



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Single-chain urokinase plasminogen activator for the treatment of pleural empyema

On 16 December 2014, orphan designation (EU/3/14/1383) was granted by the European Commission to Coté Orphan Consulting UK Limited, United Kingdom, for single-chain urokinase plasminogen activator for the treatment of pleural empyema.

What is pleural empyema?

Pleural empyema is a condition in which a bacterial lung infection spreads to the space between the pleura, the two layers of membrane that surround the lung inside the chest. This space becomes filled with fluid and dead cells as well as deposits of a protein called fibrin, producing pockets of thick pus. The pus and fibrin build-up make it harder to breathe properly and eventually lead to the formation of scar tissue that permanently reduces normal lung function.

Pleural empyema is a long-term debilitating and life-threatening condition due to restriction of the normal expansion of the lung.

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

At the time of designation, pleural empyema affected approximately 1.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 66,000 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, various antibiotics were authorised in the EU for the treatment of lung infections, which would include infections that spread to the pleura. In addition, patients were offered chest drainage, to remove the fluid and pus, or other surgery as appropriate.

*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 511,100,000 (Eurostat 2014).



The sponsor has provided sufficient information to show that single-chain urokinase plasminogen activator might be of significant benefit for patients with pleural empyema because experimental data show that it works in a different and complementary way to existing treatments by reducing the formation of fibrin that makes the pleura stick together. 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?

In patients with pleural empyema, inflammation due to the infection triggers deposits of fibrin in the fluid between the two pleura, sticking them together (adhesion) so they cannot move so easily. This creates pockets where pus can collect that are difficult to drain and into which antibiotics cannot penetrate easily. The medicine converts another protein in the body, plasminogen, into its active form plasmin which is able to break down and dissolve fibrin. Giving the medicine directly into the space between the pleura is therefore expected to result in breakdown of the fibrin there, enabling the pus to be drained and antibiotics to penetrate the space, so improving the symptoms of the condition.

What is the stage of development of this medicine?

The effects of single-chain urokinase plasminogen activator have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with the medicine in patients with pleural empyema had been started.

At the time of submission, single-chain urokinase plasminogen activator was not authorised anywhere in the EU for pleural empyema 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 November 2014 recommending the granting of this designation.

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Single-chain urokinase plasminogen activator	Treatment of pleural empyema
Bulgarian	Едноверижен урокиназен плазминогенен активатор	Лечение на плеврална емпием
Croatian	Jednolančani urokinazni aktivator plazminogena	Liječenje empijema pleure
Czech	Jednořetězcový urokinázový aktivátor plazminogenu	Léčba pleurální empyému
Danish	Enkeltkædet urokinase plasminogenaktivator	Behandling af pleuralt empyem
Dutch	Enkel-keten urokinase-plasminogeenactivator	Behandeling van pleuraal empyeem
Estonian	Üheaahelaline urokinaasi-tüüpi plasminogeeni aktivaator	Pleura empüeemi ravi
Finnish	Yksiketjuinen urokinaasi-tyyppin plasminogeeniaktivaattori	Keuhkopussin märkäkertymän hoito
French	Activateur du plasminogène de type urokinase à chaîne unique	Traitement de l'empyème pleurale
German	Einzelketten-Urokinase-Plasminogen-Aktivator	Behandlung eines pleuralen Empyems
Greek	Ενεργοποιητής πλασμινογόνου ουροκινάσης απλής αλύσου	Θεραπεία του πλευριτικού εμπύηματος
Hungarian	Egyláncú, urokináz-plazminogén aktivátor	Pleurális empyema kezelése
Italian	Attivatore del plasminogeno di tipo urochinasi a catena singola	Trattamento dell'empiema pleurico
Latvian	Vienkāršas ķēdes urokināzes plazminogēna aktivators	Pleiras empiēmas ārstēšana
Lithuanian	Viengrandis urokinazės plazminogeno aktyvatorius	Pleuros empiemos gydymas
Maltese	Attivatur tal-urokinase plasminogen b'katina waħda	Kura ta' ġabra ta' materja fil-plewra
Polish	Jednołańcuchowy urokinazowy aktywator plazminogenu	Leczenie ropniaka opłucnej
Portuguese	Activador do plasminogénio do tipo uroquinase de cadeia única	Tratamento do empiema pleural
Romanian	Activator al plasminogenului de tip urokinază cu lanț unic	Tratamentul empiemului pleural
Slovak	Jednoreťazcový urokinázový aktivátor plazminogénu	Liečba pleurálnej empyému
Slovenian	Enoverižni urokinazni aktivator plazminogena	Zdravljenje plevralne empiema
Spanish	Activador del plasminógeno de tipo uroquinasa de cadena simple	Tratamiento de l'empiema pleural
Swedish	Enkelkedjig urokinasplasminogenaktivator	Behandling av lungempyem
Norwegian	Enkeltkjedet urokinaseplasminogenaktivator	Behandling av pleuraempyem
Icelandic	Einkeðju úrókínsa plasmínógen hvati	Meðferð við brjóstholsgígerð

¹ At the time of designation

