10 November 2014
EMA/COMP/560216/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Raxibacumab for the treatment of inhalation anthrax disease

On 15 October 2014, orphan designation (EU/3/14/1352) was granted by the European Commission to GlaxoSmithKline Trading Services Limited, Ireland, for raxibacumab 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) 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 haemorrhagic meningitis (inflammation and bleeding 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 less than 0.01 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer 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 28), Norway, Iceland and Liechtenstein. This represents a population of 511,100,000 (Eurostat 2014).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

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 raxibacumab might be of significant benefit for patients with inhalation anthrax disease because preclinical studies showed that the medicine improves survival when used in combination with current treatment. 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?

Raxibacumab is a monoclonal antibody, a type of protein that has been designed to recognise and attach to the anthrax toxins (i.e. to the anthrax protective antigen). By attaching to the anthrax toxins, the medicine is expected to stop them from entering the body's cells, thereby reducing the symptoms of the disease.

What is the stage of development of this medicine?

The effects of raxibacumab have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with raxibacumab in patients with inhalation anthrax disease were ongoing.

At the time of submission, raxibacumab was authorised in the United States for the treatment of adult and paediatric patients with inhalational anthrax due to *Bacillus anthracis* in combination with appropriate antibacterial medicines, and for prophylaxis of inhalational anthrax when alternative therapies are not available or are not appropriate.

At the time of submission, raxibacumab was not authorised anywhere in the EU for the treatment of inhalation anthrax disease. Orphan designation of the medicine had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 4 September 2014 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:

GlaxoSmithKline Trading Services Limited
Currabinny
Carrigaline
County Cork
Ireland
Tel. +353 21 4512212
Fax +353 21 43789 83
E-mail: neil.2.abbott@gsk.com

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	Raxibacumab	Treatment of inhalation anthrax disease
Bulgarian	Раксибакумаб	Лечение на инхалационна антраксна болест
Croatian	Raksibakumab	Liječenje plućnog antraksa
Czech	Raxibacumab	Léčba plicního (inhalačního) antraxu
Danish	Raxibacumab	Behandling af sygdommen inhalationsanthrax
Dutch	Raxibacumab	Behandeling (na blootstelling) van inhalatie miltvuurziekte
Estonian	Raksibakumaab	Siberi katku kopsuvormi ekspositsioonijärgne ravi
Finnish	Raksibakumabi	Hengitysteiden kautta leviävän pernaruton hoito
French	Raxibacumab	Traitement de la maladie du charbon par inhalation
German	Raxibacumab	Behandlung von Lungenmilzbrand
Greek	Ραξιμπακουμάμπη	Θεραπεία της νόσου του αναπνευστικού άνθρακα
Hungarian	Raxibacumab	Inhalációs anthrax betegség kezelése
Italian	Raxibacumab	Trattamento dell'antrace da inalazione
Latvian	Raxibacumab	Sibīrijas mēra plaušu formas ārstēšanai
Lithuanian	Raksibakumabas	Inhaliacinės juodligės gydymas
Maltese	Raxibacumab	Kura tal-marda tal-ġbid man-nifs tal-anthrax
Polish	Raksybakumab	Leczenie wziewnych zakażeń wąglikiem
Portuguese	Raxibacumab	Tratamento da doença do antraz por inalação
Romanian	Raxibacumab	Tratamentul boalii antrax prin inhalare
Slovak	Raxibakumab	Liečba pľúcnej formy ochorenia antrax
Slovenian	raksibakumab	Zdravljenje pljučnega antraksa
Spanish	Raxibacumab	Tratamiento de la enfermedad de carbunco (ántrax) por inhalación
Swedish	Raxibacumab	Behandling av mjältbrand beroende av inandning
Norwegian	Raksibakumab	Behandling av lungemiltbrann
Icelandic	Raxibacúmab	Meðferð við innöndunarmiltisbrandi

[1] At the time of designation