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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

dexamethasone sodium phosphate encapsulated in human erythrocytes for the treatment of cystic fibrosis

On 20 October 2004, orphan designation (EU/3/04/230) was granted by the European Commission to Dideco S.p.A., Italy for dexamethasone sodium phosphate encapsulated in human erythrocytes for the treatment of cystic fibrosis.

The sponsor changed name to Sorin Group Italia S.r.l., Italy, in December 2005 and subsequently to Erydel S.p.A., Italy, in January 2010.

What is cystic fibrosis?

Cystic fibrosis is an inherited disease. The genetic information that determines the characteristics of each individual is carried by genes located on structures called chromosomes. In humans, each cell has 23 pairs of chromosomes. For each pair one chromosome is inherited from the mother and the other from the father. Cystic fibrosis is caused by abnormalities of a specific gene, called cystic CFTR, carried by the seventh pair of chromosomes. Cystic fibrosis appears only when the CFTR is abnormal on both chromosomes of the seventh pair. The CFTR gene is responsible for the production of a protein that regulates outflow of water and salts (like chloride) from cells that cover internal and external surfaces of the body, the so-called epithelial cells. The defective transport of water and salts due to the lack of the regulatory protein, results in the thickening of the secretions (mucous) in several organs (e.g. lungs, pancreas). This leads to reduced functioning and chronic infection of the lungs and chronic inflammation (a body response to the injury caused to the tissue). In the long run, these events can induce damage to the lung tissue and the disease can become life-threatening.

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

At the time of designation, cystic fibrosis affected approximately 1.3 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 60,000 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 25), Norway, Iceland and Liechtenstein. This represents a population of 459,700,000 (Eurostat 2004).

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What treatments are available?

At the time of submission of the application for the orphan drug designation lung infection and inflammation in cystic fibrosis were treated mainly with antibiotics. These can be taken in a number of ways such as through the mouth, through a vein or they can be inhaled as a fine mist of particles. Associated treatments included daily exercise and physical therapies and several other types of medications such as pancreatic enzymes and food supplements. Bronchodilators are medications that can enlarge the lumen of the airways. Mucolytics help to dissolve the secretions. Still other medications were used to fight the inflammation. Dexamethasone sodium phosphate encapsulated in human erythrocytes might be of potential significant benefit for the treatment of cystic fibrosis because it can act in a different way than other available treatments. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

Dexamethasone sodium phosphate encapsulated in human erythrocytes belongs to a group of substances called corticosteroids that have an anti-inflammation activity. Dexamethasone sodium phosphate is supposed to act by reducing the chronic inflammation present at the level of the lungs. This product acts by reducing the release of substances involved in inflammation called cytokines from the white blood cells. The dexamethasone salt is wrapped (encapsulated) in red blood cells (human erythrocytes) as they have the function of delivering low quantities of dexamethasone over a longer period of time.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, clinical trials in patients with cystic fibrosis were ongoing.

Dexamethasone sodium phosphate for encapsulated in human erythrocytes was not marketed anywhere worldwide for cystic fibrosis or designated as orphan medicinal product elsewhere for this condition at the time of submission.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 9 September 2004 a positive opinion recommending the grant of the above-mentioned 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 European Union) 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

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Translations of the active ingredient and indication in all official EU languages, Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Dexamethasone sodium phosphate encapsulated in human erythrocytes	Treatment of cystic fibrosis
Czech	Dexametason natriumfosfát v erytrocytárních humánních kapslích	Léčba cystické fibrózy
Danish	Dexamethasonnatriumphosphat indkapslet i humane erythrocytter	Behandling af cystisk fibrose
Dutch	Dexamethasone natriumfosfaat geïncapsuleerd in humane erythrocyten	Behandeling van cystische fibrose
Estonian	Deksametasoon-naatriumfosfaat kapseldatuna inimese erütrotsüüti	Tsüstilise fibroosi ravi
Finnish	Humaanierytrosyytteihin kapseloitu Deksametasoni-natriumfosfaatti	Kystisen fibroosin hoito
French	Phosphate de dexaméthasone sodique encapsulé dans des érythrocytes humains.	Traitement de la mucoviscidose
German	Dexamethason-Natriumphosphat eingekapselt in humanen Erythrozyten	Behandlung von zystischer Fibrose
Greek	Δεξαμεθαζόνη, άλας φωσφορικού νατρίου, έγκλειστη σε ανθρώπινα ερυθροκύτταρα	Θεραπεία της κυστικής ίνωσης
Hungarian	Dexamethasone nátrium-foszfát humán erythrocytába zárva	Cisztikus fibrózis kezelése
Italian	Desametasone sodio fosfato incapsulato in eritrociti umani	Trattamento della fibrosi cistica
Latvian	Cilvēka eritrocītos iekapsulēts deksametazona nātrija fosfāts	Cistiskās fibrozes ārstēšana
Lithuanian	Įkapsuliuotas į žmogaus eritrocitus deksametazono natrio fosfatas	Cistinės fibrozės gydymas
Maltese	Dexamethasone sodium phosphate encapsulated in human erythrocytes	Treatment of cystic fibrosis
Polish	Deksametazonu sodu fosforan zamknięty w ludzkich erytrocytach	Leczenie zwłóknienia torbielowatego
Portuguese	Fosfato sódico de dexametasona encapsulado em eritrócitos humanos	Tratamento da fibrose quística
Slovak	Dexamethasoni natrii phosphas umiestnený do ľudských erytrocytov	Terapia cystickej fibrózy
Slovenian	Deksametazon natrijev fosfat inkapsuliran v humanih eritrocitih	Zdravljenje cistične fibroze
Spanish	Fosfato sódico de dexametasona encapsulado en eritrocitos humanos	Tratamiento de la fibrosis quística
Swedish	Dexametasonnatriumfosfat inkapslad i humana eytrocyter	Behandling av cystisk fibros

Language	Active Ingredient	Indication
Norwegian	Deksametasonnatriumfosfat innkapslet i humane erytrocytter	Behandling av cystisk fibrose
Icelandic	Dexametasón natríumfosfat komið fyrir í rauðum blóðkornum	Meðferð við slímseigjusjúkdómi