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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

Pegylated recombinant anti-*Pseudomonas aeruginosa* PcrV Fab' antibody for the treatment of *Pseudomonas aeruginosa* lung infection in cystic fibrosis

First publication	12 September 2013
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<b>Disclaimer</b> Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

***Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in January 2015 on request of the Sponsor.***

On 5 August 2013, orphan designation (EU/3/13/1178) was granted by the European Commission to KaloBios Ltd, United Kingdom, for pegylated recombinant anti-*Pseudomonas aeruginosa* PcrV Fab' antibody for the treatment of *Pseudomonas aeruginosa* lung infection in cystic fibrosis.

### **What is *Pseudomonas aeruginosa* lung infection in cystic fibrosis?**

Cystic fibrosis is a genetic disease that affects the production of secretions such as mucus in the body. In patients with cystic fibrosis, there is an overproduction of thick mucus in the lungs, which leads to inflammation and a high risk for the lungs to become infected with bacteria. *Pseudomonas aeruginosa* (*P. aeruginosa*) is one of the most common types of bacteria that tend to infect the lungs of patients with cystic fibrosis.

*P. aeruginosa* lung infection in cystic fibrosis is a long-term debilitating disease and may be life threatening because it severely damages the lung tissue and does not allow the patient to breathe normally.



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

At the time of designation, *P. aeruginosa* lung infection in cystic fibrosis affected less than 0.6 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 31,000 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, *P. aeruginosa* lung infection in patients with cystic fibrosis was mainly treated with antibiotics (medicines that kill bacteria). These were available as tablets, infusions (drips into a vein) and solutions for inhalation using a special machine called a nebuliser that changes the solution into an aerosol so that the patient can breathe it in. Other medicines used to treat the lung symptoms of cystic fibrosis included bronchodilators that help open up the airways in the lungs and mucolytics that help to dissolve the mucus.

The sponsor has provided sufficient information to show that pegylated recombinant anti-*P. aeruginosa* PcrV Fab' antibody might be of significant benefit for patients with *P. aeruginosa* lung infection in cystic fibrosis because it works in a different way to existing treatments, by targeting the protein that enables *P. aeruginosa* to attack and kill the host cell. Early studies show that it may reduce infection and inflammation of the airways, including when used together with existing antibiotic 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?

The medicine consists of an antibody called the 'anti-*P. aeruginosa* PcrV Fab' antibody, which blocks the action of a protein called PcrV. This protein is used by the *P. aeruginosa* bacterium to attack and kill human cells, either by puncturing them or injecting toxins into them. By blocking this protein, the medicine is expected to prevent the effects of infection by *P. aeruginosa* in patients with cystic fibrosis.

The antibody is produced by a method known as 'recombinant DNA technology': it is made by cells into which genes (DNA) have been introduced that makes the cells able to produce the antibody. The antibody has also been modified by a process called 'pegylation'. This means that a chemical called 'polyethylene glycol' has been attached to the antibody, which is expected to prolong the time needed to remove the medicine from the body.

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

The effects of the medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the medicine in cystic fibrosis patients with *P. aeruginosa* lung infection were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for *P. aeruginosa* lung infection in patients with cystic fibrosis or designated as an orphan medicinal product elsewhere for this condition.

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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 28), Norway, Iceland and Liechtenstein. At the time of designation, this represented a population of 512,200,000 (Eurostat 2013).

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 11 July 2013 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:

