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EMA/COMP/508238/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Fibrinogen-coated albumin spheres for the treatment of acute radiation syndrome

On 10 August 2015, orphan designation (EU/3/15/1535) was granted by the European Commission to Fibreu Limited, United kingdom, for fibrinogen-coated albumin spheres for the treatment of acute radiation syndrome.

What is acute radiation syndrome?

Acute radiation syndrome (also known as radiation sickness) is a severe illness caused by exposure of the body to a high dose of penetrating radiation in a very short period of time. This can occur, for example, following an accident at a nuclear plant or the use of radioactive material for medical purposes.

Symptoms can begin within a few hours after exposure and their severity depends on the amount of radiation absorbed by the body. Relatively small amounts result in gastrointestinal effects such as nausea (feeling sick), vomiting and diarrhoea, reduction in blood cell count, and tendency to infection and bleeding. Relatively large amounts can result in effects on the central nervous system (brain and spinal cord) and rapid death.

Acute radiation syndrome is a life-threatening disease because it can lead to failure of multiple organs and death.

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

At the time of designation, acute radiation syndrome affected not more than 0.01 in 10,000 people in the European Union (EU). This was equivalent to a total of not more than 500 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).

*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 512,900,000 (Eurostat 2015).

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What treatments are available?

At the time of designation, no satisfactory methods were authorised in the EU for the treatment of acute radiation syndrome.

How is this medicine expected to work?

Acute radiation syndrome can cause the destruction of the bone marrow, the spongy tissue inside the large bones where blood cells are produced. This causes a reduction in the number of red and white blood cells, as well in the number of platelets, the blood components that help the blood to clot.

This medicine consists of spherical particles coated with fibrinogen, a protein naturally found in the blood that is involved in helping the blood to clot. When the particles reach a bleeding site, the extra fibrinogen provided by 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 and bleeding caused by lack of blood platelets and so reduce the bleeding associated with radiation exposure.

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 acute radiation syndrome had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for acute radiation syndrome 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 16 July 2014 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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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	Fibrinogen-coated albumin spheres	Treatment of acute radiation syndrome
Bulgarian	Албуминови сфери покрити с фибриноген	Лечение на остра лъчева болест
Croatian	Albuminske sfere obložene fibrinogenom	Liječenje akutnog radijacijskog sindroma
Czech	Fibrinogenem obalené albuminové koule	Léčba akutní radiační syndromu
Danish	Fibrinogenovertrukket albumin spheres	Behandling af akut bestråling syndrom
Dutch	Fibrinogeen-omhulde albumine spheren	Behandeling van stralingsziekte
Estonian	Fibrinogeeniga kaetud albumiini kerakesed	Ägeda kiirgus sündroomi ravi
Finnish	Fibrinogeenillä päällystetyt albumiinikuulat	Säteilysairauden hoito
French	Sphères d'albumine recouvertes de fibrinogène	Traitement du syndrome d'irradiation aiguë
German	Fibrinogen beschichtete Albumin Spheres	Behandlung der Strahlenkrankheit
Greek	Σφαίρες αλβουμίνης καλυμμένες με ινωδογόνο	Θεραπεία του συνδρόμου οξείας ακτινοβόλησης
Hungarian	Fibrinogén bevonatú albumin gömbök	Sugárbetegség kezelése
Italian	Sfere di albumina rivestite di fibrinogeno	Trattamento della sindrome acuta da radiazioni
Latvian	Ar fibrinogēnu pārklātas albumīna sfēras	Akūta radiācijas sindroma ārstēšana
Lithuanian	Fibrinogenu-dengtos albumino sferos	Ūminio radiacinio sindromo gydymas
Maltese	Sferi tal-albumina miksija b'fibrinoġen	Kura tas-sindrome ta' radjazzjoni akuta
Polish	Sfery albuminowe powlekane fibrynogenem	Leczenie ostrej choroby popromiennej
Portuguese	Esferas de albumina revestidas de fibrinogénio	Tratamento do sindrome agudo das radiações
Romanian	Sfere de albumină acoperite cu fibrinogen	Tratamentul sindromului acut de iradiere
Slovak	Fibrinogén-coated albumin gule	Liečba akútnehoradiačného syndrómu
Slovenian	Albuminske sfere, prekrite s fibrinogenom	Zdravljenje akutnega radiacijskega sindroma
Spanish	Esferas de albumina revestidas de fibrinogeno	Tratamiento del síndrome de radiación aguda
Swedish	Fibrinogentäckta albuminsfärer	Behandling av akut strålningssyndrom
Norwegian	Fibrinogenovertrukkede albuminkuler	Behandling av akutt strålesyndrom
Icelandic	Fíbrínógen húðaðar albúmín kúlur	Meðferð bráðrar geislunar heilkenni

[1] At the time of designation