

12 August 2015
EMA/COMP/433479/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Anti-H5N1 equine immunoglobulin F(ab')₂ fragments for the treatment of avian influenza

On 28 July 2015, orphan designation (EU/3/15/1512) was granted by the European Commission to Fab'entech, France, for anti-H5N1 equine immunoglobulin F(ab')₂ fragments for the treatment of avian influenza.

What is avian influenza?

Avian influenza (or bird flu) is an infectious disease of birds caused by certain subtypes of the influenza A virus. Avian influenza has rarely spread to humans through contact with infected poultry. To date, the subtype known as H5N1 has caused serious human infections; a very few cases in people have also been reported with another subtype, H7N9.

Avian influenza can be life threatening as it can lead to severe pneumonia (infection of the lungs), problems with breathing and multi-organ failure.

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

At the time of designation, avian influenza 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 designation, antiviral medicines such as oseltamivir were authorised in the EU to treat influenza infections including avian influenza.

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with avian influenza because early studies in experimental models indicate that it

*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).

might improve survival. 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?

This medicine is made of antibody fragments. Antibodies are proteins that attach to a specific target in the body and can help to fight infections and other diseases. The antibody fragments in the medicine are expected to attach to the H5N1 virus and neutralise its ability to infect body 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, no clinical trials with the medicine in patients with avian influenza were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for avian influenza. Orphan designation of the medicine had been granted in Australia for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 18 June 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	Anti-H5N1 equine immunoglobulin F(ab') ₂ fragments	Treatment of avian influenza
Bulgarian	F(ab') ₂ фрагменти от анти-H5N1 конски имуноглобулин	Лечение на птичи грип
Croatian	Anti-H5N1 F(ab') ₂ fragmenti konjskih imunoglobulina	Liječenje „ptičje gripe”
Czech	Fragment anti-H5N1 koňského imunoglobulinu F(ab') ₂	Léčba ptačí chřipky
Danish	Anti-H5N1 heste immunoglobulin F(ab') ₂ fragmenter	Behandling af fugle influenza
Dutch	Anti-H5N1 equine immunoglobulin F(ab') ₂ fragmenten	Behandeling van vogelgriep
Estonian	Anti-H5N1 hobuste immunoglobuliin F(ab') ₂ fragmendid	Linnugripi ravi
Finnish	Hevosen immuglobuliinin F(ab') ₂ fragmentit H5N1:tä vastaan	Lintuinfluenssan hoito
French	Fragments F(ab') ₂ d'immunoglobuline équine anti-H5N1	Traitement de la grippe aviaire
German	Gegen H5N1 gerichtetes equines Immunglobulin mit F(ab') ₂ Fragment	Behandlung der aviären Influenza
Greek	Τμήματα F(ab') ₂ ιππείας αντι-H5N1 ανοσοσφαιρίνης	Θεραπεία της γρίπης των πτηνών
Hungarian	H5N1-ellenes, lóból származó F(ab') ₂ immunoglobulin fragmentumok	Madárinfluenza kezelése
Italian	Frammenti F(ab') ₂ d'immunoglobuline equine anti-H5N1	Trattamento dell'influenza aviaria
Latvian	Anti-H5N1 zirga imūnglobulīnu F(ab') ₂ fragmenti	Putnu gripas ārstēšana
Lithuanian	Anti-H5N1 arkliai imunoglobulino F(ab') ₂ fragmentai	Paukščių gripo gydymas
Maltese	Frammenti F(ab') ₂ tal-immunoglobulina ekwina kontra H5N1	Kura tal-influenza relatata mal-ghasafar
Polish	Region F(ab') ₂ końskiej immunoglobuliny skierowanej przeciw H5N1	Leczenie ptasiej grypy
Portuguese	Fragmentos F(ab') ₂ de imunoglobulina equina anti-H5N1	Tratamento da gripe das aves
Romanian	Fragmente F(ab') ₂ de imunoglobuline ecvine anti-H5N1	Tratamentul gripei aviare
Slovak	Anti-H5N1 konské imunoglobulínové F(ab') ₂ fragmenty	Liečba vtácej chrípky
Slovenian	Anti-H5N1 konjski imunoglobulin, F(ab') ₂ frakcija	Zdravljenje aviarne influence
Spanish	Fragmentos F(ab') ₂ de inmunoglobulina equina anti-H5N1	Tratamiento de la gripe aviar
Swedish	Anti-H5N1 equine immunoglobulin F(ab') ₂ fragment	Behandling a aviär influensa
Norwegian	Anti-H5N1 immunoglobulin F(ab') ₂ fragmenter fra hest	Behandling av fugleinfluensa
Icelandic	Anti-H5N1 hrossa ónæmisglóbúlín F(ab') ₂	Meðferð á fugla influensu

¹ At the time of designation