



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Human anthrax monoclonal antibody for post-exposure prophylaxis of inhalation anthrax disease

On 5 August 2011, orphan designation (EU/3/11/891) was granted by the European Commission to Emergent Sales and Marketing Germany GmbH, Germany, for human anthrax monoclonal antibody for post-exposure prophylaxis of inhalation anthrax disease.

What is inhalation anthrax disease?

Anthrax is a severe disease caused by infection with bacteria called *Bacillus 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 and bleeding of the tissues in the chest 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 0.01 people in 10,000 in the European Union (EU)*. This is equivalent to a total of around 500 people, which is below the ceiling for orphan designation, which is 5 people in 10,000. This

*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 506,300,000 (Eurostat 2011).



is based on the information provided by the sponsor and the 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 monoclonal antibody might be of significant benefit for patients at risk of inhalation anthrax disease, because it works in a different and more targeted way to existing treatments and 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.

How is this medicine expected to work?

This medicine is a monoclonal antibody, a type of protein that has been designed to recognise and attach to the anthrax toxins. The medicine is expected to be injected into patients who are at risk of developing anthrax after having been exposed to *B. anthracis*. By attaching to the anthrax toxins, the medicine is expected to stop them from entering into the body's cells, thereby preventing the disease from developing.

What is the stage of development of this medicine?

The effects of human anthrax monoclonal antibody 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 was ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for post-exposure prophylaxis for inhalation anthrax disease. Orphan designation of human anthrax monoclonal antibody had been granted in the United States of America for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 February 2011 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

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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	Human anthrax monoclonal antibody	Post-exposure prophylaxis of inhalation anthrax disease
Bulgarian	Човешко антиантраксно моноклонално антитяло	Пост-експозиционна профилактика на инхалационна антраксна болест.
Czech	Lidská monoklonální protilátka proti antraxu	Profylaxe po vystavení infekci plicního (inhalačního) antraxu
Danish	Humant anthrax monoklonalt antistof	Posteksponeringsprofylakse af sygdommen inhalationsanthrax
Dutch	Humaan monoklonaal anthrax-antilichaam	Profylaxe (na blootstelling) van inhalatie miltvuurziekte.
Estonian	Inimese siberi katku vastane monoklonaalne antikeha	Siberi katku kopsuvormi ekspositsioonijärgne profülaktika
Finnish	Ihmisen pernaruton monoklonaalinen vasta-aine	Hengitysteiden kautta leviävän pernaruton altistumisen jälkeinen profylaksia
French	Anticorps monoclonal humain dirigé contre la toxine du bacille de la maladie du charbon	Prophylaxie postexposition de la maladie du charbon par inhalation
German	Gegen Anthrax gerichteter humaner monoklonaler Antikörper	Postexpositionsprophylaxe gegen Lungenmilzbrand
Greek	Ανθρώπινο Μονοκλωνικό Αντίσωμα Άνθρακα	Προφύλαξη μετά την έκθεση, έναντι της νόσου του αναπνευστικού άνθρακα
Hungarian	Anthrax elleni humán monoklonális antitest	Inhalációs anthrax betegség posztexpozíciós profilaxisa
Italian	Anticorpo monoclonale umano anti-antrace	Profilassi post-esposizione dell'antrace da inalazione
Latvian	Cilvēka monoklonālās antivielas pret sibīrijas mēri	Sibīrijas mēra plaušu formas profilaksei pēc kontakta
Lithuanian	Žmogaus juodligės monokloninis antikūnas	Inhaliacinės juodligės profilaktika po kontakto
Maltese	Antikorp monoklonali uman kontra l-anthrax	Profilassi ta' wara l-espożizzjoni għall-marda tal-ġbid man-nifs tal-anthrax
Polish	Ludzkie przeciwciało monoklonalne przeciw wąglikowi	Profilaktyka po wziewnej ekspozycji na węglika
Portuguese	Anticorpo monoclonal contra o antraz humano	Profilaxia pós-exposição da doença do antraz por inalação
Romanian	Anticorp monoclonal uman anti-antrax	Prevenția postexpunere a bolii antrax prin inhalare
Slovak	Ľudská monoklonálna protilátka proti antraxu	Prevencia po expozícii pľúcnej formy ochorenia antrax
Slovenian	humano monoklonsko protitelo proti antraksu	Poizpostavitvena zaščita pljučnega antraksa

¹ At the time of designation

Language	Active ingredient	Indication
Spanish	Anticuerpo monoclonal humano contra el ántrax	Profilaxis post-exposición del carbunco (ántrax) por inhalación
Swedish	Human antrax monoklonal antikropp	Profylax efter exponering för mjältbrand beroende av inandning
Norwegian	Humant monoklonalt antistoff mot miltbrann	Posteksponeringsprofylakse mot lungemiltbrann
Icelandic	Einstofna mannamótefni gegn miltisbrandi	Til varnar innöndunarmiltisbrandi eftir útsetningu

Withdrawn