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## Public summary of opinion on orphan designation

Three human monoclonal antibodies against the EBOV glycoprotein for the treatment of Ebola virus disease

On 25 May 2018, orphan designation (EU/3/18/2027) was granted by the European Commission to Regeneron Ireland U.C., Ireland, for three human monoclonal antibodies against the EBOV glycoprotein (also known as REGN3470-3471-3479) for the treatment of Ebola virus disease.

### What is Ebola virus disease?

Ebola virus disease is a severe disease caused by infection with 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 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 to develop symptoms.

Ebola virus disease is a life-threatening condition that is frequently fatal due to fluid loss through severe diarrhoea 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 0.001 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 50 people<sup>\*</sup>, 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).

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<sup>\*</sup>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 517,400,000 (Eurostat 2018).

## **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 supply to the body and blood pressure, and treating other infections if they develop.

## **How is this medicine expected to work?**

This medicine is made of 3 monoclonal antibodies, proteins that have been designed to recognise and attach to different parts of a target substance on the surface of the virus. By attaching to the virus, the antibodies are expected to prevent it from infecting cells and reproducing itself, and to stimulate the immune system (the body's natural defences) to kill the virus and infected cells.

## **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, a clinical trial with the medicine in healthy volunteers had been completed.

At the time of submission, the medicine was not authorised anywhere in the EU for Ebola virus disease. Orphan designation of the medicine had been granted in the United States for the treatment of Ebola virus infection.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 19 April 2018 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<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Three human monoclonal antibodies against the Ebola virus glycoprotein	Treatment of Ebola virus disease
Bulgarian	Три човешки моноклонални антитела срещу гликопротеин на вируса Ебола	Лечение на Ебола вирусно заболяване
Croatian	Tri ljudska monoklonska protutijela na glikoprotein Ebola virusa	Liječenje Ebola virusne bolesti
Czech	Tři lidské monoklonální protilátky proti glykoproteinu Ebola viru	Léčba Ebola virové choroby
Danish	Tre humane monoklonale antistoffer mod Ebola virus-glykoproteinet	Behandling af Ebola virus-sygdom
Dutch	Drie humane monoklonale antilichamen tegen de glycoproteïne van het ebolavirus	Behandeling van Ebola virusziekte
Estonian	Kolm inimese monoklonaalset Ebola viiruse glükoproteiini vastast antikeha	Ebola viiruse haiguse ravi
Finnish	Kolme humaania monoklonaalista Ebola-viruksen-glykoproteiinivasta-ainetta	Ebola-viruksen aiheuttaman taudin hoito
French	Trois anticorps monoclonaux humains dirigés contre la glycoprotéine du virus Ebola	Traitement de la maladie à virus Ebola
German	Drei humane monoklonale Antikörper gegen das Ebola-Virus-Glykoprotein	Behandlung der Ebola-Viruskrankheit
Greek	Τρία ανθρώπινα μονοκλωνικά αντισώματα κατά της γλυκοπρωτεΐνης του ιού Έμπολα	Θεραπεία της νόσου από τον ιό Έμπολα
Hungarian	Az Ebolavírus-glikoprotein elleni három humán monoklonális antitest	Ebola vírus fertőzés kezelése
Italian	Tre anticorpi monoclonali umani contro la glicoproteina del virus Ebola	Trattamento della malattia da virus Ebola
Latvian	Trīs cilvēka monoklonālās antivielas pret Ebolas vīrusa glikoproteīnu	Ebolas vīrusa slimības ārstēšana
Lithuanian	Trys žmogaus monokloniniai antikūnai prieš Ebola viruso glikoproteiną	Ebola viruso sukeltos ligos gydymas
Maltese	Tliet antikorpi monoklonali umani kontra l-glikoproteina ta' virus Ebola	Kura tal-marda mill-virus Ebola
Polish	Trzy ludzkie przeciwciałamonoklonalne przeciwko glikoproteinie wirusa Ebola	Leczenie choroby wywołanej przez wirus Ebola
Portuguese	Três anticorpos monoclonais humanos contra a glicoproteína do vírus Ébola	Tratamento da doença por vírus Ébola
Romanian	Trei anticorpi monoclonali umani împotriva glicoproteinei virale Ebola	Tratamentul bolii virale Ebola
Slovak	Tri ľudské monoklonálne protilátky proti glykoproteínu Ebola vírusu	Liečba Ebola vírusového ochorenia

<sup>1</sup> At the time of designation

Language	Active ingredient	Indication
Slovenian	Tri humana monoklonska protitelesa proti glikoproteinu virusa Ebola	Zdravljenje Ebola virusne bolezni
Spanish	Tres anticuerpos monoclonales humanos contra la glicoproteína del virus del Ébola	Tratamiento de la enfermedad viral de Ébola
Swedish	Tre humana monoklonala antikroppar mot Ebola virus-glycoproteinet	Behandling av Ebolavirus sjukdom
Norwegian	Tre humane monoklonale antistoffer mot ebolavirus-glykoproteinet	Behandling av ebolavirus sykdom
Icelandic	Premur manna einstofna mótefnum gegn Ebóluveiru-glykópróteininu	Meðferð Ebóluveiru sjúkdóms