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Public summary of opinion on orphan designation

Purified pasteurised and freeze-dried cell-wall fragments from *Mycobacterium tuberculosis* strain RUTI for the treatment of tuberculosis

On 23 August 2017, orphan designation (EU/3/17/1905) was granted by the European Commission to Archivel Farma S.L., Spain, for purified pasteurised and freeze-dried cell-wall fragments from *Mycobacterium tuberculosis* strain RUTI for the treatment of tuberculosis.

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, tuberculosis affected approximately 1.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 83,000 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.

[*]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 28), Norway, Iceland and Liechtenstein. This represents a population of 515,700,000 (Eurostat 2017).

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The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with tuberculosis because preliminary results suggest it can be used with an existing antibiotic to increase its effectiveness. 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 is a vaccine that contains substances from the cell walls of tuberculosis bacteria that have been killed and broken up. When the medicine is injected into the body, the immune system (the body's natural defences) recognises the substances in the cell wall as foreign. This stimulates the immune system to attack the same substances in living bacteria. In patients with TB, this helps to kill the bacteria and prevent symptoms of the disease from developing.

What is the stage of development of this medicine?

The effects of the 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, the medicine was not authorised anywhere in the EU for TB or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 July 2017 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's rare disease designations page.

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	Purified pasteurised and freeze-dried cell-wall fragments from *Mycobacterium tuberculosis* strain RUTI	Treatment of tuberculosis
Bulgarian	Пречистени пастьоризирани и лиофилизирани фрагменти от клетъчна стена на *Mycobacterium tuberculosis,* щам RUTI	Лечение на туберкулоза
Croatian	Pročišćeni pasterizirani i biofilizirani fragmenti stanične stjenke soja RUTI bakterije *Mycobacterium tuberculosis*	Liječenje tuberkuloze
Czech	Purifikované, pasterizované a lyofilizované fragmenty buněčných stěn *Mycobacterium tuberculosis* kmene RUTI	Léčba tuberkulózy
Danish	Rensede, pasteuriserede og frysetørrede cellevægsfragmenter fra *Mycobacterium tuberculosis* RUTI-stamme	Behandling af tuberkulose
Dutch	Gezuiverde, gepasteuriseerde en gevriesdroogde celwandfragmenten van de RUTI-stam van *Mycobacterium tuberculosis*	Behandeling van tuberculose
Estonian	Puhastatud, pastöriseeritud ja külmkuivatatud rakuseina fragmendid *Mycobacterium tuberculosis*'e tüvest RUTI	Tuberkuloosi ravi
Finnish	Puhdistettuja, pastöroituja ja pakastekuivattuja *Mycobacterium tuberculosis*-bakteerin solunseinämän osia RUTI-rokotteessa	Tuberkuloosin hoito
French	Fragments de paroi cellulaire purifiés, pasteurisés et lyophilisés issus de la souche RUTI de *Mycobacterium tuberculosis*	Traitement de la tuberculose
German	Gereinigte, pasteurisierte und gefriergetrocknete Zellwandfragmente des *Mycobacterium tuberculosis*-Stamms RUTI	Behandlung der Tuberkulose
Greek	Κεκαθαρμένα, παστεριωμένα και λυοφυλιωμένα τμήματα κυτταρικού τοιχώματος από το στέλεχος RUTI του *Mycobacterium tuberculosis*	Θεραπεία της φυματίωσης
Hungarian	Tisztított, pasztörizált és fagyasztva-szárított sejtfalfragmentum *Mycobacterium tuberculosis* RUTI törzséből	Tuberculosis kezelése
Italian	Frammenti di parete cellulare purificati, pastorizzati e liofilizzati del ceppo RUTI di *Mycobacterium tuberculosis*	Trattamento della tubercolosi
Latvian	Attīrīti, pasterizēti un liofilizēti šūnu sieniņu fragmenti no *Mycobacterium tuberculosis* celma RUTI	Tuberkulozes ārstēšana
Lithuanian	Išgryninti, pasterizuoti ir šaldant išdžiovinti ląstelių sienelių fragmentai iš *Mycobacterium tuberculosis* atmainos RUTI	Tuberkuliozės gydymas

[1] At the time of designation

Language	Active ingredient	Indication
Maltese	Frammenti tal-ħajt taċ-ċelloli li huma ppurifikati, pastorizzati u mnixxfin bil-friża mir-razza *Mycobacterium tuberculosis* RUTI	Kura tat-tuberkuloži
Polish	Oczyszczone, pasteryzowane i liofilizowane fragmenty ściany komórkowej szczepu RUTI *Mycobacterium tuberculosis*	Leczenie gruźlicy
Portuguese	Fragmentos da parede celular da estirpe RUTI de *Mycobacterium tuberculosis* purificados, pasteurizados e liofilizados	Tratamento da tuberculose
Romanian	Fragmente de perete celular purificate, pasteurizate și liofilizate din tulpina RUTI de *Mycobacterium tuberculosis*	Tratamentul tuberculozei
Slovak	Purifikované, pasterizované fragmenty z bunkových stien vysušené mrazením z kmeňa *Mycobacterium tuberculosis* RUTI	Liečba tuberkulózy
Slovenian	Prečiščeni, pasterizirani in liofilizirani fragmenti celične stene bakterije *Mycobacterium tuberculosis*, seva RUTI	Zdravljenje tuberkuloze
Spanish	Fragmentos de pared celular purificados, pasteurizados y liofilizados de la cepa RUTI de *Mycobacterium tuberculosis*	Tratamiento de la tuberculosis
Swedish	Renade, pastöriserade och frystorkade cellväggsfragment från *Mycobacterium tuberculosis*-stammen RUTI	Behandling av tuberkulos
Norwegian	Rensede, pasteuriserte og frysetørkede fragmenter av celleveggen fra *Mycobacterium tuberculosis,* RUTI stamme	Behandling av tuberkulose
Icelandic	Hreinsað, gerilsneytt og frostþurrkuð frumuveggjarbrot úr RUTI-stofni *Mycobacterium tuberculosis*	Meðferð við berklum