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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Amikacin sulfate (liposomal) for the treatment of *Pseudomonas aeruginosa* lung infection in cystic fibrosis

First publication	1 April 2009
Rev.1: transfer of sponsorship	17 November 2011
Rev.2: sponsor's change of address	27 June 2014

Disclaimer

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.

On 25 July 2006, orphan designation (EU/3/06/387) was granted by the European Commission to Morgan Lewis & Bockius, United Kingdom, for amikacin sulfate (liposomal) for the treatment of *Pseudomonas aeruginosa* lung infection in cystic fibrosis.

The sponsorship was transferred to Transave Inhalation Biotherapeutics Limited, United Kingdom, in July 2008. Transave Inhalation Biotherapeutics Limited changed its name to Insmed Limited in October 2011.

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

Cystic fibrosis is a hereditary (genetic) disease that affects the production of secretions (such as mucus) from the glands in the body. It affects the lungs and the digestive system (gut) in particular. Cystic fibrosis is caused by abnormalities in a gene called 'cystic fibrosis transmembrane conductance regulator' (CFTR). The *CFTR* gene is responsible for the production of CFTR, a protein that regulates the production of mucus and digestive juices by acting as a chloride ion channel to allow proper movement of salt and water in and out of certain cells in the lungs and other tissues. In patients with cystic fibrosis, there is an overproduction of mucus in the lungs and a reduced production of digestive juices from the pancreas (an organ near the stomach). This leads to long-term infection and inflammation of the lungs and problems with the digestion and absorption of food resulting in poor growth. One of the most common kinds of bacterial infections is with *Pseudomonas aeruginosa*. In the long term, these events can induce damage to the lung tissue and the disease becomes life-threatening.

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

At the time of designation cystic fibrosis affected approximately 1.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 61,000 people*, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of submission of the application for orphan drug designation, lung infection and inflammation in cystic fibrosis were treated mainly with antibiotics (drugs that kill bacteria). These can be taken in a number of ways; as tablets, as intravenous infusion or as inhalation. Other medications to treat the lung symptoms of cystic fibrosis included bronchodilators (medications that enlarge the width of the airways) and mucolytics (drugs that help to dissolve the lung secretions). Associated treatments included daily exercise and physiotherapy and several other types of medications such as pancreatic enzymes and food supplements for the digestive symptoms. Satisfactory argumentation has been submitted by the sponsor to justify the assumption that amikacin sulfate (liposomal) might be of potential significant benefit for the treatment of cystic fibrosis mainly because it may provide a major contribution to patient care. This 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?

The medicinal product is an aminoglycoside, a kind of antibiotic that works by disrupting the building of the bacterial cell wall which consequently stops the bacteria from growing and multiplying in the lungs. This will prevent *P. aeruginosa* infection from developing in the lungs and prevent the inflammation that the infection causes.

What is the stage of development of this medicine?

The effects of amikacin sulfate (liposomal) were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with cystic fibrosis were ongoing.

Amikacin sulfate was authorised in several countries worldwide as an antibiotic, at the time of submission. Orphan designation of amikacin sulfate (liposomal) was granted in the United States for treatment of bronchopulmonary *Pseudomonas aeruginosa* infection in cystic fibrosis patients.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 15 June 2006 recommending the granting of this designation.

*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 468,900,000 (Eurostat 2006).

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 (liposomal)	Treatment of *Pseudomonas aeruginosa* lung infection in cystic fibrosis
Bulgarian	Амикацин сулфат (липозомен)	Лечение на *Pseudomonas aeruginosa* белодробна инфекция при кистична фиброза
Czech	Amikacin sulfát (liposomální)	Léčba plicních infekcí vyvolaných Pseudomonádou *aeruginosa* při cystické fibróze
Danish	Amikacinsulfat (liposomal)	Behandling af lungeinfektion med *Pseudomonas aeruginosa* ved cystisk fibrose
Dutch	Amikacinesulfaat (liposomal)	Behandeling van *Pseudomonas aeruginosa* longinfectie bij cystische fibrosis
Estonian	Amikatsiinsulfaat (liposomaalne)	*Pseudomonas aeruginosa* poolt põhjustatud kopsuinfektsiooni ravi tsüstilise fibroosi korral
Finnish	Amikasiinisulfaatti (liposomaalinen)	*Pseudomonas aeruginosan* aiheuttaman keuhkoinfektion hoito kystisessä fibroosissa
French	Sulfate d'amikacine (liposomal)	Traitement des infections pulmonaires à *Pseudomonas aeruginosa* dans la mucoviscidose
German	Amikacinsulfat (liposomal)	Therapie der *Pseudomonas aeruginosa*-Infektion der Lunge bei zystischer Fibrose
Greek	Θειική αμικασίνη (λιποσωμική)	θεραπεία λοιμώξεων των πνευμόνων με *Pseudomonas aeruginosa* κατά την κυστική ίνωση
Hungarian	Amikacin szulfát (liposzómában)	*Pseudomonas aeruginosa* okozta tüdőfertőzés kezelése cisztikus fibrózisban
Italian	Amikacina solfato (liposomale)	Trattamento di infezione polmonare da *Pseudomonas aeruginosa* nella fibrosi cistica
Latvian	Amikacīna sulfāts (liposomu)	*Pseudomonas aeruginosa* izraisītas plaušu infekcijas ārstēšana cistiskās fibrozes gadījumā
Lithuanian	Amikacino sulfatas (liposomų)	Plaučių infekcijos, sukeltos *Pseudomonas aeruginosa*, gydymas, sergant cistine fibroze
Maltese	Amikacin sulfate (liposomali)	Kura ta' infezzjoni fil-pulmun mill-*Pseudomonas aeruginosa* fil-fibroži ċistiku
Polish	Siarczan amikacyny (postać liposomalna)	Leczenie zapalenia płuc wywołanych przez *Pseudomonas aeruginosa* w przebiegu zwłóknienia torbielowatego
Portuguese	Sulfato de amicacina (lipossomal)	Tratamento de infecção pulmonar por *Pseudomonas aeruginosa* na fibrosa quística
Romanian	Sulfat de amikacină (inclus in lipozomi)	Tratamentul infecţiei pulmonare cu *Pseudomonas aeruginosa* la pacienţii cu fibroză chistică
Slovak	Amikacínium sulfát (lipozomálny)	Liečba infekcií pľúc s *Pseudomonas aeruginosa* pri cystickej fibróze
Slovenian	Amikacinov sulfat (liposomalni)	Zdravljenje pljučnice povzročene s *Pseudomonasom aeruginosa* pri cistični fibrozi
Spanish	Sulfato de amicacina (liposomal)	Tratamiento de las infecciones pulmonares por *Pseudomonas aeruginosa* en la fibrosis quística

[1] At the time of transfer of sponsorship

Language	Active Ingredient	Indication
Swedish	amikasinsulfat (liposomal)	Behandling av lunginflammation orsakad av *Pseudomonas aeruginosa* vid cystisk fibros
Norwegian	Amikacinsulfat (liposomalt)	Behandling av lungeinfeksjon forårsaket av *Pseudomonas aeruginosa* ved cystisk fibrose
Icelandic	Amikasínsúlfat (í fitukornum)	Meðferð á *Pseudomonas aeruginosa* lungnasýkingum í slímseigjusjúkdómi