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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Tobramycin (inhalation use) for the treatment of *Pseudomonas aeruginosa* lung infection in cystic fibrosis

First publication	24 June 2009
Rev.1: sponsor's change of address	30 March 2011
Rev.2: sponsor's change of address	17 June 2011
Rev.3: withdrawal from the Community Register	6 October 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.	

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in June 2014 on request of the Sponsor.

On 27 February 2009, orphan designation (EU/3/09/613) was granted by the European Commission to PARI Pharma GmbH , Germany, for tobramycin (inhalation use) for the treatment of *Pseudomonas aeruginosa* lung infection in cystic fibrosis.

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 in the body. In patients with cystic fibrosis, there is an overproduction of mucus in the lungs, which leads to inflammation and a high risk for the lungs to become infected with bacteria. *P. aeruginosa* is one of the commonest types of bacteria that 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.

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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 1.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 66,000 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 submission of the application for orphan drug designation, lung infections in patients with cystic fibrosis were mainly treated with antibiotics (medicines that kill bacteria). These are available as tablets, as infusions (drips into a vein) and solutions for inhalation using a nebuliser (a special machine 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 (medicines that help open up the airways in the lungs) and mucolytics (medicines that help to dissolve the mucus). Patients were also advised to exercise and have physiotherapy.

The sponsor has provided sufficient information to show that tobramycin (inhalation use) might be of significant benefit for patients mainly because of the way in which the medicine is given to the patients.This may contribute to the patient care. 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?

Tobramycin is an antibiotic that is already available in the EU as a solution for inhalation for the treatment of *P. aeruginosa* infection in patients with cystic fibrosis. It works by blocking the bacteria's ribosomes, the parts of the bacterial cells where new proteins are made. By blocking the production of new proteins, the bacteria cannot multiply and they eventually die.

In tobramycin (inhalation use), tobramycin is given to the patient using a new type of nebuliser. The nebuliser is expected to deliver the same amount of antibiotic to the lungs as the existing forms of the medicine, but in a shorter period of time. This is expected to be more convenient for patients, helping them to continue taking their treatment.

What is the stage of development of this medicine?

The effects of tobramycin (inhalation use) have not been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients had finished, and additional trials were planned.

At the time of submission, tobramycin (inhalation use) was not authorised anywhere in the world for the treatment of *P. aeruginosa* lung infection in patients with cystic fibrosis or designated as 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 7 January 2009 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 languages[1], Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Tobramycin (inhalation use)	Treatment of *Pseudomonas aeruginosa* lung infection in cystic fibrosis
Bulgarian	Тобрамицин (за инхалаторно приложение)	Лечение на *Pseudomonas aeruginosa* белодробна инфекция при кистична фиброза
Czech	Tobramycin (inhalační podání)	Léčba plicních infekcí vyvolaných Pseudomonádou *aeruginosa* při cystické fibróze
Danish	Tobramycin (til inhalation)	Behandling af lungeinfektion med *Pseudomonas aeruginosa* ved cystisk fibrose
Dutch	Tobramycine (inhalatie gebruik)	Behandeling van *Pseudomonas aeruginosa* longinfectie bij cystische fibrosis
Estonian	Tobramütsiin (inhalatsiooniks)	*Pseudomonas aeruginosa* poolt põhjustatud kopsuinfektsiooni ravi tsüstilise fibroosi korral
Finnish	Tobramysiini (inhalaatioon)	*Pseudomonas aeruginosan* aiheuttaman keuhkoinfektion hoito kystisessä fibroosissa
French	Tobramycine (voie inhalée)	Traitement des infections pulmonaires à *Pseudomonas aeruginosa* dans la mucoviscidose
German	Tobramycin (zur Inhalation)	Therapie der *Pseudomonas aeruginosa*-Infektion der Lunge bei zystischer Fibrose
Greek	Τομπραμυκίνη (εισπνεόμενη)	θεραπεία λοιμώξεων των πνευμόνων με Pseudomonas aeruginosa κατά την κυστική ίνωση
Hungarian	Tobramycin (inhalációs alkalmazás)	Pseudomonas aeruginosa okozta tüdőfertőzés kezelése cisztikus fibrózisban
Italian	Tobramicina (uso inalatorio)	Trattamento di infezione polmonare da Pseudomonas aeruginosa nella fibrosi cistica
Latvian	Tobramicīns (inhalācijām)	Pseudomonas aeruginosa izraisītas plaušu infekcijas ārstēšana cistiskās fibrozes gadījumā
Lithuanian	Tobramicinas (inhaliuoti)	Plaučių infekcijos, sukeltos Pseudomonas aeruginosa, gydymas, sergant cistine fibroze
Maltese	Tobramycin (għal biex jinġibed man-nifs)	Kura ta' infezzjoni fil-pulmun mill-Pseudomonas aeruginosa fil-fibrożi ċistiku
Polish	Tobramycyna (podanie wziewne)	Leczenie zapalenia płuc wywołanych przez Pseudomonas aeruginosa w przebiegu zwłóknienia torbielowatego
Portuguese	Tobramicina (via inalatória)	Tratamento de infecção pulmonar por Pseudomonas aeruginosa na fibrosa quística
Romanian	Tobramicină (administrare inhalatorie)	Tratamentul infecţiei pulmonare cu Pseudomonas aeruginosa la pacienţii cu fibroză chistică
Slovak	Tobramycín (inhalačné použitie)	Liečba infekcií pľúc s Pseudomonas aeruginosa pri cystickej fibróze

[1] At the time of designation

Language	Active Ingredient	Indication
Slovenian	Tobramicin (za inhaliranje)	Zdravljenje pljučnice povzročene s Pseudomonasom aeruginosa pri cistični fibrozi
Spanish	Tobramicina (via inhalatoria)	Tratamiento de las infecciones pulmonares por Pseudomonas aeruginosa en la fibrosis quística
Swedish	Tobramycin (användning för inhalation)	Behandling av lunginflammation orsakad av Pseudomonas aeruginosa vid cystisk fibros
Norwegian	Tobramycin (bruk til inhalasjon)	Behandling av lungeinfeksjon forårsaket av Pseudomonas aeruginosa ved cystisk fibrose
Icelandic	Tobramýcín (til innöndunar)	Meðferð á Pseudomonas aeruginosa lungnasýkingum í slímseigjusjúkdómi