23 February 2015
EMA/COMP/736592/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Amikacin sulfate for the treatment of *Pseudomonas aeuriginosa* lung infection in cystic fibrosis

On 16 December 2014, orphan designation (EU/3/14/1397) was granted by the European Commission to PlumeStars s.r.l., Italy, for amikacin sulfate for the treatment of *Pseudomonas aeuriginosa* lung infections in cystic fibrosis.

What is *Pseudomonas aeuriginosa* 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 of the lungs becoming 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 approximately 0.8 in 10,000 people in the European Union (EU). This was equivalent to a total of around 41,000 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).

[*]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).

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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. 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 the medicine might be of significant benefit for patients with the condition because it will be available as a new formulation (powder for inhalation) which would allow patients to use amikacin sulfate outside of the hospital. This could be of major advantage for patients with the 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?

Amikacin is an antibiotic that is already available in the EU as a solution for infusion for the treatment of *P. aeruginosa* infection in patients with cystic fibrosis. It belongs to the group 'aminoglycosides' and works by disrupting the production of proteins that bacteria need to build their cell walls, thereby damaging the bacteria and eventually killing them. This will prevent *P. aeruginosa* infection from developing in the lungs and prevent the inflammation caused by the infection.

What is the stage of development of this medicine?

The effects of amikacin sulfate have not been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with amikacin sulfate in patients with the condition had been started.

At the time of submission, amikacin sulfate 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.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 November 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:

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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[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Amikacin sulfate	Treatment of *Pseudomonas aeruginosa* lung infection in cystic fibrosis
Bulgarian	Амикацин сулфат	Лечение на *Pseudomonas aeruginosa* белодробна инфекция при кистична фиброза
Croatian	Amikacin-sulfat	Liječenje infekcije pluća s *Pseudomonas aeruginosa* u cističnoj fibrozi
Czech	Amikacin sulfát	Léčba plicních infekcí vyvolaných *Pseudomonádou aeruginosa* při cystické fibróze
Danish	Amikacinsulfat	Behandling af lungeinfektion med *Pseudomonas aeruginosa* ved cystisk fibrose
Dutch	Amikacinesulfaat	Behandeling van *Pseudomonas aeruginosa* longinfectie bij cystische fibrosis
Estonian	Amikatsiinsulfaat	*Pseudomonas aeruginosa* poolt põhjustatud kopsuinfektsiooni ravi tsüstilise fibroosi korral
Finnish	Amikasiinisulfaatti	*Pseudomonas aeruginosan* aiheuttaman keuhkoinfektion hoito kystisessä fibroosissa
French	Sulfate d'amikacine	Traitement des infections pulmonaires à *Pseudomonas aeruginosa* dans la mucoviscidose
German	Amikacinsulfat	Therapie der *Pseudomonas aeruginosa*-Infektion der Lunge bei zystischer Fibrose
Greek	Θειική αμικασίνη	θεραπεία λοιμώξεων των πνευμόνων με *Pseudomonas aeruginosa* κατά την κυστική ίνωση
Hungarian	Amikacin szulfát	Pseudomonas aeruginosa okozta tüdőfertőzés kezelése cisztikus fibrózisban
Italian	Amikacina solfato	Trattamento di infezione polmonare da *Pseudomonas aeruginosa* nella fibrosi cistica
Latvian	Amikacīna sulfāts	*Pseudomonas aeruginosa* izraisītas plaušu infekcijas ārstēšana cistiskās fibrozes gadījumā
Lithuanian	Amikacino sulfatas	Plaučių infekcijos, sukeltos *Pseudomonas aeruginosa*, gydymas, sergant cistine fibroze
Maltese	Amikacin sulfate	Kura ta' infezzjoni fil-pulmun mill-*Pseudomonas aeruginosa* fil-fibrożi ċistiku
Polish	Amikacyny siarczan	Leczenie zapalenia płuc wywołanych przez *Pseudomonas aeruginosa* w przebiegu zwłóknienia torbielowatego
Portuguese	Sulfato de amicacina	Tratamento de infecção pulmonar por *Pseudomonas aeruginosa* na fibrosa quística
Romanian	Sulfat de amikacină	Tratamentul infecţiei pulmonare cu *Pseudomonas aeruginosa* la pacienţii cu fibroză chistică
Slovak	Amikacín sulfát	Liečba infekcií pľúc s *Pseudomonas aeruginosa* pri cystickej fibróze

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Amikacinov sulfat	Zdravljenje pljučnice povzročene s *Pseudomonasom aeruginosa* pri cistični fibrozi
Spanish	Sulfato de amikacina	Tratamiento de las infecciones pulmonares por *Pseudomonas aeruginosa* en la fibrosis quística
Swedish	amikasinsulfat	Behandling av lunginflammation orsakad av *Pseudomonas aeruginosa* vid cystisk fibros
Norwegian	Amikacinsulfat	Behandling av lungeinfeksjon forårsaket av *Pseudomonas aeruginosa* ved cystisk fibrose
Icelandic	Amikasín súlfat	Meðferð á *Pseudomonas aeruginosa* lungnasýkingum í slímseigjusjúkdómi