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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

N-[[(5S)-3-(3-fluoro-4-thiomorpholin-4-ylphenyl)-2-oxo-1,3-oxazolidin-5-yl]methyl]acetamide for the treatment of tuberculosis

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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.	

On 30 August 2011, orphan designation (EU/3/11/897) was granted by the European Commission to Pfizer Limited, United Kingdom, for N-[[(5S)-3-(3-fluoro-4-thiomorpholin-4-ylphenyl)-2-oxo-1,3-oxazolidin-5-yl]methyl]acetamide for the treatment of tuberculosis.

The sponsorship was transferred to RLM Consulting sprl, Belgium, in October 2014.

What is tuberculosis?

Tuberculosis (TB) is an infectious disease caused by bacteria called *Mycobacterium tuberculosis*. People become infected by inhaling infected droplets from the cough or sneeze of people who have the disease. TB primarily affects the lungs (called pulmonary TB) but it can also spread to other parts of the body, such as the bones or the nervous system. The symptoms of TB include persistent cough, fever, weight loss and night sweats. Not every person infected will develop the symptoms of the disease.

TB is a long-term debilitating disease that may be life threatening, mainly because of the severe damage to the lungs that does not allow the patient to breathe normally, and because the bacteria causing the disease are often resistant to existing treatments.



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

At the time of designation, TB affected approximately 2.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 117,000 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, several antibiotics were authorised in the EU to treat TB. These were used in combination and for long periods of time, normally at least six months.

The sponsor has provided sufficient information to show that N-{{[(5S)-3-(3-fluoro-4-thiomorpholin-4-ylphenyl)-2-oxo-1,3-oxazolidin-5-yl]methyl}acetamide might be of significant benefit for patients with TB because it works in a different way to existing treatments and early studies indicate that it may improve the outcome of patients, particularly for TB strains which are resistant to several existing antibiotics. 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?

This medicine belongs to the class of oxazolidinones, which are antibiotics that have been used to treat various bacterial infections for several years. Oxazolidinones work by preventing the bacterial cells from producing proteins, and the bacteria eventually die. The medicine does this by attaching to the '50S subunit' of ribosomes in the bacterial cell (ribosomes are the parts of the cell that produce proteins). This medicine is also expected to work against *M. tuberculosis* bacteria that are resistant to other antibiotics.

What is the stage of development of this medicine?

The effects of this medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with this medicine in patients with TB were ongoing.

At the time of submission, this medicine was not authorised anywhere in the EU for TB. Orphan designation of this medicine had been granted in the United States for the treatment of TB.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 10 June 2011 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 507,700,000 (Eurostat 2011).

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:

RLM Consulting sprl
Chemin du Cyclotron, 6
B-1348 Louvain-la-Neuve
Belgium
Tel. +32 10 390 084
Fax +32 10 390 001
E-mail: rlm.consulting@skynet.be

