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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Rifapentine 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 9 June 2010, orphan designation (EU/3/10/750) was granted by the European Commission to sanofi-aventis, France, for rifapentine for the treatment of tuberculosis.

In October 2012, Sanofi Aventis changed name to Sanofi-Aventis Groupe.

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 (when it is 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 everyone infected will develop the symptoms of the disease.

TB is a long-term debilitating disease. When left untreated, the disease may be life threatening mainly because of the severe damage to the lungs that does not allow the patient to breathe normally.

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

At the time of designation, TB affected not more than 2 in 10,000 people in the European Union (EU)[*]. This was equivalent to a total of not more than 101,000 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 the 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 27), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 506,300,000 (Eurostat 2010).

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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 rifapentine might be of significant benefit for patients with TB because its use with other existing antibiotics might shorten the total treatment duration by up to three months. 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?

Rifapentine is similar to a group of antibiotics known as rifamycin antibiotics, which include rifampicin. These are the antibiotics most used in the treatment of TB. Rifapentine works by blocking enzymes known as RNA polymerases, which the bacteria need to produce proteins. By blocking these enzymes, rifapentine is expected to stop the growth of the bacteria, eventually killing them.

Because of its chemical structure and the way it works in the body, rifapentine used in combination with other medicines is expected to be able to treat TB more quickly than current combinations do.

What is the stage of development of this medicine?

The effects of rifapentine have been evaluated in experimental models.

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

At the time of submission, rifapentine was authorised and designated as an orphan medicinal product in the United States of America for pulmonary TB.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 3 March 2010 recommending the granting of this designation.

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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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www.sanofi-aventis.com/contact/contact.asp

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	Rifapentine	Treatment of tuberculosis
Bulgarian	Рифапентин	Лечение на туберкулоза
Czech	Rifapentine	Léčba tuberkulózy
Danish	Rifapentin	Behandling af tuberkulose
Dutch	Rifapentine	Behandeling van tuberculose
Estonian	Rifapentiin	Tuberkuloosi ravi
Finnish	Rifapentiini	Tuberkuloosin hoito
French	Rifapentine	Traitement de la tuberculose
German	Rifapentine	Behandlung der Tuberkulose
Greek	Ριφαπεντίνη	Θεραπεία της φυματίωσης
Hungarian	Rifapentin	Tuberculosis kezelése
Italian	Rifapentina	Trattamento della tubercolosi
Latvian	Rīfapentīns	Tuberkulozes ārstēšana
Lithuanian	Rifapentinas	Tuberkuliozės gydymas
Maltese	Rifapentine	Kura tat-tuberkulosi
Polish	Ryfapentyna	Leczenie gruźlicy
Portuguese	Rifapentina	Tratamento da tuberculose
Romanian	Rifapentină	Tratamentul tuberculozei
Slovak	Rifapentín	Liečba tuberkulózy
Slovenian	Rifapentin	Zdravljenje tuberkoloze
Spanish	Rifapentina	Tratamiento de la tuberculosis
Swedish	Rifapentin	Behandling av tuberkulos
Norwegian	Rifapentin	Behandling av tuberkulose
Icelandic	Rífapentín	Meðferð við berklum

[1] At the time of designation