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EMA/COMP/48629/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Sevuparin sodium for the treatment of sickle cell disease

On 12 February 2015, orphan designation (EU/3/15/1433) was granted by the European Commission to Dilaforette AB, Sweden, for sevuparin sodium 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 other blood cells and 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. Because the abnormal red blood cells have a shorter life span, they release haemoglobin into the blood circulation rather than carrying it to the internal organs where it is needed. As a result, the disease causes severe pain and damage to these 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 2.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 113,000 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, the only medicine authorised in the EU to treat sickle cell disease was hydroxycarbamide. The main treatment for sickle cell disease was blood transfusion. This was usually combined with 'iron chelators' (medicines used to reduce the high iron levels in the body caused by

*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 512,900,000 (Eurostat 2015).

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 in experimental models suggest that it might treat 'vaso-occlusive crises' (painful episodes caused by sickle-shaped red blood cells obstructing blood vessels and restricting blood flow to an organ). 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?

Sevuparin is expected to work by blocking certain molecules that allow sickle cells to stick to the blood vessels, thereby obstructing them. By blocking these molecules, the medicine is expected to reduce the deposit of sickle cells in blood vessels, thereby allowing better blood flow and improving the symptoms of the disease.

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 sickle cell disease were planned.

At the time of submission, the medicine was not authorised anywhere in the EU for sickle cell disease 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 9 January 2015 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:

Dilaforette AB
Retzius väg 8
SE-171 65 Solna
Sweden
Tel. +46 7 33 75 98 18
E-mail: info@dilaforette.se

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Sevuparin sodium	Treatment of sickle cell disease
Bulgarian	Севупарин натрий	Лечение на сърповидно-клетъчна анемия
Croatian	Sevuparinnatrij	Liječenje bolesti srpastih stanica
Czech	Sevuparin sodný	Léčba srpkovité anémie
Danish	Sevuparinnatrium	Behandling af seglcellesygdom
Dutch	Sevuparin natrium	Behandeling van sikkelcelaandoening
Estonian	Sevuparin-naatrium	Sirprakulise aneemia ravi
Finnish	Sevupariinatrium	Sirppisolusyndrooman hoito
French	Sévuparine sodique	Traitement de la drépanocytose
German	Sevuparin Natrium	Behandlung der Sichelzellenanämie
Greek	Νατριούχος σεβουπαρίνη	Θεραπεία της δρεπανοκυτταρικής αναιμίας
Hungarian	Sevuparin nátrium	Sarlósejtes anaemia kezelése
Italian	Sevuparin sodico	Trattamento dell'anemia falciforme
Latvian	Sevuparīna nātrija sāls	Sirpjveida šūnu anēmijas ārstēšana
Lithuanian	Sevuparino natrio druska	Siklemijos gydymas
Maltese	Sevuparin sodium	Kura tal-marda taċ-ċelluli sura ta' mingel
Polish	Sól sodowa sewuparyny	Leczenie niedokrwistości sierpowatokrwinkowej
Portuguese	Sevuparina sódica	Tratamento do síndrome das células falciformes
Romanian	Sevuparină sodică	Tratamentul anemiei cu celule falciforme
Slovak	Sevuparín sodný	Liečba kosáčikovej anémie
Slovenian	Natrijev sevuparin	Zdravljenje bolezni srpastih celic
Spanish	Sevuparina sodica	Tratamiento de la anemia drepanocítica
Swedish	Sevuparin natrium	Behandling av sickle cell syndrom
Norwegian	Sevuparinnatrium	Behandling av sigdcellesykdom
Icelandic	Sevúparín natríum	Meðferð sigðkornablóðleysis

¹ At the time of designation