



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Rintatolimod for the treatment of Ebola virus disease

On 24 April 2015, orphan designation (EU/3/15/1480) was granted by the European Commission to NV Hemispherx BioPharma Europe, Belgium, for rintatolimod 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. Different treatments were used to relieve the symptoms of the disease, such as providing fluids by a drip into a vein and balancing electrolytes (body salts), maintaining oxygen status and blood pressure and treating other infections.



How is this medicine expected to work?

Rintatolimod is made up of two synthetic molecules of RNA (a type of genetic material), which are attached together. It is thought that the medicine works in two ways; first, by stimulating cells involved in the body's innate immune system (via receptors known as TLR 3), and secondly, by stimulating the production of interferons, a group of signalling proteins involved in protecting the body against viruses.

What is the stage of development of this medicine?

The effects of rintatolimod 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 19 March 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	Rintatolimod	Treatment of Ebola virus disease
Bulgarian	Ринталимод	Лечение на Ебола вирусно заболяване
Croatian	Rintatolimod	Liječenje Ebola virusne bolesti
Czech	Rintatolimod	Léčba Ebola virové choroby
Danish	Rintatolimod	Behandling af Ebola virus-sygdom
Dutch	Rintatolimod	Behandeling van Ebola virusziekte
Estonian	Rintatolimod	Ebola viiruse haiguse ravi
Finnish	Rintatolimodi	Ebola-viruksen aiheuttaman taudin hoito
French	Rintatolimod	Traitement de la maladie à virus Ebola
German	Rintatolimod	Behandlung der Ebola-Viruskrankheit
Greek	Ριντατολιμόδη	Θεραπεία της νόσου από τον ιό Έμπολα
Hungarian	Rintatolimod	Ebola vírus fertőzés kezelése
Italian	Rintatolimod	Trattamento della malattia da virus Ebola
Latvian	Rintatolimods	Ebolas vīrusa slimības ārstēšana
Lithuanian	Rintatolimodas	<i>Ebola</i> viruso sukeltos ligos gydymas
Maltese	Rintatolimod	Kura tal-marda mill-virus Ebola
Polish	Rintatolimod	Leczenie choroby wywołanej przez wirus Ebola
Portuguese	Rintatolimod	Tratamento da doença por vírus Ébola
Romanian	Rintatolimod	Tratamentul bolii virale Ebola
Slovak	Rintatolimod	Liečba Ebola vírusového ochorenia
Slovenian	Rintatolimod	Zdravljenje Ebola virusne bolezni
Spanish	Rintatolimod	El tratamiento de la enfermedad viral de Ébola
Swedish	Rintatolimod	Behandling av Ebolavirusjukdom
Norwegian	Rintatolimod	Behandling av ebolavirusjukdom
Icelandic	Rintatólímód	Meðferð Ebóluveiru sjúkdóms

¹ At the time of designation