

30 March 2015
EMA/COMP/55779/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Fibrinogen-coated albumin spheres for the treatment of Ebola virus disease

On 12 February 2015, orphan designation (EU/3/15/1442) was granted by the European Commission to Fibreu Limited, United Kingdom, for fibrinogen-coated albumin spheres for the treatment of Ebola virus disease.

What is Ebola virus disease?

Ebola virus disease is a severe disease caused by infection with viruses known as ebolaviruses. There are 5 known species of ebolavirus, 4 of which are known to cause the disease in humans. Zaire ebolavirus, sometimes referred to simply as 'ebola virus' or EBOV, is the cause of the largest outbreaks of the disease to date and has led to the most deaths.

Infection is caused by contact with the body fluids of an infected person. After infection there is an incubation period of between 2 to 21 days, following which the newly infected person starts to experience symptoms. The first symptoms typically are fever, headache, fatigue, muscle pain and sore throat. These are followed by other symptoms such as diarrhoea, vomiting, rash, kidney and liver problems and, in some cases, internal bleeding and bleeding from the gums, eyes, nose and ears. Patients are infectious once they start experiencing symptoms.

Ebola virus disease is a life-threatening condition that is frequently fatal due to fluid loss and severe bleeding.

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

At the time of designation, Ebola virus disease affected less than 0.01 in 10,000 people in the European Union (EU). This 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 application for orphan designation, there were no satisfactory treatments authorised in the EU for treating Ebola virus disease.

How is this medicine expected to work?

Patients with Ebola virus disease can suffer from severe life-threatening bleeding, especially when the disease leads to low levels of blood platelets, cells that play a role in blood clotting. This medicine consists of spherical particles coated with fibrinogen, a protein naturally found in the blood that is also involved in helping the blood to clot. When the particles reach a bleeding site, the extra fibrinogen in the medicine is converted into a fibrous protein known as fibrin, which helps plug the bleeding site, in the same way as a natural blood clot. This additional clotting effect is expected to help counteract any clotting problems caused by lack of blood platelets and so to reduce the bleeding associated with the disease.

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, no clinical trials with the medicine in patients with Ebola virus disease had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for Ebola virus disease 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 9 January 2015 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 | Fibrinogen-coated albumin spheres | Treatment of Ebola virus disease |
| Bulgarian | Албуминови сфери покрити с фибриноген | Лечение на Ебола вирусно заболяване |
| Croatian | Fibrinogenom obložene sfere albumina | Liječenje Ebola virusne bolesti |
| Czech | Fibrinogenem obalené albuminové koule | Léčba Ebola virové choroby |
| Danish | Fibrinogenovertrukket albumin spheres | Behandling af Ebola virus-sygdom |
| Dutch | Fibrinogeen-omhulde albumine sferen | Behandeling van Ebola virusziekte |
| Estonian | Fibrinogeeniga kaetud albumiini kerakesed | Ebola viiruse haiguse ravi |
| Finnish | Fibrinogeenin päällystetty albumin kuulat | Ebola-viruksen aiheuttaman taudin hoito |
| French | Sphères d'albumine recouvertes de fibrinogène | Traitement de la maladie à virus Ebola |
| German | Fibrinogen beschichtete Albumin Spheres | Behandlung der Ebola-Viruskrankheit |
| Greek | Σφαίρες αλβουμίνης καλυμμένες με ινωδογόνο | Θεραπεία της νόσου από τον ιό Έμπολα |
| Hungarian | Fibrinogén bevonatú albumin gömbök | Ebola vírus fertőzés kezelése |
| Italian | Sfere di albumina rivestite di fibrinogeno | Trattamento della malattia da virus Ebola |
| Latvian | Ar fibrinogēnu pārklātas albumīna sfēras | Ebolas vīrusa slimības ārstēšana |
| Lithuanian | Fibrinogenu dengtos albumino sferos | Ebola viruso sukeltos ligos gydymas |
| Maltese | Sferi tal-albumina miksija b'fibrinogen | Kura tal-marda mill-virus Ebola |
| Polish | Sfery albuminowe powlekane fibrynogenem | Leczenie choroby wywołanej przez wirus Ebola |
| Portuguese | Esferas de albumina revestidas de fibrinogénio | Tratamento da doença por vírus Ébola |
| Romanian | Sfere de albumină acoperite cu fibrinogen | Tratamentul bolii virale Ebola |
| Slovak | Fibrinogén-coated albumin gule | Liečba Ebola vírusového ochorenia |
| Slovenian | Albuminske sfere, prekrite s fibrinogenom | Zdravljenje Ebola virusne bolezni |
| Spanish | Esferas de albumina revestidas de fibrinogeno | El tratamiento de la enfermedad viral de Ébola |
| Swedish | Fibrinogentäckta albuminsfärer | Behandling av Ebolavirussjukdom |
| Norwegian | Fibrinogenovertruktede albuminkuler | Behandling av ebolavirussykdom |
| Icelandic | Fibrínógen húðaðar albúmin kúlur | Meðferð Ebóluveiru sjúkdóms |

¹ At the time of designation