KaloBios Ltd  
5 Fleet Place  
EC4M 7RD London  
United Kingdom  
Tel. +44 (0)800 279 5233  
Fax +44 (0)800 471 5087  
E-mail: [kalobios.info@chiltern.com](mailto:kalobios.info@chiltern.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<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Pegylated recombinant anti- <i>Pseudomonas aeruginosa</i> PcrV Fab' antibody	Treatment of <i>Pseudomonas aeruginosa</i> lung infection in cystic fibrosis
Bulgarian	Пегилирано рекомбинантно анти - <i>Pseudomonas aeruginosa</i> PcrV Fab' антитяло	Лечение на <i>Pseudomonas aeruginosa</i> белодробна инфекция при кистична фиброза
Croatian	Pegilirani rekombinantni Fab' fragment protutijela protiv PcrV <i>Pseudomonas aeruginosa</i>	Liječenje infekcije pluća s <i>Pseudomonas aeruginosa</i> u cističnoj fibrozi
Czech	Rekombinantní pegylovaná protilátka proti PcrFab <i>Pseudomonas aeruginosa</i>	Léčba plicních infekcí vyvolaných <i>Pseudomonádou aeruginosa</i> při cystické fibróze
Danish	Pegyleret recombinant anti- <i>Pseudomonas aeruginosa</i> PcrV Fab' antistof	Behandling af lungeinfektion med <i>Pseudomonas aeruginosa</i> ved cystisk fibrose
Dutch	Gepegyleerd recombinant anti- <i>Pseudomonas aeruginosa</i> PcrV Fab' antilichaam	Behandeling van <i>Pseudomonas aeruginosa</i> longinfectie bij cystische fibrosis
Estonian	Pegüleeritud rekombinantne <i>Pseudomonas aeruginosa</i> PcrV Fab' vastane antikeha	<i>Pseudomonas aeruginosa</i> poolt põhjustatud kopsuinfektsiooni ravi tsüstilise fibroosi korral
Finnish	Pegyloitu, rekombinanttiteknikalla tehty <i>Pseudomonas aeruginosa</i> PcrV Fab - proteiinin vasta-aine	<i>Pseudomonas aeruginosa</i> aiheuttaman keuhkoinfektion hoito kystisessä fibroosissa
French	Anticorps anti <i>Pseudomonas aeruginosa</i> PcrV Fab' recombinant et pegylé	Traitement des infections pulmonaires à <i>Pseudomonas aeruginosa</i> dans la mucoviscidose
German	Pegylierter rekombinanter anti- <i>Pseudomonas aeruginosa</i> PcrV Fab' Antikörper	Therapie der <i>Pseudomonas aeruginosa</i> -Infektion der Lunge bei zystischer Fibrose
Greek	Πεγκυλιωμένο ανασυνδυσμένο Fab τμήμα αντισώματος έναντι της PcrV της πυοκυανικής ψευδομονάδας	θεραπεία λοιμώξεων των πνευμόνων με <i>Pseudomonas aeruginosa</i> κατά την κυστική ίνωση
Hungarian	Pegilált rekombináns anti- <i>Pseudomonas aeruginosa</i> PcrV Fab' antitest	<i>Pseudomonas aeruginosa</i> okozta tüdőfertőzés kezelése cisztikus fibrózisban
Italian	Anticorpo Ricombinante Pegilato PcrV Fab' contro <i>Pseudomonas aeruginosa</i>	Trattamento di infezione polmonare da <i>Pseudomonas aeruginosa</i> nella fibrosi cistica
Latvian	Pegilēta rekombinantā pret- <i>Pseudomonas aeruginosa</i> PcrV Fab'antiviela	<i>Pseudomonas aeruginosa</i> izraisītas plaušu infekcijas ārstēšana cistiskās fibrozes gadījumā

<sup>1</sup> At the time of designation

Language	Active ingredient	Indication
Lithuanian	Pegiliuotas rekombinantis anti- <i>Pseudomonas aeruginosa</i> PcrV Fab' antikūnas	Plaučių infekcijos, sukeltos <i>Pseudomonas aeruginosa</i> , gydymas, sergant cistine fibroze
Maltese	Antikorp Fab' rikombinanti pegilat kontra l- <i>Pseudomonas aeruginosa</i> PcrV	Kura ta' infezzjoni fil-pulmun mill- <i>Pseudomonas aeruginosa</i> fil-fibrozi čistiku
Polish	Pegylowane rekombinowane przeciwciało monoklonalne Fab przeciw <i>Pseudomonas aeruginosa</i> PcrV	Leczenie zapalenia płuc wywołanych przez <i>Pseudomonas aeruginosa</i> w przebiegu zwłóknienia torbielowatego
Portuguese	Anticorpo recombinante peguilado anti- <i>Pseudomonas aeruginosa</i> PcrV Fab'	Tratamento de infecção pulmonar por <i>Pseudomonas aeruginosa</i> na fibrosa quística
Romanian	Anticorp PcrV Fab' recombinant pegilat anti <i>Pseudomonas aeruginosa</i>	Tratamentul infecției pulmonare cu <i>Pseudomonas aeruginosa</i> la pacienții cu fibroză chistică
Slovak	Pegylovaná rekombinantná anti- <i>Pseudomonas aeruginosa</i> PcrV Fab' protilátka	Liečba infekcií pľúc s <i>Pseudomonas aeruginosa</i> pri cystickej fibróze
Slovenian	Pegilirano rekombinantno PcrV Fab' protitelo proti <i>Pseudomonas aeruginosa</i>	Zdravljenje pljučnice povzročene s <i>Pseudomonas aeruginosa</i> pri cistični fibrozi
Spanish	Anticuerpo recombinante anti- <i>Pseudomonas aeruginosa</i> PcrV Fab'	Tratamiento de las infecciones pulmonares por <i>Pseudomonas aeruginosa</i> en la fibrosis quística
Swedish	Pegylerad rekombinant anti- <i>Pseudomonas aeruginosa</i> PcrV Fab' antikropp	Behandling av lunginflammation orsakad av <i>Pseudomonas aeruginosa</i> vid cystisk fibros
Norwegian	Pegylert rekombinant anti- <i>Pseudomonas aeruginosa</i> PcrV Fab' antistoff	Behandling av lungeinfeksjon forårsaket av <i>Pseudomonas aeruginosa</i> ved cystisk fibrose
Icelandic	Pegýlorað raðbrigða anti- <i>Pseudomonas aeruginosa</i> PcrV Fab' mótefni	Meðferð á <i>Pseudomonas aeruginosa</i> lungnasýkingum í slímseigjusjúkdómi