European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

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Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of human anthrax immunoglobulin for the post-exposure prophylaxis of inhalation anthrax disease

On 9 November 2009, orphan designation (EU/3/09/690) was granted by the European Commission to Emergent Sales and Marketing Germany GmbH, Germany, for human anthrax immunoglobulin for the post-exposure prophylaxis (prevention) of inhalation anthrax disease.

What is inhalation anthrax disease?
Anthrax is a severe disease caused by infection with bacteria called *Bacillus anthracis* (*B. anthracis*). The bacteria produce spores that are very resistant and can lay 'dormant' (inactive) until they find an organism where they can develop and multiply. Anthrax commonly affects animals such as sheep and cows, but can spread to humans when they are exposed to spores from infected animals or contaminated animal products.
The most severe type of anthrax is inhalation anthrax, which occurs when a person has breathed in the bacteria's spores. The first symptoms of inhalation anthrax are similar to a cold. Several days after the spores have been inhaled, they grow into new bacteria and start to release toxins, which cause internal bleeding, swelling and the death of tissue.
Inhalation anthrax disease is a life threatening disease because, if not treated early, it leads to the accumulation of fluid in the lungs, severe inflammation of the lungs and meningitis (inflammation of the membranes that surround the brain and spine).

What is the estimated number of patients at risk of developing the condition?
At the time of designation, the number of patients at risk of inhalation anthrax disease was estimated to be approximately 0.02 people in 10,000 in the European Union (EU)[*]. This is equivalent to a total of around 1,000 people, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

What methods of prevention are available?
At the time of designation, several antibiotics were authorised in the EU for the prevention of inhalation anthrax disease following exposure to *B. anthracis.*
The sponsor has provided sufficient information to show that human anthrax immunoglobulin might be of significant benefit for patients at risk of inhalation anthrax disease, because early studies in experimental models indicate that it might improve the prevention of inhalation anthrax disease in people who have been exposed to *B. anthracis*. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

[*]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 27), Norway, Iceland and Liechtenstein. This represents a population of 504,800,000 (Eurostat 2009).

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How is this medicine expected to work?
Human anthrax immunoglobulin is a solution of polyclonal antibodies, proteins naturally found in the blood that help the body to fight infections and other diseases. These antibodies have been extracted from the blood of people who have been vaccinated against *B. anthracis* bacteria. When injected into a patient who is at risk of developing anthrax after inhaling *B. anthracis* bacteria spores, the antibodies in the medicine are expected to attach to and help to destroy the bacteria, preventing the symptoms of the disease.

What is the stage of development of this medicine?
The effects of human anthrax immunoglobulin have been evaluated in experimental models.
At the time of submission of the application for orphan designation, a clinical trial in healthy volunteers was ongoing.
At the time of submission, human anthrax immunoglobulin was not authorised anywhere in the EU for post-exposure prophylaxis of inhalation anthrax disease or designated as 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 2 September 2009 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 Community) 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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Patient associations' contact points:
None available

Translations of the active ingredient and indication in all official EU languages, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Human anthrax immunoglobulin	Post-exposure prophylaxis of inhalation anthrax disease
Bulgarian	Човешки антраксен имуноглобулин	Пост-експозиционна профилактика на инхалационна антраксна болест.
Czech	Lidský imunoglobulin proti antraxu	Profylaxe po vystavení infekci plicního (inhalačního) antraxu
Danish	Human anthrax immunglobulin	Posteksponeringsprofylakse af sygdommen inhalationsanthrax
Dutch	Humaan anthrax immunoglobuline	Profylaxe (na blootstelling) van inhalatie miltvuurziekte
Estonian	Inimese siberi katku vastane immunoglobuliin	Siberi katku kopsuvormi ekspositsioonijärgne profülaktika
Finnish	Ihmisen immunoglobuliini pernaruttoa vastaan	Hengitysteiden kautta leviävän pernaruton altistumisen jälkeinen profylaksia
French	Immunoglobuline humaine du charbon	Prophylaxie postexposition de la maladie du charbon par inhalation
German	Menschliches Anthrax-Immunglobulin	Postexpositionsprophylaxe gegen Lungenmilzbrand
Greek	Ανθρώπινη ανοσοσφαιρίνη κατά του άνθρακα	Προφύλαξη μετά την έκθεση, έναντι της νόσου του αναπνευστικού άνθρακα
Hungarian	Humán anthrax immunglobulin	Inhalációs anthrax betegség posztexpozíciós profilaxisa
Italian	Immunoglobulina umana contro l'antrace	Profilassi post-esposizione dell'antrace da inalazione
Latvian	Cilvēka imūnglobulīns pret Sibīrijas mēri	Sibīrijas mēra plaušu formas profilaksei pēc kontakta
Lithuanian	Juodligės žmogaus imunoglobulinas	Inhaliacinės juodligės profilaktika po kontakto
Maltese	Immunoglobulina umana għall-anthrax	Profilassi ta' wara l-espożizzjoni għall-marda tal-ġbid man-nifs tal-anthrax
Polish	Ludzka immunoglobulina przeciwwąglikowa	Profilaktyka po wziewnej ekspozycji na wąglika
Portuguese	Imunoglobulina humana de antraz	Profilaxia pós-exposição da doença do antraz por inalação
Romanian	Imunoglobulină umană contra antraxului	Prevenţia postexpunere a bolii antrax prin inhalare
Slovak	Ľudský imunoglobín proti antraxu	Prevencia po expozícii pľúcnej formy ochorenia antrax
Slovenian	Humani imunoglobulin proti antraksu	Poizpostavitvena zaščita pljučnega antraksa
Spanish	Inmunoglobulina humana contra el carbunco (ántrax)	Profilaxis post-exposición del carbunco (ántrax) por inhalación
Swedish	Mänskligt immunglobulin mot mjältbrand	Profylax efter exponering för mjältbrand beroende av inandning
Norwegian	Immunglobulin mot miltbrann, humant	Posteksponeringsprofylakse mot lungemiltbrann
Icelandic	Mannamiltisbrandsónæmisglóbúlín	Til varnar innöndunarmiltisbrandi eftir útsetningu