25 September 2012
EMA/COMP/516702/2012
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Humanised monoclonal antibody against P-selectin for the treatment of sickle cell disease

On 9 August 2012, orphan designation (EU/3/12/1034) was granted by the European Commission to Quintiles Ireland Ltd, Ireland, for humanised monoclonal antibody against P-selectin for the treatment of sickle cell disease.

What is sickle cell disease?

Sickle cell disease is a genetic disease in which the red blood cells become rigid and sticky, and change from being disc-shaped to being crescent-shaped (like a sickle). The change in shape is caused by the presence of an abnormal form of haemoglobin, the protein in red blood cells that carries oxygen around the body. In patients with sickle cell disease, the abnormal red blood cells attach to the walls of blood vessels and block them, restricting the flow of oxygen-rich blood to the internal organs such as the heart, lungs and spleen. The abnormal red blood cells have also a shorter life span and release haemoglobin and other toxic molecules into the blood circulation. As a result, patients with the disease have severe pain and damage to multiple organs as well as repeated infections and anaemia (low red blood cell counts).

Sickle cell disease is a severe disease that is long lasting and may be life threatening because of damage to the heart and the lungs, anaemia and infections.

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

At the time of designation, sickle cell disease affected approximately 1.3 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 66,000 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 27), Norway, Iceland and Liechtenstein. This represents a population of 506,300,000 (Eurostat 2011).

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

What treatments are available?

At the time of designation, the only medicine authorised in the EU to treat pain associated with sickle cell disease was hydroxycarbamide. The main treatment for sickle cell disease was blood transfusion and analgesics (medicines to relieve pain). This was usually combined with 'iron chelators' (medicines used to reduce the high iron levels in the body caused by repeated blood transfusions), which are necessary in patients with long-term anaemias such as sickle cell disease. In some cases, haematopoietic (blood) stem cell transplantation was used (a complex procedure where the patient receives stem cells from a matched donor to help restore the bone marrow) to allow the patient to produce red blood cells containing normal haemoglobin.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with sickle cell disease because it works in a different way to existing medicines, which may improve the blood flow to the organs. 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?

This medicine contains a monoclonal antibody (a type of protein), which has been designed to recognise and attach to a protein called 'P-selectin' that is present on the surface of the cells lining the blood vessels. P-selectin is thought to be involved in the attachment of sickle cells and inflammatory cells to the blood vessels, leading to the blockage of the blood flow and pain in patients with sickle cell disease. By attaching to P-selectin, the medicine is expected to prevent the deposit of sickle and inflammatory cells in blood vessels, thereby allowing a better blood flow and improving the symptoms of the disease.

What is the stage of development of this medicine?

The effects of humanised monoclonal antibody against P-selectin have been evaluated in experimental models.

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

At the time of submission, the medicine was not authorised anywhere in the EU for sickle cell disease. Orphan designation of this medicine had been granted in the United States of America for the treatment of vaso-occlusive crisis in patients with sickle cell disease.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 11 July 2012 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

Sponsor's contact details:

Quintiles Ireland Limited
Eastpoint Business Park,
Fairview, Dublin 3
Ireland
Telephone: +353 1 819 51 00
Telefax: +353 1 819 95 11
E-mail: ronan.donelan@quintiles.com

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 monoclonal antibody against P-selectin	Treatment of sickle cell disease
Bulgarian	Хуманизирано моноклонално антитяло, насочено към P-селектин	Лечение на сърповидно-клетъчна анемия
Czech	Humanizovaná monoklonální protilátka cílená proti P-selektinu	Léčba srpkovité anémie
Danish	Humaniseret monoklonalt antistof rettet mod P-selectin	Behandling af seglcellesygdom
Dutch	Gehumaniseerd monoklonaal antilichaam tegen P-selectine	Behandeling van sikkelcelaandoening
Estonian	P-selektiini vastane humaniseeritud monoklonaalne antikeha	Sirprakulise aneemia ravi
Finnish	Humanisoitu monoklonaalinen vasta kohdistaminen P-selektiini	Sirppisolusyndrooman hoito
French	Anticorps monoclonal humanisé ciblé contre la P-sélectine	Traitement de la drépanocytose
German	Humanisierter monoklonaler Antikörper gegen P-Selectin	Behandlung der Sichelzellanämie
Greek	Εξανθρωποποιημένο μονοκλωνικό αντίσωμα έναντι της P-Σελεκτίνης	Θεραπεία της δρεπανοκυτταρικής αναιμίας
Hungarian	P-szelektin ellenes humanizált monoklonális antitest	Sarlósejtes anaemia kezelése
Italian	Anticorpo monoclonale umanizzato diretto contro la P-selectina	Trattamento dell'anemia falciforme
Latvian	Humanizēta monoklonāla antiviela Orientēšanās P-selektīna	Sirpjveida šūnu anēmijas ārstēšana
Lithuanian	Humanizuotas monokloninis antikūnas prieš P-selektiną	Siklemijos gydymas
Maltese	Antikorp monoklonali umanizzat kontra *P-selectin*	Kura tal-marda taċ-ċelluli sura ta' minġel
Polish	Humanizowane przeciwciało monoklonalne przeciw selektynie P	Leczenie niedokrwistości sierpowatej
Portuguese	Anticorpo monoclonal humanizado anti selectina-P	Tratmento do sindrome das células falciformes
Romanian	Anticorp monoclonal umanizat anti P-selectină	Tratamentul anemiei cu celule falciforme
Slovak	Humanizovaná monoklonálna protilátka proti P-selektínu	Liečba kosáčikovej anémie
Slovenian	Humanizirano monoklonsko protitelo proti P-seletinu	Zdravljenje bolezni srpastih celic
Spanish	Anticuerpo monoclonal humanizado contra P-selectina	Tratamiento de la anemia drepanocítica
Swedish	Humaniserad monoklonal antikropp mot P-selektin	Behandling av sickle cell sjukdom
Norwegian	Humanisert monoklonalt antistoff rettet mot P-selektin	Behandling av sigdcellesykdom
Icelandic	Mannaaðlagað einstofna mótefni gegn P-selectíni	Meðferð sigðkornablóðleysis

[1] At the time of designation