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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Amikacin sulfate for the treatment of nontuberculous mycobacterial lung disease

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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 8 April 2014, orphan designation (EU/3/14/1259) was granted by the European Commission to Insmed Limited, United Kingdom, for amikacin sulfate for the treatment of nontuberculous mycobacterial lung disease.

What is nontuberculous mycobacterial lung disease?

Nontuberculous mycobacterial lung disease is an infection of the lungs by nontuberculous mycobacteria.

Nontuberculous mycobacteria (also known as environmental mycobacteria) are types of bacteria found widely in the environment, such as in water and in soil, but unlike other species of mycobacteria that cause tuberculosis and leprosy they do not usually cause disease in people. When they do cause disease, patients are infected by direct exposure to the bacteria in the environment and not from close contact with other people as can happen with tuberculosis and leprosy.

Lung infection with nontuberculous mycobacteria typically occurs in patients who already have lung disease or who have weakened immune systems. Symptoms include long-term coughing, excess sputum (phlegm) and tiredness and, less commonly, coughing up blood, difficulty breathing, fever and weight loss.

Nontuberculous mycobacterial lung disease is debilitating in the long term because of the lung damage caused by severe forms of the infection, which do not respond well to treatment.



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

At the time of designation, nontuberculous mycobacterial lung disease affected approximately 0.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 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, a number of antibiotics were authorised in the EU for treating nontuberculous mycobacterial lung disease.

The sponsor has provided sufficient information to show that amikacin might be of significant benefit for patients with nontuberculous mycobacterial lung disease because it is being developed as a medicine for inhalation and early studies show that it has better lung penetration and less frequent side effects compared with the intravenous (into a vein) antibiotics used to treat the condition. In addition, there is a possibility that, as a medicine for inhalation, patients may be able to use it outside of the hospital. These assumptions 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 belongs to the group 'aminoglycosides'. It works by disrupting the production of proteins that bacteria need to build their cell walls, thereby damaging the bacteria and eventually killing them.

In this medicine, amikacin is contained within tiny fat particles known as liposomes, which can transport the medicine into the lungs when inhaled. As an inhalation medicine, it is expected to be better at penetrating the lung tissue and targeting bacteria in the lungs than intravenous medicines, while at the same time causing fewer side effects in the rest of 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 patients with nontuberculous mycobacterial lung disease were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for nontuberculous mycobacterial lung disease. Orphan designation of the medicine had been granted in the United States for the condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 6 February 2014 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 28), Norway, Iceland and Liechtenstein. This represents a population of 511,100,000 (Eurostat 2014).

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	Amikacin sulfate	Treatment of nontuberculous mycobacterial lung disease
Bulgarian	Амикацин сулфат	Лечение на нетуберкуозна микобактериална белодробна болест
Czech	Amikacin sulfát	Léčba netuberkulózního mykobakteriálního onemocnění plic
Croatian	Amikacin-sulfat	Liječenje bolesti pluća uzrokovanih netuberkuloznim mikobakterijama
Danish	Amikacinsulfat	Behandling af non-tuberkulose mykobakteriel lungeinfektion
Dutch	Amikacinesulfaat	Behandeling van niet-tuberculeuze mycobacteriële longziekte
Estonian	Amikatsiinsulfaat	Mittetuberkuloosse mükobakteriaalse kopsuhaiguse ravi
Finnish	Amikasiinisulfaatti	Ei-tuberkuloottisten mykobakteerien aiheuttaman keuhkosairauden hoito
French	Sulfate d'amikacine	Traitement de l'infection pulmonaire à mycobactéries non tuberculeuses
German	Amikacinsulfat	Behandlung von durch nichttuberkulöse Mykobakterien hervorgerufenen Lungenerkrankung
Greek	Θειική αμικασίνη	Θεραπεία της άτυπης πνευμονικής μυκοβακτηρίωσης
Hungarian	Amikacin szulfát	Nem-tuberkulotikus mikobaktérium (NTM) fertőzés okozta tüdőbetegség -kezelése
Italian	Amikacina solfato	Trattamento della pneumopatia micobatterica non tubercolare
Latvian	Amikacīna sulfāts	Netuberkulozo mikobaktēriju izraisītas plaušu slimības ārstēšana
Lithuanian	Amikacino sulfatas	Plaučių ligos, sukeltos netuberkuliozinių mikobakterijų, gydymas
Maltese	Amikacin sulfate	Kura ta' mard tal-pulmun minn mikobatterji li ma jikkawżawx it-tuberkulosi
Polish	Amikacyny siarczan	Leczenie choroby płuc wywołanej przez prątki niegruźlicze
Portuguese	Sulfato de amicacina	Tratamento de doença pulmonar por micobactérias não tuberculosas
Romanian	Sulfat de amikacină	Tratamentul infectilor pulmonare cu micobacterii netuberculoase
Slovak	Amikacin sulfát	Liečba netuberkulózneho mykobakteriálneho ochorenia pľúc
Slovenian	Amikacinov sulfat	Zdravljenje netuberkulozne mikobakterijske pljučne bolezni

¹ At the time of designation

Language	Active ingredient	Indication
Spanish	Sulfato de amikacina	Tratamiento de la enfermedad pulmonar por micobacterias no tuberculosas
Swedish	amikasinsulfat	Behandling av icke-tuberkulös mykobakteriell lungsjukdom
Norwegian	Amikacinsulfat	Behandling av ikke-tuberkuløs mykobakteriell lungesykdom
Icelandic	Amikasín súlfat	Meðferð við mýkóbakteríal lungnasjúkdómi sem stafar ekki af dæmigerðri berklabakteríu