22 November 2018
EMA/532463/2018

Public summary of opinion on orphan designation

Obiltoxaximab for the treatment of anthrax

On 24 August 2018, orphan designation (EU/3/18/2065) was granted by the European Commission to SFL Regulatory Services GmbH, Austria, for obiltoxaximab for the treatment of anthrax.

What is anthrax?

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.

Anthrax 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, anthrax 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).

What treatments are available?

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

[*]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 517,400,000 (Eurostat 2018).

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

The sponsor has provided sufficient information to show that obiltoxaximab might be of significant benefit for patients with anthrax because laboratory studies indicate that the medicine may improve survival when used in combination with current treatments. 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?

Obiltoxaximab is a monoclonal antibody, a type of protein which has been designed to recognise and attach to a component of anthrax toxin called 'anthrax protective antigen' that allows the toxin to lock onto and enter cells. By attaching to the protective antigen in this way, the medicine is expected to stop the toxin 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 obiltoxaximab 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 anthrax had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for anthrax. Orphan designation was granted in the United States for the treatment of exposure to *B. anthracis* spores.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 19 July 2018 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's rare disease designations page.

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	Obiltoxaximab	Treatment of anthrax
Bulgarian	Обилтоксаксимаб	Лечение на антракс
Croatian	Obiltoksaksimab	Liječenje antraksa
Czech	Obiltoxaximab	Léčba plicního antraxu
Danish	Obiltoxaximab	Behandling af anthrax (miltbrand)
Dutch	Obiltoxaximab	Behandeling van miltvuur
Estonian	Obiltoksaksimab	Siberi katku ravi
Finnish	Obiltoksaksimabi	Pernaruton hoito
French	Obiltoxaximab	Traitement de la maladie du charbon
German	Obiltoxaximab	Behandlung von Milzbrand
Greek	Ομπιλτοξαξιμάμπη	Θεραπεία του άνθρακα
Hungarian	Obiltoxaximab	Anthrax betegség kezelése
Italian	Obiltoxaximab	Trattamento dell'antrace
Latvian	Obiltoksaksimabs	Sibīrijas mēra ārstēšana
Lithuanian	Obiltoksaksimabas	Juodligės gydymas
Maltese	Obiltossassimab	It-trattament tal-antraċe
Polish	Obiltoxaximab	Leczenie wąglika
Portuguese	Obiltoxaximab	Tratamento do antraz
Romanian	Obiltoxaximab	Tratamentul antraxului
Slovak	Obiltoxaximab	Liečba antraxu
Slovenian	Obiltoksaksimab	Zdravljenje antraksa
Spanish	Obiltoxaximab	Tratamiento de la enfermedad de carbunco (ántrax)
Swedish	Obiltoxaximab	Behandling av mjältbrand
Norwegian	Obiltoxaximab	Behandling av miltbrann
Icelandic	Obiltoxaximab	Meðferð við miltisbrandi

[1] At the time of designation