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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Levofloxacin hemihydrate for the treatment of cystic fibrosis

First publication	24 April 2009
Rev.1: sponsor's change of address	24 May 2011
Rev.2: transfer of sponsorship	12 September 2013
Rev.3: withdrawal from the Community Register	17 April 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.	

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in February 2015 on request of the Sponsor.

On 23 September 2008, orphan designation (EU/3/08/566) was granted by the European Commission to Mpex London Ltd, United Kingdom, for levofloxacin hemihydrate for the treatment of cystic fibrosis.

The sponsorship was transferred to Aptalis Pharma SAS, France, in July 2013.

What is cystic fibrosis?

Cystic fibrosis is a hereditary (genetic) disease that affects the production of secretions (such as mucus) from the glands in the body. It affects the lungs and the digestive system (gut) in particular. Cystic fibrosis is caused by abnormalities in a gene called 'cystic fibrosis transmembrane conductance regulator' (CFTR). The CFTR gene is responsible for the production of CFTR, a protein that regulates the production of mucus and digestive juices by acting as a chloride ion channel to allow proper movement of salt and water in and out of certain cells in the lungs and other tissues. In patients with cystic fibrosis, there is an overproduction of mucus in the lungs and a reduced production of digestive juices from the pancreas (an organ near the stomach). This leads to long-term infection and inflammation of the lungs and problems with the digestion and absorption of food resulting in poor growth.

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Cystic fibrosis is a long lasting and life-threatening disease.

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 was equivalent to a total of around 65,000 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 submission of the application for orphan drug designation, lung infection and inflammation in cystic fibrosis were mainly treated with physiotherapy and antibiotics. Other medicines used to treat the lung disease included bronchodilators (medicines that help to open up the airways in the lungs) and mucolytics (medicines that help dissolve the mucus in the lungs). In addition, patients are often given other types of medicine such as pancreatic enzymes (substances that help to digest and absorb food) and food supplements. They are also advised to exercise and to undergo physiotherapy.

The sponsor has provided sufficient information to show that levofloxacin hemihydrate might be of significant benefit for the patients because it could improve the treatment of lung infection in cystic fibrosis and could be inhaled quickly. 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?

Levofloxacin hemihydrate is an antibiotic that belongs to the group 'fluoroquinolones'. It works by blocking an enzyme that bacteria use to make more DNA. By doing this, it stops the bacteria that are causing an infection from growing and multiplying. Levofloxacin hemihydrate is currently used to treat various infections as an intravenous infusion or tablets. This sponsor is developing a product containing levofloxacin hemihydrate for inhalation for the treatment of lung infections in patients with cystic fibrosis.

What is the stage of development of this medicine?

The effects of levofloxacin hemihydrate have been evaluated in experimental models.

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

The medicinal product was not authorised anywhere in the world for cystic fibrosis at the time of submission. Orphan designation of this product had been granted in the United States of America for the treatment of lung infections due to *Pseudomonas aeruginosa* and other bacteria in patients with cystic fibrosis.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 July 2008 recommending the granting of this designation.

[*] 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.
At the time of designation, this represented a population of 502,800,000 (Eurostat 2008).

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	Levofloxacin hemihydrate	Treatment of cystic fibrosis
Bulgarian	Левофлоксацин хемихидрат	Лечение на кистозна фиброза
Croatian	Levofloksacin hemihidrat	Liječenje cistične fibroze
Czech	Levofloxacin hemihydrate	Léčba cystické fibrózy
Danish	Levofloxacin-hemihydrat	Behandling af cystisk fibrose
Dutch	Levofloxacine hemihydraat	Behandeling van cystische fibrose
Estonian	Levofloksatsiinhemihüdraat	Tsüstilise fibroosi ravi
Finnish	Levofloksasiinihemihydraatti	Kystisen fibroosin hoito
French	Lévofloxacine hémihydrate	Traitement de la mucoviscidose
German	Levofloxacin- hemihydrat	Behandlung zystischer Fibrose
Greek	Λεβοφλοξακίνη ημιϋδρική	Θεραπεία της κυστικής ίνωσης
Hungarian	Levofloxacin-hemihidrát	Cisztikus fibrózis kezelése
Italian	Levofloxacina emiidrato	Trattamento della fibrosi cistica
Latvian	Levofloksacīna hemihidrāts	Cistiskās fibrozes ārstēšana
Lithuanian	Levofloksacino hemihidratas	Cistinės fibrozės gydymas
Maltese	Levofloxacin hemihydrate	Kura tal-fibrożi ċistiku
Polish	Półwodzian lewofloksacyny	Leczenie zwłóknienia torbielowatego
Portuguese	Levofloxacina hemi-hidratada	Tratamento da fibrose quística
Romanian	Levofloxacină hemihidrat	Tratamentul fibrozei chistice
Slovak	Hemihydrát levofloxacínu	Terapia cystickej fibrózy
Slovenian	Levofloksacinijev hemihidrat	Zdravljenje cistične fibroze
Spanish	Levofloxacino hemihidrato	Tratamiento de la fibrosis quística
Swedish	Levofloxacinhemihydrat	Behandling av cystisk fibros
Norwegian	Levofloksacinhemihydrat	Behandling av cystisk fibrose
Icelandic	Levófloxacín hemihýdrat	Meðferð við slímseigjusjúkdómi

[1] At the time of transfer of sponsorship