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	N-{{[(5S)-3-(3-fluoro-4-thiomorpholin-4-ylphenyl)-2-oxo-1,3-oxazolidin-5-yl]methyl}acetamide	Treatment of tuberculosis
Bulgarian	N-{{[(5S)-3-(3-флуоро-4-тиоморфолин-4-илфенил)-2-оксо-1,3-оксазолидин-5-ил]метил}ацетамид	Лечение на туберкулоза
Croatian	N-{{[(5S)-3-(3-fluoro-4-tiomorfolin-4-ilfenil)-2-okso-1,3-oksazolidin-5-il]metil}acetamid	Liječenje tuberkuloze
Czech	N-{{[(5S)-3-(3-fluoro-4-thiomorfolin-4-ylfenyl)-2-oxo-1,3-oxazolidin-5-yl]metyl}acetamid	Léčba tuberkulózy
Danish	N-{{[(5S)-3-(3-fluor-4-thiomorpholin-4-ylphenyl)-2-oxo-1,3-oxazolidin-5-yl]methyl}acetamid	Behandling af tuberkulose
Dutch	N-{{[(5S)-3-(3-fluoro-4-thiomorpholin-4-ylphenyl)-2-oxo-1,3-oxazolidin-5-yl]methyl}acetamide	Behandeling van tuberculose
Estonian	N-{{[(5S)-3-(3-fluoro-4-tiomorfoliin-4-üülfenüül)-2-okso-1,3-oksasolidiin-5-üül]metüül}atsetamiid	Tuberkuloosi ravi
Finnish	N-{{[(5S)-3-(3-fluoro-4-tiomorfolin-4-yylifenyyl)-2-okso-1,3-oksatsolidin-5-yyli]metyyli}asetamidi	Tuberkuloosin hoito
French	N-{{[(5S)-3-(3-fluoro-4-thiomorpholin-4-ylphényl)-2-oxo-1,3-oxazolidin-5-yl]méthyl}acétamide	Traitement de la tuberculose
German	N-{{[(5S)-3-(3-fluoro-4-thiomorpholin-4-ylphenyl)-2-oxo-1,3-oxazolidin-5-yl]methyl}acetamid	Behandlung der Tuberkulose
Greek	N-{{[(5S)-3-(3-fluoro-4-thiomorpholin-4-ylphenyl)-2-oxo-1,3-oxazolidin-5-yl]methyl}acetamide	Θεραπεία της φυματίωσης
Hungarian	N-{{[(5S)-3-(3-fluoro-4-tiomorfolin-4-ilfenil)-2-oxo-1,3-oxazolidin-5-il]metil}acetamid	Tuberculosis kezelése
Italian	N-{{[(5S)-3-(3-fluoro-4-tiomorfolin-4-ilfenil)-2-osso-1,3-ossazolidin-5-il]metil}acetammide	Trattamento della tubercolosi
Latvian	N-{{[(5S)-3-(3-fluoro-4-tiomorfolīn-4-ilfenil)-2-okso-1,3-oksazolidīn-5-il]metil}acetamīds	Tuberkulozes ārstēšana
Lithuanian	N-{{[(5S)-3-(3-fluoro-4-tiomorfolin-4-ilfenil)-2-okso-1,3-oksazolidin-5-il]metil}acetamidas	Tuberkuliozės gydymas

¹ At the time of transfer of sponsorship

Language	Active ingredient	Indication
Maltese	N-{{(5S)-3-(3-fluoro-4-thiomorpholin-4-ylphenyl)-2-oxo-1,3-oxazolidin-5-yl}methyl}acetamide	Kura tat-tuberkulosi
Polish	N-{{(5S)-3-(3-fluoro-4-tiomorfolin-4-ylofenylo)-2-okso-1,3-oksazolidyn-5-ylo}metylo}acetamid	Leczenie gruźlicy
Portuguese	N-{{(5S)-3-(3-fluoro-4-tiomorfolina-4-ilfenil)-2-oxo-1,3-oxazolidina-5-il}metil}acetamida	Tratamento da tuberculose
Romanian	N-{{(5S)-3-(3-fluoro-4-tiomorfolin-4-ilfenil)-2-oxo-1,3-oxazolidin-5-il}metil}acetamidă	Tratamentul tuberculozei
Slovak	N-{{(5S)-3-(3-fluoro-4-tiomorfolín-4-yľfenyl)-2-oxo-1,3-oxazolidín-5-yl}metyl}acetamid	Liečba tuberkulózy
Slovenian	N-{{(5S)-3-(3-fluoro-4-tiomorfolin-4-ilfenil)-2-okso-1,3-oksazolidin-5-il}metil}acetamid	Zdravljenje tuberkuloze
Spanish	N-{{(5S)-3-(3-fluoro-4-tiomorfolin-4-ilfenil)-2-oxo-1,3-oxazolidin-5-il}metil}acetamida	Tratamiento de la tuberculosis
Swedish	N-{{(5S)-3-(3-fluoro-4-tiomorfolin-4-yľfenyl)-2-oxo-1,3-oxazolidin-5-yl}metyl}acetamid	Behandling av tuberkulos
Norwegian	N-{{(5S)-3-(3-fluor-4-tiomorfolin-4-yľfenyl)-2-okso-1,3-oksazolidin-5-yl}metyl}acetamid	Behandling av tuberculose
Icelandic	N-{{(5S)-3-(3-flúoró-4-tíomorfólín-4-yľfenyl)-2-oxó-1,3-oxazólidín-5-yl}metýl}acetamíð	Meðferð við berklum