



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

14 January 2013
EMA/COMP/390293/2009 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

(S)-3'-(OH)-desazadesferrithiocin-polyether, magnesium salt for the treatment of chronic iron overload requiring chelation therapy

On 24 July 2009, orphan designation (EU/3/09/647) was granted by the European Commission to FerroKin BioSciences Ltd, United Kingdom, for (S)-3'-(OH)-desazadesferrithiocin-polyether, magnesium salt for the treatment of chronic iron overload requiring chelation therapy.

The sponsorship was transferred to Shire Pharmaceutical Development Limited, United Kingdom, in July 2012.

What is chronic iron overload requiring chelation therapy?

Chronic iron overload is a disease in which there is an excess of iron in the body. The body does not have a natural way of removing excess iron, which can build up and damage organs such as the heart and liver. Patients with chronic iron overload may need 'chelation therapy'. This is treatment with 'iron chelators', substances that attach to and remove the excess iron from the body.

Chronic iron overload mainly results from too much iron being absorbed from the diet (known as haemochromatosis) or the patient receiving high levels of iron through repeated blood transfusions. Repeated transfusions are necessary in patients with long-term anaemias (diseases causing low red blood cell counts) such as thalassaemia and sickle cell anaemia.

Chronic iron overload is a serious debilitating disease that may be life threatening because of the severe damage to the heart and liver. It may result in shortened life expectancy.

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

At the time of designation, chronic iron overload requiring chelation therapy affected approximately 2.3 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 116,000 people, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and 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 504,800,000 (Eurostat 2009).



What treatments are available? <or> What methods of <prevention / diagnosis> are available?

At the time of designation, iron chelators were authorised in the EU for the treatment of chronic iron overload in patients with anaemias that need blood transfusion. The first-choice treatment for patients with haemochromatosis was phlebotomy (removal of some blood from the body), although this was not possible in all patients.

(S)-3'-(OH)-desazadesferrithiocin-polyether, magnesium salt is an iron chelator. The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with chronic iron overload requiring chelation therapy because preliminary studies in experimental models have shown that it might have an improved action compared to other iron chelators. 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?

(S)-3'-(OH)-desazadesferrithiocin-polyether, magnesium salt is an iron chelator that is to be given by mouth once a day. As with other iron chelators, this medicine is expected to work by attaching to excess iron in the body to form a compound called a 'chelate'. The chelate can then be excreted by the body, mainly in the stools. This may help to correct the iron overload and prevent damage due to excess iron.

What is the stage of development of this medicine?

The effects of (S)-3'-(OH)-desazadesferrithiocin-polyether, magnesium salt have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials in patients with this medicine had been started.

At the time of submission, this medicine was not authorised anywhere in the EU for chronic iron overload. Orphan designation of this medicine had been granted in the United States of America for chronic iron overload in patients with transfusion-dependent anaemias.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 4 June 2009 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:

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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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	(S)-3'-(OH)-desazadesferrithiocin-polyether, magnesium salt	Treatment of chronic iron overload requiring chelation therapy
Bulgarian	(S)-3'-(OH)-дезазадесферитиоцин-полиетер, магнезиева сол	За лечение на хронично претоварване с желязо, изискващо хелатираща терапия
Czech	(S)-3'-(OH)-desazadesferithiocin-polyéter, magnéziová sůl	Léčba chronického nadbytku železa vyžadující chelační terapii
Danish	(S)-3'-(OH)-desazadesferrithiocin-polyether, magnesiumsalt	Behandling af kronisk jern overskud, der nødvendiggør chelat behandling
Dutch	(S)-3'-(OH)-desazadesferrithiocinepolyether, magnesiumzout	Behandeling van chronische ijzerstapeling welke chelatatie therapie vergt
Estonian	(S)-3'-(OH)-desasadesferritiotsiini polüeeter, magneesiumisool	Kroonilise, kelaativat ravi vajava raua ülekoormuse ravi
Finnish	(S)-3'-(OH)-desatsadesferritiosiini-polyeetteri, magnesiumsuola	Kroonisen, kelatointihoitoa vaativan raudan liikavarastoitumisen hoito
French	polyéther désaza-desferrithiocine (S)-3'-(OH), sel de magnésium	Traitement de la surcharge ferrique chronique nécessitant un traitement chélateur
German	(S)-3'-(OH)-desaza-desferrithiocin-polyether, Magnesiumsalz	Behandlung einer Eisenüberladung die eine Chelattherapie benötigt
Greek	Άλας μαγνησίου του (S)-3'-(OH)-δεσαζαδεσφερριθειοσινο-πολυαιθέρα	θεραπεία της χρόνιας υπερφόρτωσης σιδήρου που χρειάζεται θεραπεία με χηλικούς παράγοντες
Hungarian	(S)-3'-(OH)-desazadesferrithiocin-poliéter, magnézium só	Olyan krónikus vastúlterhelés kezelésére, amely kelátképző terápiát igényel
Italian	(S)-3'-OH-deazadesferrithiocina-polietera, sale di magnesio	Trattamento dell'accumulo cronico di ferro che necessita di terapia chelante
Latvian	(S)-3'-(OH)-desazadesferitiocīna poliētera, magnija sāls	Hroniskas organisma pārsātināšanas ar dzelzi , kam nepieciešama helātterapija ārstēšana
Lithuanian	(S)-3'-(OH)-desazadesferitiocino polieteris, magnio druska	Lėtinio geležies pertekliaus gydymas, reikalaujantis chelatų terapijos
Maltese	(S)-3'-(OH)-desazadesferrithiocin-polyether, melh tal-manjesju	Kura ta' tagħbija żejda żejda kronika ta' ħadid li teħtiegħ terapija kelanti
Polish	Polieter (S)-3'-(OH)-deazadeferritiocynowy, sól magnezowa	Leczenie przewlekłego obciążenia żelazem wymagającego stosowania środków chelatujących
Portuguese	(S)-3'-(OH)-desazadesferrithiocin-poliéter, sal de magnésio	Tratamento da sobrecarga crónica de ferro que necessita de uma terapia quelante
Romanian	Sare de magneziu (S)-3'-(OH)-deazadeferitiocină-polieter	Tratamentul supraîncărcării cronice cu fier, care necesită terapie chelatoare

¹ At the time of designation

Language	Active ingredient	Indication
Slovak	(S)-3'-(OH)-desazadesferitiocín-polyéter, horečnatá soľ	Liečba chronického nadbytku železa, ktorá si vyžaduje liečbu chelátmi
Slovenian	(S)-3'-(OH)-desazadesferitiocin-polieter, magnezijeva sol	Zdravljenje kronične preobremenitve z železom, ki zahteva zdravljenje s kelati.
Spanish	(S)-3'-(OH)-desazadesferritiocin-poliéter, sal de magnesio	Tratamiento de la sobrecarga crónica de hierro que necesita de terapia quelante.
Swedish	(S)-3'-(OH)-desazadesferritiocinpolyeter, magnesiumsalt	Behandling av kronisk järnupplagring som kräver kelatterapi
Norwegian	(S)-3'-(OH)-desazadesferritiosin-polyeter, magnesiumsalt	Behandling av kronisk jernoverskudd, som krever kelatering
Icelandic	(S)-3'-(OH)- desazadesferrítíósín-pólýeter, magnesíumsalt	Behandling av kronisk jernoverskudd som krever kelatorbehandling