-arginyl-L-tryptophyl-aminohexanyl-N-carboxamidomethyl-glycine N-hexadecylamid	Behandling af beta-thalassæmia intermedia og major
Dutch	N-carboxymethyl-glycyl-L-threonyl-L-histidyl-L-3,3-diphenylalanyl-L-piperidincarboxy-3-yl-L-arginyl-L-S-methylthio-cystyl-L-arginyl-L-tryptophyl-aminohexanyl-N-carboxamidomethyl-glycine N-hexadecylamide	Behandeling van bètathalassemie intermedia en major
Estonian	N-karboksümetüül-glütsüül-L-treonüül-L-histidüül-L-3,3-difenüülalanüül-L-piperidiinkarboksü-3-üül-L-arginüül-L-S-metüültio-tsüstüül-L-arginüül-L-trüptofüül-aminoheksanüül-N-karboksamidometüül-glütsiin N-heksadeküülamiid	Keskmise ja raske beetatalasseemia ravi
Finnish	N-karboksimetyyli-glysyyli-L-treonyyli-L-histidyyli-L-3,3-difenyylialanyyli-L-piperidiinikarboksi-3-yyli-L-arginyyli-L-S-metyylitio-kystyyli-L-arginyyli-L-tryptofyyli-aminoheksanyyli-N-karboksamidometyyli-glysiini N-heksadesyyliamidi	Beetatalassemia intermedia-ja major-tyypin hoito
French	N-carboxyméthyl-glycyl-L-thréonyl-L-histidyl-L-3,3-diphénylalanyl-L-pipéridincarboxy-3-yl-L-arginyl-L-S-méthylthio-cystyl-L-arginyl-L-tryptophyl-aminohexanyl-N-carboxamidométhyl-glycine N-hexadecylamide	Traitement de la bêta-thalassémie intermédiaire et majeure

[1] At the time of designation

Language	Active ingredient	Indication
German	N-carboxymethyl-glycyl-L-threonyl-L-histidyl-L-3,3-diphenylalanyl-L-piperidincarboxy-3-yl-L-arginyl-L-S-methylthio-cystyl-L-arginyl-L-tryptophyl-aminohexanyl-N-carboxamidomethyl-glycine N-hexadecylamid	Behandlung der Beta-Thalassämie (Intermediäre und Major-Form)
Greek	Ν-καρβοξυμεθυλ-γλυκυλ-L-θρεονυλ-L-ιστιδυλ-L-3,3-διφαινυλαλανυλ-L-πιπεριδινκαρβοξυ-3-υλ-L-αργινυλ-L-S-μεθυλθειο-κυστυλ-L-αργινυλΙ-L-τρυπτοφυλ-αμινοεξανυλ-Ν-καρβοξαμιδομεθυλ-γλυκίνη Ν-εξαδεκυλαμίδιο	Θεραπεία της β-μεσογειακής αναιμίας, ενδιάμεσης και μείζονος
Hungarian	N-carboxymethyl-glycyl-L-threonyl-L-histidyl-L-3,3-diphenylalanyl-L-piperidincarboxy-3-yl-L-arginyl-L-S-methylthio-cystyl-L-arginyl-L-tryptophyl-aminohexanyl-N-carboxamidomethyl-glycine N-hexadecylamid	Béta-talasszémia intermedia és major kezelése
Italian	N-carboxymethyl-glycyl-L-threonyl-L-histidyl-L-3,3-diphenylalanyl-L-piperidincarboxy-3-yl-L-arginyl-L-S-methylthio-cystyl-L-arginyl-L-tryptophyl-aminohexanyl-N-carboxamidomethyl-glycine N-hexadecylamide	Trattamento della beta-talassemia intermedia e major
Latvian	N-karboksimetil-glicil-L-treonil-L-histidil-L-3,3-difenilalanil-L-piperidīnkarboksi-3-il-L-arginil-L-S-metiltio-cistil-L-arginil-L-triptofil-aminoheksanil-N-karboksamidometil-glicīna N-heksadecilamīds	Vidēji izteiktas un izteiktas bēta talasēmijas ārstēšana
Lithuanian	N-karboksimetil-glycil-L-treonil-L-histidil-L-3,3-difenilalanil-L-piperidinkarboksi-3-il-L-arginil-L-S-metiltio-cistil-L-arginil-L-triptofil-aminoheksanil-N-karboksamidometil-glicino N-heksadecilamidas	Vidutinio sunkumo ir sunkios β-talasemijos gydymas
Maltese	N-carboxymethyl-glycyl-L-threonyl-L-histidyl-L-3,3-diphenylalanyl-L-piperidincarboxy-3-yl-L-arginyl-L-S-methylthio-cystyl-L-arginyl-L-tryptophyl-aminohexanyl-N-carboxamidomethyl-glycine N-hexadecylamide	Kura tal-beta talassemija intermedja u maġġuri
Polish	N-carboksymetyl-glycyl-L-treonyl-L-histidyl-L-3,3-difenylalanyl-L-piperidincarboksy-3-yl-L-arginyl-L-S-metyltio-cystyl-L-arginyl-L-tryptofyl-aminoheksanyl-N-carboksamidometyl-glicyny N-heksadecylamid	Leczenie talasemii beta-intermedia i major
Portuguese	*N*-hexadecilamida da *N*-carboximetil-glicil-L-treonil-L-histidil-L-3,3-difenilalanil-L-piperidinocarboxi-3-il-L-arginil-L-S-metiltio-cistil-L-arginil-triptofil-aminohexanil-*N*-carboxamidometil-glicina	Tratamento da beta talassémia intermédia e major
Romanian	N-carboximetil-gliiyl-L-treonil-L-histidil-L-3,3-difenilalanil-L-piperidincarboxi-3-il-L-arginil-L-S-metiltio-cistil-L-arginil-L-triptofil-aminohexanil-N-carboxamidometil-glicin N-hexadecilamidă	Tratamentul beta talasemiei intermediare şi majore
Slovak	N-karboxyymetyl-glycyl-L-treonyl-L-histidyl-L-3,3-difenylalanyl-L-piperidínkarboxy-3-yl-L-arginyl-L-S-metyltio-cystyl-L-arginyl-L-tryptofyl-aminohexanyl-N-karboxamidometyl-glycín N-hexadecylamid	Liečba stredne závažnej a závažnej beta talasémie

