



26 June 2014  
EMA/COMP/42574/2004 Rev.2  
Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

Temocillin sodium for the treatment of *Burkholderia cepacia* lung infection in cystic fibrosis

First publication	23 September 2004
Rev.1: sponsor's change of address	14 March 2013
Rev.2: transfer of sponsorship	26 June 2014
<b>Disclaimer</b> 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.	

On 14 January 2004, orphan designation (EU/3/03/183) was granted by the European Commission to Belpharma N.V, Belgium, for temocillin sodium for the treatment of *Burkholderia cepacia* lung infection in cystic fibrosis.

The sponsorship was transferred to Eumedica N.V., Belgium, in May 2014.

### What is *Burkholderia cepacia* lung infection in cystic fibrosis?

Cystic fibrosis is a genetic 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 CFTR, carried by the 7<sup>th</sup> pair of chromosomes. 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. Cystic fibrosis appears only when the CFTR is abnormal on both chromosomes of the 7<sup>th</sup> pair. The defective transport of water and salts due to the lack of the regulatory protein results in the thickening of the secretions in several organs (e.g. lungs, pancreas). This leads to chronic infection of the lungs and chronic inflammation (a response to the injury caused to the tissue). *Burkholderia cepacia* is a bacteria (micro-organisms that can cause certain types of infections). Infection of the lung with *Burkholderia cepacia* is a severe condition that can complicate cystic fibrosis. It can damage the lung tissue and cause respiratory insufficiency (failure of the lungs to



provide adequately oxygen to the cells of the body and to remove the excess of carbon dioxide from them), which is life threatening.

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

At the time of designation, *Burkholderia cepacia* lung infection in cystic fibrosis affected approximately 1.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 70,000 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?**

*Burkholderia cepacia* lung infection in cystic fibrosis is treated mostly with antibiotics (drugs that kill micro-organisms) (e.g. meropenem and ceftazidime). At the time of submission of the application for orphan designation, antibiotics have not been specifically authorised for this condition in the Community. Satisfactory argumentation has been submitted by the sponsor to justify the assumption that temocillin sodium might be of potential significant benefit for the treatment of *B. cepacia* lung infection in cystic fibrosis, particularly in regards to the narrow spectrum of activity of the medicinal product. This means in fact, that the use of temocillin sodium would not increase the risk of appearance of antibiotic resistances among infectious bacteria. The assumption of benefit is yet to be validated and 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?**

Bacteria have an external wall with vital functions. This wall is formed by a complex structure in which several different molecules should be produced and assembled to end in the final structure. Temocillin sodium belongs to the group of drugs called penicillins. Penicillins prevent the linkage of some of the structures of the bacterial wall, resulting in an abnormal wall, unable to perform the vital functions of the bacteria, that finally will lead to the death of the bacteria.

### **What is the stage of development of this medicine?**

At the time of the submission of the application for orphan designation temocillin sodium was authorised in three member states of the European community for the treatment of conditions other than the applied for orphan designation.

No clinical trials in patients with *Burkholderia cepacia* lung infection in cystic fibrosis were initiated.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 5 December 2003 recommending the granting of this designation.

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\*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. At the time of designation, this represented a population of 464,200,000 (Eurostat 2004).

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:

Eumedica N.V.  
Avenue Winston Churchill 67  
1180 Brussels  
Belgium  
Tel. +32 64 271725  
Fax +32 64 311558  
E-mail: [regulatory@eumedica.com](mailto:regulatory@eumedica.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 languages<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Temocillin sodium	Treatment of <i>Burkholderia cepacia</i> lung infection in cystic fibrosis
Bulgarian	Натриев темоцилин	Лечение на белодробна инфекция причинена от <i>Burkholderia Cepacia</i> при пациенти с муковисцидоза
Croatian	Temocilinnatrij	Liječenje <i>Burkholderia cepacia</i> infekcije pluća kod cistične fibroze
Czech	Temocillin sodný	Léčba plicní infekce <i>Burkholderia cepacia</i> u cystické fibrózy
Danish	Temocillin natrium	Behandling af <i>Burkholderia cepacia</i> lungeinfektion ved cystisk fibrose
Dutch	Temocillin natrium	Behandeling van <i>Burkholderia cepacia</i> longinfectie bij cystische fibrose
Estonian	Temotsilliin naatrium	<i>Burkholderia cepacia</i> kopsuinfektsiooni ravi tsüstilise fibroosi haigetel
Finnish	Temosilliinatrium	<i>Burkholderia cepacia</i> aiheuttaman keuhkoinfektion hoito kystisessä fibroosissa
French	Témocilline disodique	Traitement de l'infection pulmonaire causée par <i>Burkholderia cepacia</i> chez les patients atteints de mucoviscidose
German	Temocillin Natrium	Behandlung von <i>Burkholderia cepacia</i> Lungeninfektionen in Mukoviscidose
Greek	Τεμοκιλλίνη - άλας νατρίου	Θεραπεία ασθενών με πνευμονική λοίμωξη από <i>Burkholderia cepacia</i> κατά την κυστική ίνωση
Hungarian	Temocillin nátrium	<i>Burkholderia cepacia</i> okozta tüdő fertőzés kezelése tisztás fibrózisban
Italian	Temocillina sodica	Treatmento di infezione polmonare da <i>Burkholderia cepacia</i> nella fibrosi cistica
Latvian	Temocilīna nātrija sāls	<i>Burkholderia cepacia</i> plaušu infekcijas ārstēšana pacientiem ar cistisko fibrozi
Lithuanian	Temocilino natris	Plaučių infekcijos, sukeltos <i>Burkholderia cepacia</i> , gydymas sergant cistine fibroze
Maltese	Temocillin sodium	Kura ta' infezzjoni fil-pulmun mill- <i>Burkholderia cepacia</i> fil-fibrozi čistiku
Polish	Temocyлина sodu	Leczenie zakażenia płuc wywołanego <i>Burkholderia cepacia</i> u chorych na mukowiscydozę
Portuguese	Temocilina sódica	Tratamento da infecção pulmonar por <i>Burkholderia cepacia</i> na fibrose quística
Romanian	Temocillin sodicsodium	Tratamentul infectiei pulmonare cu <i>Burkholderia cepacia</i> in fibroza chistica
Slovak	Temocillin sodný	Liečba infekcie pľúc spôsobenej <i>Burkholderia cepacia</i> u pacientov s cystickou fibrózou

<sup>1</sup> At the time of transfer of sponsorship

Language	Active ingredient	Indication
Slovenian	Temocilin natrij	Zdravljenje okužbe pljuč z <i>Burkholderia cepacia</i> pri cistični fibrozi
Spanish	Temocilina sódica	Tratamiento de la infección pulmonar causada por <i>Burkholderia cepacia</i> en pacientes con fibrosis quística
Swedish	Temocillin natrium	Behandling av <i>Burkholderia cepacia</i> luftvägsinfektion vid cystisk fibros