



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Chimeric monoclonal antibody to shiga-toxin 1 and 2 for the treatment of shiga-toxin producing bacterial infection

On 26 August 2005, orphan designation (EU/3/05/301) was granted by the European Commission to Albany Regulatory Consulting Ltd, United Kingdom, for chimeric monoclonal antibody to shiga-toxin 1 and 2 for the treatment of shiga-toxin producing bacterial infection.

The sponsorship was transferred to LFB-Biotechnologies, France, in March 2012.

#### What is shiga-toxin producing bacterial infection?

Shiga-toxin producing bacterial infection comprises infections caused by bacteria called *Shigella dysenteriae* type 1 and *Escherichia coli*. These bacteria can produce a certain poison (shiga toxin). Shiga-toxin induces destruction of cell parts needed to produce proteins. Patients get the disease through infected food. As a consequence, these kinds of infections can cause a serious gastrointestinal disease (acute inflammation of the lining of the stomach and the intestines with symptoms such as lack of appetite, nausea, diarrhea with the presence of blood in the stools and severe abdominal cramps). As a consequence of the infection, the kidneys may be damaged; this is usually accompanied by the destruction of red blood cells (haemolysis) and platelets (cells involved in blood clotting) and called haemolytic uraemia syndrome (HUS). Shiga-toxin producing bacterial infection is a life-threatening condition.

#### What is the estimated number of patients affected by the condition?

At the time of designation, shiga-toxin producing bacterial infection affected approximately 2.1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 98,000 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).

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\*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 25), Norway, Iceland and Liechtenstein. At the time of designation, this represented a population of 466,600,000 (Eurostat 2005).



## What treatments are available?

The current management of the disease consists of supportive treatment, such as the restoration of fluid content to the body that is dehydrated. Several medicinal products were authorised in the Community for treatment of infections caused by *Shigella spp.* or *E. coli* at the time of submission of the application for orphan designation. Satisfactory argumentation has been submitted by the sponsor to justify the assumption that chimeric monoclonal antibody to shiga-toxin 1 and 2 might be of potential significant benefit for the treatment of shiga-toxin producing bacterial infection because it may offer a new way to fight the disease and it might improve the long-term outcome of the patients. The assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

## How is this medicine expected to work?

Some bacteria have the ability to produce toxins that have a harmful effect on the normal functioning of cells. Chimeric monoclonal antibody to shiga-toxin 1 and 2 is expected to neutralize the circulating toxins thus blocking the direct action of the shiga-toxin 1 and 2 on the kidney.

## What is the stage of development of this medicine?

The effects of chimeric monoclonal antibody to shiga-toxin 1 and 2 were evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials in patients with shiga-toxin producing bacterial infection were initiated.

Chimeric monoclonal antibody to shiga-toxin 1 and 2 was not authorised anywhere worldwide for shiga-toxin producing bacterial infection or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 July 2005 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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France  
Telephone: + 33 1 69 82 17 41  
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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<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Chimeric monoclonal antibody to shiga-toxin 1 and 2	Treatment of shiga-toxin producing bacterial infection.
Bulgarian	Химерно моноклонално анти тяло срещу шига токсини 1 и 2	Лечение на инфекция с бактерии, отделящи шига токсини
Czech	Chimérické monoklonální protilátky proti shiga-toxinu 1 a 2	Léčba bakteriálních infekcí vyvolaných shiga-toxinem
Danish	Kimerisk monoklonalt antistof til shigatoksin 1 og 2	Behandling af shigatoksin-producerende bakterieinfektion.
Dutch	Chimeer monoklonaal antilichaam gericht tegen shiga-toxine 1 en 2	Behandeling van een shiga-toxine producerende bacteriële infectie.
Estonian	Shigatoksiin 1 ja 2 vastane kimäärne monoklonaalne antikeha	Shigatoksiinist põhjustatud bakteriaalse infektsiooni ravi
Finnish	Kimeerinen monoklonaalinen vasta-aine shiga-toksiinille 1 ja 2	Shiga-toksiinia tuottavan bakteeri-infektion hoito.
French	Anticorps monoclonal chimérique dirigé contre les shiga-toxines 1 et 2	Traitement de l'infection bactérienne productrice de shiga-toxines
German	Chimärer monoklonaler Antikörper gegen Shiga-Toxin-1 und -2	Behandlung von Shiga-Toxin produzierender bakterieller Infektion.
Greek	Χιμαϊρικό μονοκλωνικό αντίσωμα έναντι τοξίνης shiga 1 και 2	Θεραπεία βακτηριακής λοίμωξης που παράγει τοξίνη shiga.
Hungarian	Shiga toxin 1 és 2 elleni chimer monoklonális antitest	Shiga toxint termelő bakteriális fertőzés kezelése
Italian	Anticorpo monoclonale chimerico anti-shiga-tossina 1 e 2	Trattamento dell'infezione batterica che produce la shiga-tossina
Latvian	Himērās monoklonālās antivielas pret <i>shiga</i> - toksīniem 1 un 2	<i>Shiga</i> - toksīnu izdalošu baktēriju izraisītas infekcijas ārstēšana
Lithuanian	Chimeriniai monokloniniai antikūnai šiga-toksinui 1 ir 2	Bakterinės infekcijos, kurios metu išsiskiria šiga-toksinas, gydymas
Maltese	Antikorp monoklonali kimeriku għat-tossini shiga 1 u 2	Kura ta' infezzjoni minn batterji li jipproduċu it-tossina shiga

<sup>1</sup> At the time of transfer of sponsorship

Language	Active ingredient	Indication
Polish	Chimeryczne przeciwciało monoklonalne przeciwko toksynom Shiga 1 i 2	Leczenie zakażenia wywołanego bakteriami wytwarzającymi toksyny Shiga
Portuguese	Anticorpo monoclonal quimérico da toxina shiga 1 e 2	Tratamento da infecção causada por bacterias productoras de toxina shiga.
Romanian	Anti-corpi monoclonali chimerici contra toxinei shiga 1 si 2	Tratamentul infectiilor cauzate de bacterii care produc toxina shiga
Slovak	Chimérická monoklonová protilátka proti šigatoxínu 1 a 2	Liečba baktérievej infekcie vyvolanej šigatoxínom
Slovenian	Himerno monoklonsko protitelo za shiga-toksin 1 in 2	Zdravljenje okužb z bakterijami, ki proizvajajo toksin shiga.
Spanish	Anticuerpo monoclonal quimérico contra las toxinas Shiga 1 y 2	Tratamiento de las infecciones causadas por bacterias productoras de Shiga toxina
Swedish	Chimär monoklonal antikropp mot shigatoxin 1 och 2	Behandling av shigatoxinproducerande bakterieinfektion.
Norwegian	Kimært monoklonalt antistoff mot shiga-toksin 1 og 2	Behandling av shiga-toksinproduserende bakterieinfeksjon
Icelandic	Einstofna blendingsmótefni gegn shiga-eitri 1 og 2	Meðferð við sýkingum af bakteríum sem framleiða shiga-eitur