Language	Active ingredient	Indication
Slovenian	N-karboksimetil-glicil-L-treonil-L-histidil-L-3,3-difenilalanil-L-piperidinkarboksi-3-il-L-arginil-L-S-metiltio-cistil-L-arginil-L-triptofil-aminoheksaanil-N-karboksamidometil-glicine N-heksaadecilamid	Zdravljenje srednje in velike talasemije beta
Spanish	N-carboximethil-glicil-L-treonil-L-histidil-L-3,3-difenilalanil-L-piperidincarboxi-3-il-L-arginil-L-S-metiltio-cistil-L-arginil-L-triptofil-aminohexanil-N-carboxamidomethil-glycine N-hexadecilamide	Tratamiento de la beta talasemia intermedia y mayor
Swedish	N-carboxymethyl-glycyl-L-threonyl-L-histidyl-L-3,3-diphenylalanyl-L-piperidincarboxy-3-yl-L-arginyl-L-S-methylthio-cystyl-L-arginyl-L-tryptophyl-aminohexanyl-N-carboxamidomethyl-glycine N-hexadecylamide	Behandling av beta-thalassaemia intermedia och major
Norwegian	N-carboxymethyl-glycyl-L-threonyl-L-histidyl-L-3,3-diphenylalanyl-L-piperidincarboxy-3-yl-L-arginyl-L-S-methylthio-cystyl-L-arginyl-L-tryptophyl-aminohexanyl-N-carboxamidomethyl-glycine N-hexadecylamide	Behandling av beta-thalassemia intermedia og beta-thalassemia major
Icelandic	N-carboxýmethýl-glýcýl-L-threónýl-L-histidýl-L-3,3-díphenýlalanýl-L-píperidíncarboxý-3-ýl-L-arginýl-L-S-methýlthíó-cýstýl-L-arginýl-L-trýptóphýl-amínóhexanýl-N-carboxamídómethýl-glýcín N-hexadecýlamíð	Meðferð á langvinnu járnofhleðslu sem krefst klómeðferðar