



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

3 July 2012
EMA/COMP/64665/2012
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Human monoclonal antibody targeting *Staphylococcus aureus* alpha-toxin for the treatment of pneumonia caused by *Staphylococcus aureus*

On 5 March 2012, orphan designation (EU/3/12/968) was granted by the European Commission to Envestia Limited, United Kingdom, for human monoclonal antibody targeting *Staphylococcus aureus* alpha-toxin (also known as KBSA301) for the treatment of pneumonia caused by *Staphylococcus aureus*.

What is pneumonia caused by *Staphylococcus aureus*?

Pneumonia (infection of the lungs) can be caused by the bacterium *Staphylococcus aureus* (*S. aureus*). About 30% of people carry this bacterium in their nose, but most of the time it does not cause any harm. People who are seriously ill and whose immune system (the body's natural defences) is weakened are particularly vulnerable to infection with *S. aureus*. Methicillin-resistant *S. aureus* (MRSA) is a strain of *S. aureus* that has become resistant to most antibiotics and is therefore difficult to treat.

Pneumonia caused by *S. aureus* is a life-threatening disease because it may lead to septicaemia (blood infection) and respiratory failure.

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

At the time of designation, pneumonia caused by *S. aureus* affected not more than 1.2 in 10,000 people in the European Union (EU)*. This is equivalent to a total of not more than 61,000 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).

What treatments are available?

At the time of designation, several antibiotics were authorised in the EU to treat this condition.

*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).



The sponsor has provided sufficient information to show that 'human monoclonal antibody targeting *Staphylococcus aureus* alpha-toxin' might be of significant benefit for patients with pneumonia caused by *S. aureus* because it works in a different way to existing treatments, and early studies in experimental models indicate that it might be used together with antibiotics to improve the treatment 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 *S. aureus* alpha-toxin, which is one of the toxic substances released by the bacterium during the infection. By attaching to alpha-toxin, this medicine is expected to reduce the ability of the bacterium to cause damage to the body's cells.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of the medicine in experimental models was ongoing.

At the time of submission, no clinical trials with the medicine in patients with pneumonia caused by *S. aureus* had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for pneumonia caused by *S. aureus* or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 11 January 2012 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:

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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 monoclonal antibody targeting <i>Staphylococcus aureus</i> alpha-toxin	Treatment of pneumonia caused by <i>Staphylococcus aureus</i>
Bulgarian	Човешко моноклонално антитяло, насочено срещу алфа-токсина на <i>Staphylococcus aureus</i>	Лечение на пневмония, причинена от <i>Staphylococcus aureus</i>
Czech	Lidská monoklonální protilátka cílená proti alfatoxinu produkovanému zlatým stafylokokem	Léčba zápalu plic způsobeného zlatým stafylokokem
Danish	Human monoklonalt antistof rettet mod <i>Staphylococcus aureus</i> alfatoksin	Behandling af pneumoni forårsaget af <i>Staphylococcus aureus</i>
Dutch	Humaan monoklonaal antilichaam tegen <i>Staphylococcus aureus</i> alfa-toxine	Behandeling van pneumonie veroorzaakt door <i>Staphylococcus aureus</i>
Estonian	Inimese monoklonaalne antikeha <i>Staphylococcus aureus</i> 'e produtseeritud alfatoksiini vastu	<i>Staphylococcus aureus</i> 'e põhjustatud kopsupõletiku ravi
Finnish	Ihmisen monoklonaalinen vasta-aine <i>Staphylococcus aureus</i> -bakteerin alfa-toksiineja vastaan	Ihmisen monoklonaalinen vasta-aine <i>Staphylococcus aureus</i> -bakteerin alfa-toksiineja vastaan
French	Anticorps monoclonal humain ciblant la toxine alpha de <i>Staphylococcus aureus</i>	Traitement de la pneumonie due à <i>Staphylococcus aureus</i>
German	Humaner monoklonaler Antikörper gegen das <i>Staphylococcus aureus</i> alpha-Toxin	Behandlung von Pneumonien, die von <i>Staphylococcus aureus</i> verursacht werden
Greek	Ανθρώπινο μονοκλωνικό αντίσωμα που στοχεύει την άλφα τοξίνη του χρυσίζοντος Σταφυλόκοκκου	Θεραπεία πνευμονίας προκαλούμενης από τον χρυσίζοντα Σταφυλόκοκκο
Hungarian	<i>Staphylococcus aureus</i> alfa-toxint célzó humán monoklonális antitest	<i>Staphylococcus aureus</i> által okozott pneumonia kezelése
Italian	Anticorpo monoclonale umano contro la tossina alfa di <i>Staphylococcus aureus</i>	Trattamento della polmonite causata da <i>Staphylococcus aureus</i>
Latvian	Cilvēka monoklonālās antivielas pret zeltainā stafilokoka alfa toksīnu	Zeltainā stafilokoka izraisītās pneimonijas ārstēšana
Lithuanian	Žmogaus monokloniniai antikūnai nukreipti prieš <i>Staphylococcus aureus</i> alfa toksiną	Pneumonijos, sukeltos <i>Staphylococcus aureus</i> , gydymas
Maltese	Anti-korp monoklonali uman kontra alfa-tossin ta' <i>staphylococcus aureus</i>	Kura ta' pnevmonja kkawżata minn <i>Staphylococcus aureus</i>
Polish	Ludzkie przeciwciało monoklonalne przeciwko toksynie alfa wytwarzanej przez gronkowca złocistego	Leczenie zapalenia płuc wywołanego przez gronkowca złocistego (<i>Staphylococcus aureus</i>)

¹ At the time of designation

Language	Active ingredient	Indication
	(<i>Staphylococcus aureus</i>)	
Portuguese	Anticorpo monoclonal humano anti-toxina alfa do <i>Staphylococcus aureus</i>	Tratamento da pneumonia provocada por <i>Staphylococcus aureus</i>
Romanian	Anticorp monoclonal uman îndreptat împotriva toxinei alfa a <i>Staphylococcus aureus</i>	Tratamentul pneumoniei produse de <i>Staphylococcus aureus</i>
Slovak	Ľudská monoklonálna protilátka zameraná proti alfa-toxínu <i>Staphylococcus aureus</i>	Liečba pneumónie spôsobenej <i>Staphylococcus aureus</i>
Slovenian	Človeško monoklonsko protitelo, ki deluje na alfa toksin bakterije <i>Staphylococcus aureus</i>	Zdravljenje pljučnice, ki jo povzroči bakterija <i>Staphylococcus aureus</i>
Spanish	Anticuerpo monoclonal humano dirigido contra la toxina alfa de <i>Staphylococcus aureus</i>	Tratamiento de la neumonía causada por <i>Staphylococcus aureus</i>
Swedish	Human monoklonal antikropp riktad mot <i>Staphylococcus aureus</i> alfatoxin	Behandling av pneumoni orsakad av <i>Staphylococcus aureus</i>
Norwegian	Humant monoklonalt antistoff rettet mot <i>Staphylococcus aureus</i> alfa-toksin	Behandling av pneumoni forårsaket av <i>Staphylococcus aureus</i>
Icelandic	Einstofna manna mótefni sem beinist gegn <i>Staphylococcus aureus</i> alfatoxíni	Meðferð við <i>Staphylococcus aureus</i> lungnabólgu