20 April 2011
EMA/COMP/65174/2011
Committee for Orphan Medicinal Products

# Public summary of opinion on orphan designation

## Human anthrax monoclonal antibody for the treatment of inhalation anthrax disease

On 15 April 2011, orphan designation (EU/3/11/852) was granted by the European Commission to Emergent Sales and Marketing Germany GmbH, Germany, for human anthrax monoclonal antibody for the treatment 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) in the environment until they find an organism in which 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 affected by the condition?

At the time of designation, inhalation anthrax disease affected not more than 0.01 in 10,000 people in the European Union (EU)*. This is equivalent to a total of not more than 500 people, 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).

*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).

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## What treatments are available?

At the time of designation, several antibiotics were authorised in the EU for the treatment of inhalation anthrax disease.

The sponsor has provided sufficient information to show that human anthrax monoclonal antibody might be of significant benefit for patients with 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 outcome of patients with this condition. 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 in patients who have developed the first signs of inhalation anthrax. By attaching to the anthrax toxins, the medicine is expected to stop them from entering into the body's cells, thereby reducing the symptoms of the disease.

## 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, human anthrax monoclonal antibody was not authorised anywhere in the EU for the treatment of 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 12 January 2011 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:

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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 languages[1], Norwegian and Icelandic

| Language | Active ingredient | Indication |
|---|---|---|
| English | Human anthrax monoclonal antibody | Treatment of inhalation anthrax disease |
| Bulgarian | Човешко антиантраксно моноклонално антитяло | Лечение на инхалационна антраксна болест |
| Czech | Lidská monokolnální protilátka proti antraxu | Léčba plicního (inhalačního) antraxu |
| Danish | Humant anthrax monoklonalt antistof | Behandling af sygdommen inhalationsanthrax |
| Dutch | Humaan monoklonaal anthrax-antilichaam | Behandeling (na blootstelling) van inhalatie miltvuurziekte |
| Estonian | Inimese siberi katku vastane monoklonaalne antikeha | Siberi katku kopsuvormi ekspositsioonijärgne ravi |
| Finnish | Ihmisen pernaruton monoklonaalinen vasta-aine | Hengitysteiden kautta leviävän pernaruton hoito |
| French | Anticorps monoclonal humain dirigé contre la toxine du bacille de la maladie du charbon | Traitement de la maladie du charbon par inhalation |
| German | Gegen Anthrax gerichteter humaner monoklonaler Antikörper | Behandlung von Lungenmilzbrand |
| Greek | Ανθρώπινο Μονοκλωνικό Αντίσωμα Άνθρακα | Θεραπεία της νόσου του αναπνευστικού άνθρακα |
| Hungarian | Anthrax elleni humán monoklonális antitest | Inhalációs anthrax betegség kezelése |
| Italian | Anticorpo monoclonale umano anti-antrace | Trattamento dell'antrace da inalazione |
| Latvian | Cilvēka monoklonālās antivielas pret sibīrijas mēri | Sibīrijas mēra plaušu formas ārstēšanai |
| Lithuanian | Žmogaus juodligės monokloninis antikūnas | Inhaliacinės juodligės gydymas |
| Maltese | Antikorp monoklonali uman kontra l-anthrax | Kura tal-marda tal-ġbid man-nifs tal-anthrax |
| Polish | Ludzkie przeciwciało monoklonalne przeciw wąglikowi | Leczenie wziewnych zakażeń wąglikiem |
| Portuguese | Anticorpo monoclonal contra o antraz humano | Tratamento da doença do antraz por inalação |
| Romanian | Anticorp monoclonal uman anti-antrax | Tratamentul boalii antrax prin inhalare |
| Slovak | Ľudská monoklonálna protilátka proti antraxu | Liečba pľúcnej formy ochorenia antrax |
| Slovenian | humano monoklonsko protitelo proti antraksu | Zdravljenje pljučnega antraksa |
| Spanish | Anticuerpo monoclonal humano contra el ántrax | Tratamiento de la enfermedad de carbunco (ántrax) por inhalación |
| Swedish | Human antrax monoklonal antikropp | Behandling av mjältbrand beroende av inandning |

[1] At the time of designation

| Language | Active ingredient | Indication |
|---|---|---|
| Norwegian | Humant monoklonalt antistoff mot miltbrann | Behandling av lungemiltbrann |
| Icelandic | Einstofna mannamótefni gegn miltisbrandi | Meðferð við innöndunarmiltisbrandi |