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EMA/COMP/417562/2013
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Dexamethasone sodium phosphate encapsulated in human autologous erythrocytes for the treatment of ataxia telangiectasia

On 17 July 2013, orphan designation (EU/3/13/1158) was granted by the European Commission to Erydel S.p.A., Italy, for dexamethasone sodium phosphate encapsulated in human autologous erythrocytes for the treatment of ataxia telangiectasia.

What is ataxia telangiectasia?

Ataxia telangiectasia is an inherited disorder that affects the part of the brain that controls balance and movement, leading to an increasing inability to control movement (ataxia) and other neurological symptoms. The disorder also affects the immune system, increasing patients' risk of infections and certain blood cancers. Patients may also have telangiectasias, which are clusters of dilated blood vessels, which may occur in the white of the eyes.

Ataxia telangiectasia is life-threatening and debilitating due to the progressive destruction of parts of the brain and increased risk of infections and cancers.

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

At the time of designation, ataxia telangiectasia affected approximately 0.1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 5,000 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, there were no medicinal products authorised for treating ataxia telangiectasia. Treatments were used to alleviate the symptoms but none were satisfactory.

*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 509,000,000 (Eurostat 2013).

How is this medicine expected to work?

Dexamethasone sodium phosphate encapsulated in human autologous erythrocytes is intended to deliver low and persistent levels of the anti-inflammatory medicine dexamethasone. The dexamethasone is enclosed within red blood cells (erythrocytes) taken from the patient's own blood. When given to the patient by infusion (drip), it is expected to reduce the inflammatory processes associated with ataxia telangiectasia and thereby to alleviate the symptoms of the disease and slow down its progression.

What is the stage of development of this medicine?

The effects of the medicinal product have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the medicinal product in patients with ataxia telangiectasia were ongoing.

At the time of submission, the medicinal product was not authorised anywhere in the EU for ataxia telangiectasia. Orphan designation had been granted in the United States for the condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 June 2013 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:

EryDel SpA
Via Sasso 36
61029 Urbino
Italy
Telephone: +39 072 2378 711
Telefax: +39 0792 2328 166
E-mail: info@erydel.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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Dexamethasone sodium phosphate encapsulated in human autologous erythrocytes	Treatment of ataxia telangiectasia
Bulgarian	Дексаметазон натриев фосфат за инкапсулиране в човешки автоложни еритроцити	Лечение на атаксия телангиектазия
Czech	Dexamethason sodium fosfát enkapsulovaný v humánních autologních erytrocytech	Léčba ataxie telangiectasie
Croatian	Natrijev deksametazonfosfat inkapsuliran u ljudske autologne eritrocite	Liječenje ataksije-teleangiektazije
Danish	Dexametasonnatriumfosfat enkapsulerat i humana autologa erythrocyter	Behandling af ataxia telangiectasia
Dutch	Dexamethasone natriumfosfaat geïncapsuleerd in humane autologe erythrocyten	Behandeling van ataxia telangiëctasie
Estonian	Deksametasoon-naatriumfosfaat, mis kapseldatud inimese autoloogsetes erütrotsüütides	Teleangiiektaasataksia ravi
Finnish	Autologisiin ihmisen erytrosyytteihin kapseloitava deksametasoninatriumfosfaatti	Ataksia-teleangiektasian hoito
French	Phosphate sodique de dexamethasone encapsulé dans des érythrocytes autologues humains.	Traitement de l'ataxie télangiectasie
German	Dexamethason Natriumphosphat verkapselt in humanen autologen Erythrozyten	Behandlung der Ataxia teleangiectatica/Louis-Bar-Syndrom
Greek	Νατριούχος φωσφορική δεξαμεθαζόνη ενθυλακωμένη σε ανθρώπινα αυτόλογα ερυθροκύτταρα	Θεραπεία της αταξίας-τηλαγγειεκτασίας
Hungarian	Humán autológ vörösvérsejtbe zárt dexametazon nátrium foszfát	Ataxia-telangiectasia kezelése
Italian	Dexametasone sodio fosfato incapsulato in eritrociti umani autologhi	Trattamento della Ataxia telangiectasia
Latvian	Nātrija fosfāta deksametazons iekapsulēšanai cilvēka autologos eritrocītos	Teleangektāziskās ataksijas ārstēšana
Lithuanian	Deksametazono natrio fosfatas inkapsuliuotas į žmogaus autologinius eritrocitus	Ataksijos telangiektazijos gydymas
Maltese	Dexamethasone sodium phosphate inkapsulat f'eritroċiti umani awtologi	Kura tal-atassja telangektasija

¹ At the time of designation

Language	Active ingredient	Indication
Polish	Deksametazonu sodu fosforan do zamykania w ludzkich erytrocytach autologicznych	Leczenie zespołu ataksji-teleangiectazji
Portuguese	Fosfato de dexametasona e sódio encapsulado em eritrócitos autólogos humanos	Tratamento da ataxia-telangiectasia
Romanian	Dexametazonă fosfat disodic incapsulată în eritrocite umane autologe	Tratamentul ataxiei telangiectaziei
Slovak	Dexametazón natriumfosfát enkapsulovaný v ľudských autológnych erytrocytoch	Liečba ataxie teleangiectázie
Slovenian	Deksametazon natrijev fosfat, enkapsuliran v humane avtologne eritrocite	Zdravljenje ataksija-teleangiopatije
Spanish	Fosfato sódico de dexametasona encapsulado en eritrocitos autólogos humanos	Tratamiento de la ataxia telangiectasia
Swedish	Dexametasonnatriumfosfat enkapsulerat i humana autologa erythrocyter	Behandling av ataxia telangiectasia
Norwegian	Deksametasonnatriumfosfat enkapsulert i humane autologe erythrocytter	Behandling av ataxia telangiectasia
Icelandic	Dexametasón natríum fósfat komið fyrir í samgena manna rauðum blóðkornum	Meðferð á ataxia telangiectasia