6 March 2015
EMA/COMP/89560/2013 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Poloxamer 188 for the treatment of sickle cell disease

First publication	26 March 2013
Rev.1: sponsor's change of address	6 March 2015
Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

On 12 March 2013, orphan designation (EU/3/13/1112) was granted by the European Commission to Theradex (Europe) Ltd, United Kingdom, for poloxamer 188 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 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 less than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 51,000 people*, which was considered to be

*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.
At the time of designation, this represented a population of 512,200,000 (Eurostat 2013).

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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, Siklos (hydroxycarbamide) was authorised in the EU to prevent recurrent, painful vaso-occlusive crises (that happen when blood vessels become blocked) in patients with sickle-cell disease. The main treatment for sickle-cell disease was blood transfusion and analgesics (medicines to relieve pain). This was usually combined with 'iron chelator' 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 early studies suggest it may be used in addition to other treatments to control sudden episodes of blood vessel blockage and reduce their duration. 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?

Poloxamer 188 is a molecule thought to have the ability to attach to and seal the damaged surface of cells. In sickle cell disease, the medicine is thought to attach to sickle cells, reducing their rigidity and their tendency to stick to the walls of blood vessels. This is expected to prevent the blockage of blood vessels by sickle cells, thereby allowing a better blood flow and improving the symptoms of the disease.

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 poloxamer 188 in experimental models was ongoing.

At the time of submission, clinical trials with poloxamer 188 in patients with sickle cell disease were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for sickle cell disease. Orphan designation of poloxamer 188 has been granted in the United States of America for treatment of sickle cell disease (this includes the treatment and prevention of complications of sickle cell disease).

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 6 February 2013 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:

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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	Poloxamer 188	Treatment of sickle cell disease
Bulgarian	Полоксамер 188	Лечение на сърповидно-клетъчна анемия
Czech	Poloxamer 188	Léčba srpkovité anémie
Danish	Poloxamer 188	Behandling af seglcellesygdom
Dutch	Poloxameer 188	Behandeling van sikkelcelaandoening
Estonian	Poloksameer 188	Sirprakulise aneemia ravi
Finnish	Poloksameeri 188	Sirppisolusyndrooman hoito
French	Poloxamer 188	Traitement de la drépanocytose
German	Poloxamer 188	Behandlung der Sichelzellenanämie
Greek	Πολοξαμέρη 188	Θεραπεία της δρεπανοκυτταρικής αναιμίας
Hungarian	Poloxamer 188	Sarlósejtes anaemia kezelése
Italian	Poloxamer 188	Trattamento dell'anemia falciforme
Latvian	Poloksamērs 188	Sirpjveida šūnu anēmijas ārstēšana
Lithuanian	Poloksameras 188	Siklemijos gydymas
Maltese	Poloxamer 188	Kura tal-marda taċ-ċelluli sura ta' minġel
Polish	Poloksamer 188	Leczenie niedokrwistości sierpowatokrwinkowej
Portuguese	Poloxâmero 188	Tratmento do sindrome das células falciformes
Romanian	Poloxamer 188	Tratamentul anemiei cu celule falciforme
Slovak	Poloxamér 188	Liečba kosáčikovej anémie
Slovenian	Poloksamer 188	Zdravljenje bolezni srpastih celic
Spanish	Poloxámero 188	Tratamiento de la anemia drepanocítica
Swedish	Poloxamer 188	Behandling av sickle cell syndrom
Norwegian	Poloksamer 188	Behandling av sigdcellesykdom
Icelandic	Póloxamer 188	Meðferð sigðkornablóðleysis

[1] At the time of designation