



European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

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Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in September 2006 on request of the sponsor.

Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of

octovalent *Pseudomonas aeruginosa* O-polysaccharide-toxin A conjugate vaccine for the prevention of *Pseudomonas aeruginosa* infections in patients with cystic fibrosis

On 11 December 2001, orphan designation (EU/3/01/076) was granted by the European Commission to Orphan Europe SARL, France, for octovalent *Pseudomonas aeruginosa* O-polysaccharide-toxin A conjugate vaccine for the prevention of *Pseudomonas aeruginosa* infections in patients with cystic fibrosis.

What is *Pseudomonas aeruginosa* infection in patients with cystic fibrosis?

Chronic infection of the lung with the bacterium *Pseudomonas aeruginosa* represents a hallmark of cystic fibrosis. It can induce damage to the lung tissue and respiratory insufficiency, which is life threatening.

What is the estimated number of patients at risk of developing the condition*?

At the time of designation *Pseudomonas aeruginosa* infection in patients with cystic fibrosis affected approximately 1.5 in 10,000 people in the European Union (EU)*. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP). This is below the threshold for orphan designation which is 5 in 10,000. This is equivalent to a total of around 56,000 people.

What methods of prevention are available?

There were no medicinal products authorised for the prevention of *Pseudomonas aeruginosa* infections in patients with cystic fibrosis in the Community, at the time of submission of the application for orphan designation.

How is this medicine expected to work?

Octovalent *Pseudomonas aeruginosa* O-polysaccharide-toxin A conjugate vaccine consists of specific parts from the *Pseudomonas aeruginosa* bacterium. The vaccine is given to the patient with the aim to activate the patient's immune system against any *Pseudomonas aeruginosa*, and prepare the body to fight larger amounts of the bacteria.

What is the stage of development of this medicine?

The effects of octovalent *Pseudomonas aeruginosa* O-polysaccharide-toxin A conjugate vaccine 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.

*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union. This represents a population of 377,000,000 (Eurostat 2001).

Octovalent *Pseudomonas aeruginosa* O-polysaccharide-toxin A conjugate vaccine was not marketed anywhere worldwide for the prevention of *Pseudomonas aeruginosa* infections in patients with cystic fibrosis or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 26 October 2001 a positive opinion recommending the grant of the above-mentioned designation.

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;
- and either the rarity of the condition (affecting not more than five in 10,000 people in the Community) or the 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 the quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information:

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**Translations of the active ingredient and indication in all EU languages
and Norwegian and Icelandic**

Language	Active Ingredient	Indication
English	Octovalent <i>Pseudomonas aeruginosa</i> O-polysaccharide-toxin A conjugate vaccine	Prevention of <i>Pseudomonas aeruginosa</i> infections in patients with cystic fibrosis
Danish	Oktovalent <i>Pseudomonas aeruginosa</i> O-polysakkarid-toksin A konjugeret vaccine	Forebyggelse af infektioner med <i>Pseudomonas aeruginosa</i> hos patienter med cystisk fibrose
Dutch	Octovalent <i>pseudomonas aeruginosa</i> O-polysaccharidetoxine A conjugaatvaccin	Voorkomen van <i>pseudomonas aeruginosa</i> -infecties bij patiënten met mucoviscidose
Finnish	Oktovalentti <i>pseudomonas aeruginosa</i> O-polysakkaridi-myrkky A liitännäisrokote	<i>Pseudomonas aeruginosa</i> infektion torjunta potilailla, joilla on rakkomainen sidekudostuma
French	Vaccin polyvalent contenant des conjugués de O-polysaccharide et toxine A de <i>Pseudomonas aeruginosa</i>	Prévention des infections à <i>Pseudomonas aeruginosa</i> chez les patients atteints de mucoviscidose
German	Octovalentes <i>Pseudomonas Aeruginosa</i> O-Polysaccharid-Toxin A konjugiertes Vakzin	Zur Prophylaxe von <i>Pseudomonas Aeruginosa</i> Infektionen bei Patienten mit Cystischer Fibrose
Greek	Οκταδύναμος συνδυασμός εμβολίου με βακτηρίδια του κυανού πύου O-πολυσακχαρίτη-τοξίνη Α	Πρόληψη λοιμώξεων από βακτηρίδια του κυανού πύου σε ασθενείς με κυστική ίνωση
Italian	Vaccino ottavalente coniugato con O-polisaccaride -tossina A di <i>pseudomonas aeruginosa</i>	Prevenzione delle infezioni da <i>Pseudomonas aeruginosa</i> nei pazienti con fibrosi cistica
Portuguese	Vacina conjugada com O-polissacáridos-toxina A octovalentes de <i>Pseudomonas aeruginosa</i>	Prevenção das infecções por <i>Pseudomonas aeruginosa</i> em doentes com fibrose quística
Spanish	Vacuna octavalente conjugada con O-polisacárido-toxina A de <i>Pseudomona aeruginosa</i>	Prevención de las infecciones de <i>Pseudomona aeruginosa</i> en pacientes con fibrosis quística
Swedish	Oktovalent konjugat vaccin mot <i>Pseudomonas aeruginosa</i> O-polysackarid-toxin A	Förebyggande av <i>Pseudomonas aeruginosa</i> infektioner hos patienter med cystisk fibros